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BIOTECHNOLOGY SUMMIT: PUTTING A HUMAN FACE ON BIOTECHNOLOGY

HEARING

before the

JOINT ECONOMIC COMMITTEE
CONGRESS OF THE UNITED STATES

ONE HUNDRED SIXTH CONGRESS

FIRST SESSION

September 29, 1999

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BIOTECHNOLOGY SUMMIT: PUTTING A HUMAN FACE ON BIOTECHNOLOGY

September 29, 1999

Congress of the United States,
Joint Economic Committee,
Washington, D.C.

The Committee met at 9:45 a.m., in Room SH-216 of the Hart Senate Office Building, the Honorable Connie Mack, Chairman of the Committee, presiding.

Senators present: Senators Mack, Frist, Bennett, Kennedy, Sessions, and Robb.

Representatives present: Representatives Stark and Minge.

Staff present: Shelley S. Hymes, James D. Gwartney, Colleen J. Healy, Victor Wolski, Chris Edwards, Kevin Boyle, Lori Hodo, Stephen Schultz, Joseph Pasetti, Angela Ritzert, Josephine Robinson, James Carter, Kerry Fennelly, Kurt Schuler, Chuck Skipton, Lawrence Whitman, Howard Rosen, Leah Liston, and Daphne Clones.

OPENING STATEMENT OF SENATOR CONNIE MACK, CHAIRMAN

Senator Mack. Good morning, everyone. Can you all hear me out there?

Good. All right.

We're still waiting for some of the panelists to arrive. But I think we will go ahead and get started. And we expect some other members of either the House or the Senate to join us.

I have an opening statement. I'm sure that Mr. Stark has as well, and possibly one other. So we'll go ahead and get started.

Again, I want to welcome all of you to the Joint Economic Committee's Second High Technology Summit, "Putting a Human Face on Biotechnology."

Oftentimes, when we discuss high technology, especially here at the Joint Economic Committee, we tend to speak in numbers. What is its effect on the economy, on the financial market? How can we continue this growth?

All very important and relevant topics and we will obviously get into those during the balance of the day.

But the technological revolution we are experiencing is touching all of us in a much more personal way—by improving the quality of our everyday lives.

Today's panelists of industry leaders, innovators and heroes are here to help us understand what Washington can do and what it should do—to ensure that the biotechnology industry continues to thrive and develop products that will improve our standard of living.

U.S. biotechnology is clearly outpacing the rest of the world. Our biotech industry is about five times larger than in Europe. There are almost 1300 U.S. biotech companies that employ more than 140,000 people in high-wage and high-value jobs.

As I see it, we've entered the era of the Innovation Economy, a system in which we see as never before the value of an idea.

Today, more than ever before in our history, brain power is being valued as the engine of economic growth.

I view this new economy as kind of a continuum—a logical progression rooted in the freedom that sets our country apart:

Freedom leads to Knowledge.

Knowledge leads to Innovation.

Innovation leads to Capital Formation.

Capital Formation leads to New Products.

And New Products lead to New Jobs.

It is a virtuous cycle which has produced immeasurable blessings for men and women all around the world.

It has lifted millions out of poverty. It has stretched the limits of human achievement and it will generate benefits tomorrow that we can't begin to comprehend today.

And it doesn't just generate wealth. It generates progress.

As a case in point, consider biotechnology.

And when it comes to strengthening government policy to advance medicine and science, let me be very clear and declare my special interest right up front.

I'm a cancer survivor. I'm a big believer in the miracle of modern medicine.

However, I know that for every medical miracle, there are a hundred failed efforts—promising ideas that just don't pan out.

Are they wastes of time and effort and research and development? No, not if each failure narrows the search for the answer. Making miracles is hard work, expensive work. That's why we need a system that recognizes the interplay between our market economy and the pursuit of medical knowledge.

To show you what happens when we don't, when we flirt with policies that punish market incentives instead of promote them, think back to the beginning of the Clinton presidency.

President and Mrs. Clinton attacked free market pricing for pharmaceuticals. Gordon Binder, the CEO of Amgen, recently testified before this Committee that because of the President's price controls threats, R&D spending stagnated. The free market reassessed what these biotech companies would be worth under a Clinton-style national health care system, and their value plummeted—$100 billion just vanished.

Now you might be thinking, that's too bad if you're a stockholder in a big drug company.

But the fact is that we're all stakeholders in modern medicine's search for cures for our most deadly diseases. And that $100 billion would have supported untold numbers of research projects into Alzheimer's, AIDS and cancer or cardiovascular research—research that might have saved someone's life.

I am strongly committed to the effort to double funding to the NIH over the next five years. Medical research is the key to saving lives.

We are on the verge of discovery in so many different areas of disease, it is crucial we provide our scientists with the tools necessary to continue the tremendous advances being made in biomedical research.

My wife is alive today, my daughter is alive today, and I am alive today because of the advances made in cancer research and in early detection.

I believe it is the freedom that we enjoy as Americans that help bring us the ideas and products that change our lives. The vitality of high technology in this country reflects our economic freedom.

And it is our freedom that allows each and every person to innovate, to exercise the entrepreneurial spirit that turns innovation into jobs and GDP.

That's the genius of free enterprise.

That's the genius of America.

For more than two centuries, it's what helped make America the envy of the world.

Now, as we approach the new Millennium, it's what will make the next century a new American century as well.

And now I turn to my colleague and Ranking Member, Mr. Stark.

OPENING STATEMENT OF REPRESENTATIVE PETE STARK, RANKING MINORITY MEMBER

Representative Stark. Thank you, Mr. Chairman. I want to thank you for holding this hearing. I think it promises to be fascinating. It gives us an opportunity to learn about technological advances, many of which have the potential to save lives.

Certain innovations are allowing us to determine which genes lead to breast cancer, down syndrome, and other diseases. And we're now looking at ways to use fetal tissue to reverse terrible debilitating diseases.

And if the Kansas school boards don't turn us around from that course, maybe we can continue research in that and other areas. There are a lot of roadblocks in the way and a lot of things helping us.

Another group of advances, particularly in agriculture, have somewhat of an Orwellian nature for many of our constituents. And I'm not referring to the thought that crossing a rooster with a rooster gives you a cross-rooster. I am referring to the fact that many of our colleagues and our constituents are concerned about the unknown—what's going on in soybeans? What are they doing to sexually change vegetables?

And in Europe, they also want to know, and they want us to label. And they're not going to let us export if we don't.

My bit of advice, and I hesitate to do this with such a highly-skilled group of witnesses, is that Congress isn't going to sit around and dream up ways to force labels onto things which you all produce; our constituents will. And better you all do it before we do it here, because we will be, per force, ham-handed and create more problems for you. You can solve these problems early on.

You decided, Mr. Chairman, in your judgment and wisdom to call this summit, "Putting a Human Face on Biotechnology." In addition to helping improve the understanding about the industry, I hope we'll have an opportunity to discuss what impact those developments have on society as well as the economy, and most importantly, on individual Americans.

There is one point I'd like to highlight, and it is a great accolade for the biotech industry. In contrast to the pharmaceutical industry, biotech supports a Medicare prescription drug benefit that provides catastrophic stop-loss coverage. To me, that's a meaningful first step in a drive to enact universal prescription drug availability, particularly for our nation's seniors who are most impacted by the higher costs.

Medicare, as you know, lacks a basic drug benefit and many of those seniors without drug coverage can't afford the drugs they need, especially when they are the sickest and need them most. Our nation's

seniors need and use prescription drugs more than any other age cohort. Between one-half and a third of Medicare beneficiaries lack coverage and currently pay up to twice as much as those who have prescription drug insurance.

Recently I found out that a certain group of drugs that Medicare pays for, the 20-percent copay that our beneficiaries pay, or their Medigap insurers pay, is more than the wholesale cost to the physicians who administer these drugs. This means that the 80 percent the government pays goes right to the bottom line. I suspect that we won't be able to tolerate that much longer and we're going to have to begin to look at reasonable reimbursement. I think that that will make sense.

My colleague Senator Kennedy and I joined with other members of Congress to introduce the Access to Prescription Medications in Medicare bill. Our legislation hopes to provide seniors and the disabled with the coverage they need.

Today, we'll hear from Ms. Joan London, who suffers Rheumatoid Arthritis. Under the guise of Citizens for Better Medicare, the pharmaceutical industry, which is spending about $30 million on ads, are making an attempt to kill the President's Medicare drug plan. The pharmaceutical industry, PhRMA, are running radio and TV commercials which bring you a fictitious Flo—the senior citizen who adamantly states that she doesn't want big government in her medicine cabinet. Flo may not want big government in her medicine cabinet, but it's sure obvious that PhRMA wants big government when it comes around to tax credits for research, FISC plans for exports, funding medical research, and patent protection for drugs so that they can keep overcharging seniors.

I'd like to suggest to PhRMA that this is a two-way street.

Now Ms. London, like the real Flo—she's the real Flo—like the actress in the commercials, Ms. Joan London suffers from arthritis.

But unlike the commercial, Ms. London is probably not as worried about the government being in her medicine cabinet because her cabinet is bare.

She cannot afford the new medications which could halt the development of her crippling disease and bring her some relief.

Flo is fictitious. But there are millions of Joan Londons around the country and I want to thank Ms. London for taking the time and the courage and the effort to join us this morning to share her real story.

Advances made by the biotech industry help people live longer lives. But that doesn't necessarily mean healthier lives if they can't get the medications.

And I'd like to add that if we fail in providing a universal drug benefit, millions of middle-class seniors will effectively find themselves denied the innovations that the witnesses here today are trying to bring and they must be accessible.

Now I've often wondered why we're having this hearing. Is it economic?

And the fact is it is.

This Committee has always been interested in the income gaps between particularly the unemployed and those of us who enjoy a more comfortable life.

We are now beginning to experience a health gap. And I hope we can keep that in focus.

One last comment, and that's in this morning's *Post*—the story of a young man who died volunteering in an experimental gene therapy program, which he didn't need.

He was a volunteer. He had a disease. But it was under control with standard therapy. He volunteered. Perhaps there was an overly aggressive treatment in this gene therapy in this experiment, and the kid died.

His parents are not unhappy. They think their son was a hero, they say in the newspaper article, and they felt he volunteered to do it to help others.

But I do think that we have to, in our rush to get drugs approved, make sure that we don't overreach, that in blind studies, that we are not medicating people who otherwise might be harmed and that we do that very carefully.

This again could be something that we would receive a tremendous reaction from the public and have to legislate. I doubt if we would legislate in the absence of that, but, as I say, a word to the wise will save the Chairman and myself an awful lot of work and many more hearings to try and regulate in an area which, if the industry takes the responsibility initially, we will not have to do it.

So thank you. Again, I look forward to hearing from our witnesses and finding what bridges we can to provide older and eventually all Americans access to the life-saving advancements that we'll hear about.

Senator Mack. Thank you.

Senator Frist?

OPENING STATEMENT OF SENATOR BILL FRIST

Senator Frist. Thank you, Mr. Chairman.

I, too, want to commend you for holding this extraordinary hearing on a field which means a great deal to me as a public servant, as a United

States Senator, and as a physician, someone who spent 20 years of his life watching, observing and participating in the great advances that have been made over the last 20 years. But also having the opportunity to see those advances be translated in a very direct way to better health care, longer lives and better qualities of life.

It all comes from having a foundation; a past investment. We'll be talking about today as we look ahead to the future.

I very much appreciate your efforts on doubling the funding for medical research, something that I believe so strongly in because it pulls us both into the long-term, as well as the short-term, in a way that few other issues do, in that the investments today pays out two years, three years, four years. But the greatest pay-out is probably five or ten years down the road.

It's difficult to pull policymakers that far into the future. And I appreciate your leadership on this issue.

As I look through the schedule and format today, there are so many things that biotechnology touches upon—the medicines themselves.

I look back to my own practice in the field of transplantation, which has literally been revolutionized by the use of polyclonal antibodies and monoclonal antibodies.

I remember those first patients who had acute rejection, which used to kill everybody.

Now that's not the issue, we're able to prolong life and give a much better quality of life to people because of the great break-throughs in the biotechnology industry.

I had the opportunity to chair the Subcommittee on Public Health and I also chair the Subcommittee on Africa.

In Africa alone, if we look at infectious diseases, about 4000 people a day die of AIDS and about 2000 to 3000 people die of malaria, tuberculosis and other types of infectious diseases.

And when you look at the great advances that are being made in vaccine and vaccine development, it translates very directly to me into human life.

If you look at the field of diagnostics—again, in the transplantation field where, when I participated in doing those scores and scores of transplants, each time that you screen blood for a donor to a recipient, which it's critical to do, it's based on diagnostic tests, almost all of which came out of the biotechnology field, whether you're screening that blood for HIV or hepatitis or hepatitis C, which we didn't know anything about 20 years ago, and now we know it is one of the greatest public health

challenges that we have today, those diagnostic tests, how critical they've been.

Gene therapy has been mentioned. We're just on the cusp. As the human genome project, which is unleashing three billion bits of information we didn't have six years ago, as that is unleashed, how we use that and apply that in a very practical sense.

In my own field of coronary artery disease, where as a heart surgeon, atherosclerosis, hardening of the arteries, is the number one cause of heart disease.

Great breakthroughs are being made in terms of genetic therapy, most directly through direct infusion. Tremendous opportunities exist there as well. So this is a very exciting conference for me from a personal basis.

Because this is the Joint Economic Committee, our focus today will also be on the economic aspects. As we look at the dramatic double-digit increases year after year, we'll see the huge impact.

Just the one statistic—the U.S. biotech industry did spend about $10 billion in R&D in 1998. The five top biotech companies spent an average of $121,000 per employee on research and development. That compares to an average of about $31,000 per employee for the top pharmaceutical companies, again driving home that this is a different field.

So, again, I'll close by thanking the Joint Economic Committee for its interest in a very important topic, one that we can benefit from by listening to our witnesses and the discussion today.

Senator Mack. Thank you, Senator Frist.

Again, I welcome each of the panelists. We're still several short, but I am confident that as we proceed, the other panelists will be here.

I think what I'll do is start with Ms. Boyer-Fortier. If you would, why don't you proceed with your testimony. I probably will then look to Dr. Shak for a few comments. And then we'll see how the rest of the morning goes. Ms. Fortier?

PANEL I

STATEMENT OF CAROLYN BOYER-FORTIER, BREAST CANCER SURVIVOR

Ms. Boyer-Fortier. Thank you, Senator Mack.

Can you hear me with this?

Senator Mack. Yes.

Ms. Boyer-Fortier. Good morning. I am Carolyn Boyer-Fortier. I would like to thank you and the Committee for making it possible

for me to be here today to share with you my experience as a breast cancer patient.

I would also like to thank the people at Genentech who have literally made it possible for me to be here today.

Their dedication in developing new treatments for breast cancer and for other life-threatening diseases is greatly appreciated by all of us.

I was first diagnosed with breast cancer in 1993. It was Stage 3.

For those of you unfamiliar with the stages of breast cancer, a Stage 3 and particularly a Stage 4 diagnosis is pretty serious. It's an advanced case of breast cancer.

At the time, my doctors recommended immediate surgery and chemotherapy, which was pretty standard treatment at the time.

At that time, the chemotherapy is quite toxic and I was quite sick. There were new anti-nausea medicines, but either I didn't respond or they weren't working for me.

But in any event, I suffered a lot of the normal chemotherapy reactions—severe nausea, dehydration. I had a lot of cases of shingles, severe infections, things that we don't wish of anyone through the initial nine-course treatment.

It took about a year actually to fully recover from the effects of surgery and chemotherapy. But I fortunately was able to return to work full-time as a tax counsel.

And I was lucky enough to have a full year of disease-free time.

But then, in 1996, I was diagnosed with metastatic breast cancer. Metastatic disease is disease that spreads to the distant organs.

We discovered widespread metastatic disease in my liver and multiple sites in my bones.

Unfortunately, those are very common areas for breast cancer to spread. I think a lot of us are familiar with that. It goes to the liver and the bones and the lungs and the brain.

Unfortunately, there's no known cure for metastatic disease. One of the things is that patients are faced with decisions on what kind of fairly aggressive treatments to undertake, knowing that these treatments are not likely to significantly prolong your life.

Those are fairly aggressive treatments—the bone marrow transplants, the aggressive chemotherapy, and you have to think about the side effects of treatment at the same time that you're debating on maintaining quality of life.

My doctors were understandably reluctant to predict how long I would live. But I do understand that, statistically, a patient with

metastatic disease in the liver would have a survival time of about 18 to maybe 24 months.

And as I said, the usual treatments are the aggressive chemotherapy, which are pretty toxic on your system.

But while we were deciding on what course to proceed, one of my doctors learned that Genentech was undertaking a clinical trial. It's a Phase 3 clinical trial of what they called a monoclonal antibody, which Dr. Frist mentioned.

We called it HER2-new. I think they now call it Herceptin, which we call it Herceptin.

But one of the things we did as patients was called it a smart bomb because it targets just the cancer cells and not the rest of your body, which takes away the toxicity.

There's a good news/bad news in being eligible for that trial.

The bad news is that it meant that your tumor was aggressive and chemotherapy-resistant. But the good news was that you may be able to get the drug in the trial.

And I do say, fortunately, I was randomized to get the drug.

So in July of 1996, I started taking Herceptin, along with conventional chemotherapy, Taxol, because that was part of the trial.

I'm very pleased to report that the adverse side effects of the Herceptin were minimal. I did find that I developed congestive heart failure, which Dr. Shak may mention. But we've been able to keep that under control with the usual heart medications.

And I did have some of the side effects that you get with Taxol, which is the numbness in your hands and your feet, the nausea, the fatigue, the bald look, which my family has become accustomed to.

I've been bald three times and I'm afraid I will be again soon.

But, anyway, most important, even with frequent treatment and with not knowing how long you would have, I have had a wonderful quality of life.

I've had some limitations on my sports activities, but I have been able to travel, socialize. I worked part-time as long as I could. I took golf trips.

I've lived a very full and contented life.

And it's actually been 39 months now since I started on Herceptin. And the metastasises in my liver are still under control.

I mention that because that was the most life-threatening condition we thought. I do, and it's unusual, still have progression in my bones. And so I am continuing with the Herceptin and I'm also trying a variety of other conventional chemotherapies to control pain and progression.

But I do have to say that my quality of life is still very good.

I'm very thankful to Genentech and to all of the cancer R&D programs that are making these new treatments possible for us.

The quality of life is wonderful and the management of the disease that at present is considered terminal is something we're truly grateful for.

I don't really have time to recount all of the wonderful moments that I've had in the last three years. But we've celebrated weddings, birthdays, Christmas and Thanksgiving with families, summer at the beach, vacations, golf trips.

Life is never so precious as when you live on borrowed time. I've been grateful to have more time and I'm looking forward to celebrating the Millennium with family and friends.

We thank you for everything you've done for us, making this possible for the companies, and we urge you to continue to support this kind of research.

Thank you for your time.

[The prepared statement of Ms. Boyer-Fortier can be found in the submissions for the record.]

Senator Mack. Thank you for your testimony. You have, I think, outlined the experiences that so many individuals and families have gone through. And thank you for telling us your story.

It's not always the easiest thing to do, and especially not maybe in front of a joint committee of the Congress. But thank you very much for doing it.

Ms. Boyer-Fortier. Well, thank you. I'm glad to be able to do it.

Senator Mack. Dr. Shak, do you want to take just a couple of moments to just add something to that? And then we'll move on to the panels and we'll get back to questions.

STATEMENT OF DR. STEVEN SHAK, STAFF SCIENTIST AND SENIOR DIRECTOR OF MEDICAL AFFAIRS, GENENTECH

Dr. Shak. Thank you. Mr. Chairman and members of the Joint Economic Committee, thank you very much for the opportunity to give testimony here today.

I'm here today for the same reason that I go into work every day—I hate disease and the way that it unfairly strikes us, and I want to do something about it.

The founders of Genentech, Robert Swanson and Herbert Boyer, had a simple idea in the 1970s that launched the biotech industry.

This was the idea that if one could sequence, cut and then splice DNA, one might make it possible now to manufacture normal human proteins and use them as drugs.

It was this simple idea and my desire to make a real difference in the lives of patients that brought me to Genentech in 1986.

You've now heard about Herceptin and what it has meant. Clearly, Herceptin is one of the jewels of biotechnology.

Herceptin is the first monoclonal antibody that was developed for the treatment of breast cancer.

About a third of women with this breast cancer have tumors with a specific genetic alteration, causing overproduction of her2 on the surface of their cells.

Too much her2 drives those cells to grow more rapidly, as it was described, and to spread sooner to other tissues.

Leonard Presa, Paul Carta, other scientists at Genentech performed a dramatic feat which I will never forget.

Our bodies normally make antibodies to fight infections. They re-engineered a human antibody to bind to her2 on those breast cancer cells to target and fight the cancer.

Clearly, as you've heard, the results of these clinical trials did exceed all of our expectations. Few trials in metastatic breast cancer have ever demonstrated a survival advantage.

The success of Herceptin, I do believe, signals an end of the beginning in our fight against breast cancer.

We can understand what makes a cancer cell grow uncontrollably and with biotechnology, we can find new drugs that can target the tumor, hopefully being tough on the tumor and gentler on the woman's body.

I should stress here that without the R&D tax credit, it's likely that this success story might never have happened.

The early results of the clinical trials were not so robust. This project was held in skeptical regard both inside and outside the academic community.

An antibody had never done the kinds of things we had hoped for.

This project was on the bubble and like other projects on the bubble, without the R&D tax credit, it is possible that we might never have performed the study that allowed us to understand this important, new innovation.

I am one of many scientists who work at Genentech. We do all know the challenges of drug development and the odds of success. We know, as the Chairman pointed out, that most of our most promising

ideas in the laboratory will never even satisfy our criteria for human testing.

And we know that of the small percentage of drugs that actually are tested in humans, only about one in five are successful.

Nevertheless, we all have a strong belief in what people can do, in the power of science, and in the power of this technology.

We thank you for your leadership and for preserving an environment that supports our efforts.

Thank you very much.

[The prepared statement of Dr. Shak can be found in the submissions for the record.]

Senator Mack. Thank you.

I want to welcome Dr. Breyer-Lewis and Ms. Fagan.

I think we will go first, though, to Ms. London, if you're prepared.

And again, I welcome you. And to Dr. Bunning. We're delighted that you're with us as well.

Ms. London?

STATEMENT OF JOAN LONDON, RHEUMATOID ARTHRITIS PATIENT

Ms. London. Mr. Chairman, and honorable members of the Joint Economic Committee, my name is Joan London and I appreciate the opportunity to testify before you this morning.

I'm accompanied by Dr. Robert Bunning, who is director of the arthritis program at the National Rehabilitation Hospital here in Washington, D.C.

We commend the Committee for focusing on the biotechnology industry and the important role it plays in our country's future.

Please forgive me if I seem a little stiff. It's either because this is my first time in front of a Congressional Committee, or that five weeks ago, I had titanium rods installed in my neck, which makes me about as stiff as one of Lance Armstrong's bikes.

(Laughter)

On a more serious note, this risky, six-hour, $70,000 surgery and rehabilitation was one of eight major operations and multiple hospitalizations I've had to endure during my 30-year battle with rheumatoid arthritis.

Hopefully, however, it will be the last, now that advances in a new class of drugs are available that can halt and perhaps even reverse this disease's painful and debilitating effects.

However, I must have access to the drugs.

I'm here today to tell my story. My story is not unique. Rather, I'm one of 43 million Americans who attempt to live fully, despite arthritis, which is the nation's leading cause of disability among Americans over age 15.

Of those 43 million people, I, along with approximately 2.1 million Americans, suffer from rheumatoid arthritis, which I'll refer to as RA.

This virulent disease has attacked the lining in the joints throughout my body, neck, shoulders, hands, elbows, knees and ankles.

It has gradually destroyed my cartilage, bones, tendons and ligaments, leading to chronic and really debilitating pain, disfigurement, and a potentially fatal ending.

Unlike osteoarthritis and other forms of arthritis caused by wear and tear, there's no known cause of RA. Unlike other autoimmune diseases, it can attack anyone at any age, even very small children.

In particular, it attacks women of child-bearing age and there's no known cure.

I was 25 when I was first diagnosed. While my doctors prescribed anti-inflammatory drugs, steroids, cancer drugs, and other medications as they became available, I gradually lost my ability to participate in many of life's activities. I could no longer play sports and over the years, caring for my three children, cooking, turning the car's ignition key, and even buttoning my own buttons became difficult, if not impossible.

Day by day, week by week, month by month and year by year, this disease relentlessly progressed, playing havoc with every aspect of my life.

Despite these limitations, I've raised three fantastic children as a single parent and worked at Texas Children's Hospital as communications director for 11 years.

As a volunteer advocate for arthritis awareness and education, I have a commission on the Maryland Governor's Committee on Arthritis and Related Diseases. I am assistant editor of the disability interest group newsletter of the Association of Educators in Journalism and Mass Communication. And I serve as a board member on the Maryland chapter of the National Arthritis Foundation.

Regrettably, however, in 1996, I had to go on long-term disability in order to undergo surgery on both hands, including a fusion of my right wrist.

Losing my ability to work, I enrolled at the University of Maryland's PhD program in mass communication with the hope of teaching, even if from a wheelchair.

In 1997, I earned my master's in journalism and last spring completed my PhD course work.

That's when my life came to a halt.

I was scheduled to take my comprehensive doctor exams next week, on October 4th. However, 30 years of destruction caused by RA to the joints surrounding my spinal cord put my life at imminent risk.

Tingling and numbness ran up and down my fingers, an urgent warning that my spinal cord was being compressed.

If I was bumped, fell, or even sneezed too hard, I could sever the cord, since the joints were no longer able to protect it.

It was very scary. I had no choice but to agree to the surgery.

On August 23rd, I underwent the potentially life-threatening surgery, fusing four joints in my neck, including the two closest to my brain.

I suggest to the Committee that drugs are now available that can potentially prevent further deterioration. New therapies, such as Enbrel, Rava, Remicaid and Prescorba column, have produced improved results and relief of RA symptoms with minimal side effects.

Enbrel is the first in a new class of RA drugs known as biologic response modifiers. Although the FDA has approved Enbrel, Medicare, which is my health insurance coverage, doesn't cover this drug because, as an injection, it can be self-administered. And Enbrel costs about $1000 a month.

That's a huge sum of money to pay for one medicine, especially when I already spend about $350 out of pocket on other medications.

However, clinical trials show that had I been able to take Enbrel even five years ago, I potentially could have foregone the spinal cord operation that has permanently reduced the mobility in my neck and done nothing to curb the progression of my disease.

Importantly, the surgery has contributed to the $4.8 billion spent annually by Medicare on RA-related causes for the 700,000 Medicare recipients with RA, according to HCFA statistics of 1994, and it's probably higher now.

Those were the latest statistics I could get.

I am here today to put a human face on rheumatoid arthritis. Before I close, I'd like to introduce another face, a lady seated behind me.

It's the face of Cindy Lorenz, a 25-year-old University of Maryland graduate student, and my friend.

Cindy has had RA since age 11. Just think—if Cindy gains access to these medications, she may not have to face the future I did.

We are just two of the 43 million faces of people with arthritis who would benefit if we had better access to the new medicines.

For nearly 20 percent of the population in America, suffering from the more than 100 forms of arthritis, I submit to the Committee that you have the ability to change many people's lives.

I urge you to add an inclusive Medicare pharmacy benefit and encourage private insurers to include these drugs in their formulary list.

Mr. Chairman, once again I want to thank you and the other members of the Committee for your gracious invitation and your patience while I told my story.

On the surface, it may not seem uplifting, especially looking at me in this brace. However, I do believe my story is uplifting.

With the promising advances in biotechnology at hand, I could be back here next year describing how these new drugs, after 30 years, have allowed me to resume life in a positive and fulfilling manner.

Thank you for your time.

Senator Mack. Ms. London, thank you very much for your testimony.

I wish we had more time for all of you to make your statements. But I assure you that your being here and your statement is very uplifting.

Dr. Bunning?

STATEMENT OF DR. ROBERT BUNNING, DIRECTOR OF ARTHRITIS PROGRAM AT THE NATIONAL REHABILITATION HOSPITAL

Dr. Bunning. Notice that I was the only one that didn't get a microphone.

(Laughter)

Thank you, Mr. Chairman, and members of the Joint Economic Committee.

There were a couple of questions posed to me. Maybe I'll just try to focus on one of them. And that was, what are the implications for your patients if they are unable to receive these medicines?

It is a very exciting time to be a rheumatologist because for the first time in about ten years, there are a couple of new drugs approved for the treatment of rheumatoid arthritis.

Ms. London mentioned them—Enbrel, which is a biologic modifier, and Rava. It's a very exciting time. But when I have to tell a patient that they can't afford these drugs or they're not approved, it does have an effect on them.

One effect I think is that they're probably going to suffer more disease activity.

This is hard to measure and I think it takes a specialist to probably appreciate what that increased activity is, and also the cost of it.

Some of these costs are hard to figure out on a year-to-year basis.

As Ms. London said, she worked many years. But with rheumatoid arthritis, at least the way we were treating it up until now, about 67 percent of the patients who hold jobs at the time of their diagnosis are unable to and become disabled.

And I think that these new medicines will have a lower incidence of disability. But it will take years for that to show up.

So when we try to calculate that on a year-by-year basis, it's difficult.

But in addition to facing perhaps the increased disease activity, the patients also face disappointment and a loss of hope.

Without hope, a number of patients, it's been shown, turn to unproven remedies. There's a very high incidence of use of unproven remedies in arthritis, probably related to the trouble we've had finding good medications.

That's another factor that's hard to measure the cost, when people spend money on unproven remedies, most of which are not helpful, and some of them suffer bad side effects from unproven remedies, which is another cost that doesn't easily go into your equation.

It's much easier to figure out how much these drugs cost than to figure out what it costs not to use them.

In addition to the loss of hope, many patients go away disappointed. And I think they feel in our country, they know that in this country, if you're a criminal and commit a terrible crime, the country will pay to give you a lawyer if you need one. And sometimes I think they feel disappointed that not everybody gets access to our wonderful health care that we have overall in this country.

In addition to the individual patient, the group of patients with a certain disease suffer somewhat when there's not access to the medicines.

As rheumatologists who spend most of our time treating arthritis, we get a feel for these medications and if we are allowed to use them in a larger group of people, we find out more quickly which ones work better.

We try to go with the ones that are working the best.

The early studies give us a good clue. But it isn't until you get it out into private practice and use it—the clinical trials are a smaller group of patients that are perhaps healthier and in some ways, selected carefully. But it isn't until we get it out into our common practice and into our

regular practice that we find out some of the fine points about the medicine, which works better.

And if we can't use these medicines in our patients, it takes a longer time to figure out what's working well.

And then there's a possible loss to all of us, even those that aren't patients, if we don't allow access to the best therapies that are available.

The cost/benefit analysis on an annual basis really considers literally global issues; such as, next month, I'll be going to the American College of Rheumatology meeting in Boston and there will be 3000 rheumatologists there. But there will also be about 2000 to 3000 international physicians and therapists.

Right now, we export rheumatology health care. It's a product we have. Because of our expertise in using the latest and best medications, people come from all over the world to hear how we do it in America.

I'm not an economist, but I read something the other day about the balance of payments not being so good. I think this is one product that we would like to keep.

But it doesn't show up when you balance the cost of the medicine on an annual basis. But right now, that's a product we have.

Just to bring it closer to home, I work at National Rehabilitation Hospital in Washington, D.C., where if anybody who needs rehabilitation, they may end up going.

Ten percent of our patients come from across the world. They come here for rehabilitation because it's a product we export. We are the best at it.

Our profit margin right now—I'm glad we have a profit margin, which is difficult to have as a hospital these days. But our profit margin is this group of international patients.

The reason we can provide that is that up until now, we have been very good as a country at developing the best medicines, implementing the best medicines.

I think it's important that we continue to make a commitment to do that, not only for the individual patient who can benefit, but for all of us and for our country.

Senator Mack. Thank you, Dr. Bunning.

Ms. Fagan, again welcome. We look to you for your testimony this morning.

We're all delighted that you're here and we look forward to what you have to say.

STATEMENT OF ERIN FAGAN, KIDNEY TRANSPLANT PATIENT

Ms. Fagan. Well, thank you.

Hi. My name is Erin Fagan. I'm from Nashville, Tennessee. I'm a patient with the Vanderbilt University Medical Center.

I have had a history of end-stage renal disease which began after having pneumonia when I was 13 years old.

I began dialysis shortly thereafter. And I received a kidney transplant when I was 17, and it rejected eight years later.

I have been and continue to be a peritoneal dialysis patient, meaning I am on dialysis every night for ten hours.

Earlier this year, I was suffering from severe tiredness and became very anemic.

At that time, my Vanderbilt doctor, Dr. Julia Lewis, felt I was a good candidate for EPO and in April of this year, I began receiving EPO treatments.

At first, I was receiving an EPO injection three times a week, but I am now getting a full-dose injection just once a week.

I feel great and, even better, I am able to hold a full-time job.

At the beginning of the EPO treatments in April, I started a full-time job with the Tennessee State Department of Labor and Workforce Development.

I work as a secretary in the Employer Services office. And I also work several nights a week at the Bluebird Café, a famous Nashville restaurant where you can hear local songwriters and singers.

I know today's hearing is looking at how biotechnology drugs can change people's lives, and I can tell you how important this drug has been for both my health and my ability to work, and I am very excited about a future that will be less disruptive because of my illness.

Thank you for inviting me to join today, and I want to say a special hello to my senator, Senator Bill Frist.

Thank you.

[The prepared statement of Ms. Fagan appears in the Submissions for the Record.]

Senator Mack. Thank you very much.

Dr. Lewis, is there something that you want to add?

STATEMENT OF DR. JULIA BREYER-LEWIS, DIABETIC NEPHROLOGIST

Dr. Breyer-Lewis. Sure. Thank you, Mr. Chairman.

I also want to particularly thank Dr. Frist, who is not only my senator from Tennessee, but a former colleague from Vanderbilt.

We still miss him in that role.

EPO is actually, I think, a wonderful success story. Erythropoietin is a hormone made by the kidney that makes you make blood in the bone marrow.

So it gets transported in your blood from the kidney to the bone narrow.

It's a wonderful story. The NIH funded basic research in the '70s, where EPO was purified from human urine. It was classic laborious protein biochemistry.

No one at that time could foresee where it would go. They did purify human erythropoietin from the urine and subsequently, the development of identifying the gene and the biotechnology to produce mass quantities of pure human erythropoietin allowed clinical trials which demonstrated its great benefit in humans.

One hundred fifty thousand Americans receive EPO. This is a huge number of patients to benefit from a gene therapy.

These are patients who are on dialysis, whose own kidneys won't make the hormone.

And I'm old enough to remember the life of dialysis patients before EPO was available. The blood transfusions that they required, we could never keep up. They were always fatigued, extremely anemic, and there were many complications, both short-term and long-term, to those blood transfusions.

The use of EPO, which now in 90 percent of our dialysis patients has dramatically allowed them to return to the work force, as Erin told you told.

In our older patients, it's allowed them to attend religious services, babysit their grandkids.

They walk into the dialysis unit, which is a dramatic difference. Before, they were often wheeled in, or canes. They were just very fatigued.

It's a success story from the point of view of the Senate and the Congress. You have chosen to fund erythropoietin therapy through the Medicare acts that fund dialysis.

So it's sort of a all-the-way-around win story. And we thank you.

Senator Mack. Thank you very much.

Dr. Einhorn, we thought you were going to have a partner this morning. And Lance may or may not make it.

But, in any event, we're delighted that you're here. You might give us some insight on his experiences and your observations.

STATEMENT OF DR. LARRY EINHORN, LANCE ARMSTRONG'S PHYSICIAN

Dr. Einhorn. Yes. Thank you very much and it's an honor and pleasure to be here and talking before this Committee.

As mentioned, I thought I would be on the tandem bicycle riding behind Lance over here. We're talking about biotechnology, but commercial airlines are not high-tech, obviously, with the delays with Lance trying to get into Reagan National Airport over here.

Lance Armstrong is a story of hope and inspiration, not just for himself and winning the Tour de France, but what can be done in cancer research. And particularly his disease, testicular cancer.

Lance was treated at my institution, Indiana University, and we've had the honor and privilege of treating patients from all over the world with testicular cancer.

One of our cured patients 25 years ago, when platinum was an experimental drug, the drug that revolutionized the cure rate in this disease, is a former U.S. Congressman.

And so, this is a disease that attacks all walks of life.

Testicular cancer is the most common cancer that young men between the ages of 15 and 35 get. And it strikes them at the prime of their life.

Prior to the discovery and usage of platinum and further advances made in testicular cancer, this disease was a death sentence.

The average age was 25. After disease had spread to the lungs, which is a common scenario, the average survival would be six months. The five-year survival would be less than five percent.

Today, when cancer spreads to the lungs, as it did in Lance Armstrong's situation, the cure rate is 80 percent.

One of the most dramatic advances in any field of medicine, taking a previously uniformly lethal disease into a story of hope and courage and determination.

I was struck by some of the comments that you made, Senator Mack, about looking at biotechnology experiments and those experiments that don't work, not necessarily being viewed as failures.

There's a nice example of this with Lance Armstrong.

Lance came to us as a world-class professional athlete. The standard traditional cure that we use to treat these young men includes a

drug that can lower your lung function, something that would not affect you or I or the average weekend warrior, but something that would greatly impact upon a world-class athlete.

So we gave Lance an option—to take a more toxic drug that would produce more nausea and vomiting, more fatigue on the short-term basis, while he was getting his 12 weeks of chemotherapy, but a drug that would have no long-term effect.

This is an experiment that we had done, testing this drug against the standard drug.

We found, disappointingly, that the experimental more toxic drug did not produce better results. It produced equivalent results.

But it allowed us, despite this negative experiment, to give him an option that allowed him to tough it out on the short-term basis, but still compete as a world-class athlete.

I have no doubt in my mind that Lance would not have won the Tour de France if he had to take the standard drug that would have slightly impaired his lung function.

The final comment that I would like to make: Representative Stark talked about the Medicare recipients' cupboard being bare.

One of the problems that I face as an oncologist is new drugs are developed by the biotechnology industry that are exciting and hopefully, capable of prolonging life and quality of life.

Yet, we are not able to use them because it is not covered by insurance. Clinical trials are viewed as experimental therapy.

Let me make it perfectly clear.

Lance Armstrong received platinum in 1996. If Lance Armstrong was diagnosed the year that platinum was an experimental drug, we would not have been allowed to treat him because his insurance would not have covered the cost of an experimental drug.

Lance's story is a story of hope and courage. Testicular cancer is the story of hope and courage.

If we can do this in testicular cancer, and we have, we'll do this in other dread diseases because cancer is the scourge of this century.

We don't want it to be the scourge of the next century.

Thank you very much for your attention.

Senator Mack. Dr. Einhorn, thank you very much for your comments.

There are all kinds of different questions, both technical and economic, that are going through my mind. But what strikes me, what I really want to question Ms. London, Ms. Fagan and Ms. Fortier about, is another element to all of us, and that is the human spirit.

As I listened to your different stories, I couldn't help but be captured by that will to live, the will to overcome.

Clearly, that has to play a role in an individual's recovery. I wonder if you want to take a moment and just react to that, any of you.

Ms. London?

Ms. London. I'd like to react to that.

As I was writing my testimony, I thought about how to make it uplifting, because I consider that I've had a wonderful life.

I think it's all in attitude. And if you think, I can do it, instead of I can't do it, or these are the things that I can still do, instead of all the things I cannot do, it helps to go on day by day.

Ms. Boyer-Fortier. Senator Mack, I agree.

I found that the love and support of friends and family have made the difference, and the prayers of everyone.

But I think if you look at life optimistically and know that every day that you have here is a day to treasure, it makes a difference, because I saw quite a bit of difference and pessimism when you go into that chemo room every week.

The people who aren't here are the ones who were negative and discouraged right from the beginning.

So I think, without question, if we all can have that uplifting spirit and the support of our friends and family, it makes all the difference in the world.

Senator Mack. Ms. Fagan, do you want to add anything to that?

Ms. Fagan. No, I think they got it.

(Laughter)

Senator Mack. Okay.

Ms. London. I'd like to add something, though.

But we can't do it alone. We need the help of our physicians, which I've been very fortunate to have, and I'm very glad that this Committee today is addressing the issues because there are so many social services that are necessary, so many helpers and care-givers.

At this point in my recovery, there's an occupational therapist, a physical therapist, a primary nurse, and a social worker involved in my case.

All of these people are part of a team. And my family, of course.

And so, we can't do it alone. And that's why I'm glad your Committee is addressing these issues. And there are bills before Congress to address these issues.

Senator Mack. Before I recognize Lance Armstrong, let me just say again, I was struck by the spirit that each of you expressed, the desire to live, to overcome.

It did remind me, however, of my brother Michael, who died of melanoma at the age of 35, diagnosed about the age of 23, in his last year of law school at the University of Florida.

He had to work a little extra hard that year so that he could graduate number one in his class and beat his older brother, who had graduated number two in his class with honors.

Michael had to do a little bit better.

But he fought those 12 years. He just would not give up. Some people would say it was denial. But I don't think it was denial at all.

It was the will to live, the will to overcome. And I certainly recognize that in the comments that you all made this morning.

Lance, we welcome you this morning. We've heard words like inspiration and hope and courage.

Frankly, most of us believe that you captured the attention of the entire world as a result of your feat—no pun intended.

(Laughter.)

Mr. Armstrong. It was legs, not feet.

(Laughter.)

Senator Mack. Right. But you have been an inspiration to us. And I appreciate your comment that it was obviously more important to win the fight against cancer than to win the Tour de France, we are moved by what you have done and we are delighted that you are here.

We look forward to your testimony.

STATEMENT OF LANCE ARMSTRONG, WINNER OF THE 1999 TOUR DE FRANCE AND TESTICULAR CANCER SURVIVOR

Mr. Armstrong. Thank you.

Mr. Chairman, members of the Committee, thank you for inviting me here to speak today on a subject that is near and dear to my heart and of importance to millions and millions of Americans.

I'm honored to not primarily be here as a professional cyclist, but as a cancer survivor, my proudest achievement in all of my life.

Simply put, I'm a living example of successful cancer research and the effect it can have on life or death.

I'm told that 20 years ago, I almost certainly would have died from testicular cancer that invaded by body.

In October of '96, I arrived to my doctor with widespread pulmonary metastasises and was later found to have had two tumors on my brain.

My serum tumor marker, a unique protein elaborated by cancer cells, was massively elevated at greater than 100,000, whereas the normal level for you and me today is two, or less than two.

Because of research, in part funded by the Federal Government, Dr. Larry Einhorn, seated here to my right, was able to create a successful therapy regime based on the use of platinum and other drugs which saved my life.

I am deeply grateful for Dr. Einhorn's research and for the Government's support of it. Without it, there would be no Tour de France wins or forthcoming children in my life today.

As each of you knows, cancer is the second leading killer in America today—soon to be number one. But the advancements in the past 20 years give us all hope that we can beat this disease during our lifetime.

Government funding of quality research is a key component in meeting that goal and I ask you today to give that goal your full support.

If you do, you will save the lives of countless Americans just like me.

Since my diagnosis in October of '96, I started a private foundation in my name to raise money for cancer research and awareness.

I am proud to report to you that the Lance Armstrong Foundation has raised more than a million dollars and has poured that money back into scientifically viable research projects at institutions like M.D. Anderson in Houston and Vanderbilt University in Nashville.

But we can only do so much as a private foundation. We need the Federal Government's help as our partner in this fight. And it is my hope that you will consider a substantial increase to the current level of funding for cancer and biotechnology research.

In closing, let me again say thank you for having me and hopefully we can achieve some great things here today.

Senator Mack. Thank you very much for being here and thank you for your comments.

I'm just going to raise one additional question, which I will probably ask Lance. And then I'm going to turn to Mr. Stark and then to Senator Frist, and then back to Senator Kennedy, and back and forth.

And again, we will get into more technical questions a little bit later.

But Lance, you said that if you never had cancer, you would not have won the Tour de France.

I think it's been reported that you said that.

Mr. Armstrong. That's correct.

Senator Mack. That's an amazing statement. Tell us a little bit about that.

How were you able to keep the right attitude, overcome the cancer, and so forth?

Mr. Armstrong. I think a little bit—part of that answer is a physical one. But most of it is a psychological one.

Physically, my body changed through the illness and through the therapy. Before, I was a bigger rider.

This gets kind of technical, but I was a bigger rider. And through the illness, I lost all of my muscle and basically lost all of my athletic physique and had to build it back up from scratch.

It changed when it came back.

But I think that's less than ten percent of the equation. The big part of it is the psychological factor.

Since October of 1996, and all throughout 1997, when I was treated and recovering, I changed as a person and I changed as an athlete.

My outlook on life and my outlook on my sport and my outlook on winning completely changed.

The way that I was an aggressive, successful rider in the past, the way I came back and wanted to be much better and realized that I was given a second change, it changed everything for me.

So now when I have the opportunity to win bike races or be successful in sports, I'm stronger. I'm stronger in the mind and even stronger in the body.

Cancer does that to you. And I'm sorry that I missed out on part of this hearing, but I'm sure that we are all sharing these thoughts and our feelings on this.

We're different people. We're better people.

Early on in my illness, I got an e-mail from a guy—and this is one of the first e-mails I got. He said—he was in the military. He was based on Japan, I think.

He said, you don't realize it, but you're a lucky man. You're going to consider yourself lucky.

And I thought, this guy's crazy because this is not luck. But he's right. He's very right. I am a lucky man. I'm lucky to be alive and I'm glad that I went through it and I'm glad that I have this perspective that I have now.

Senator Mack. Well, again, thank you very much.

Pete?

Representative Stark. Thank you, Mr. Chairman. And I want to thank Mr. Armstrong, Ms. Fortier, Ms. Fagan, Ms. London, for taking the trouble and the effort—and I know it was trouble and effort—to come here and share with us your stories.

I would like to inquire of Doctors Bunning, Lewis, Shak, and Einhorn, in order, if they could relate to us how many times they have had to withhold or not be able to provide treatment to candidates because they neither had the funds or you didn't have the funds, because I know Senator Kennedy and I are trying to make sure that not only do the witnesses who are here today, but people who will be coming along, have an opportunity.

There is no question, Dr. Einhorn, that we would like to provide—and for me, just as an individual, there's no reason that I can think of why this country doesn't.

I suspect we are the only industrialized nation that does not. And people can make a lot of comment.

But would you mind, the physicians in order—do you have cases where you can't provide?

Dr. Bunning. If I speak just of rheumatoid arthritis and with regard to the two new drugs that have been approved in the last year, I'd say there's probably ten to 20 patients I haven't been able to give it to. And there's about 3000 rheumatologists in the country that probably have similar stories.

So the math would be, what, 30,000 patients possibly haven't received these medicines.

Representative Stark. Dr. Lewis?

Dr. Breyer-Lewis. When the clinical trials finished, Medicare covered erythropoietin to be given to hemodialysis patients in in-center dialysis, which meant kind of ironically, and I think it was just almost a clerical mistake, that home patients, patients who in fact were taking more responsibility for their care and spending less health care dollars, didn't receive this drug, like peritoneal dialysis patients.

Representative Stark. They don't get EPO at home treatment?

Dr. Breyer-Lewis. They do now.

Representative Stark. They do now, yes.

Dr. Breyer-Lewis. But we went through a period of years where there was just the chemo patients.

Representative Stark. Is there any reason we shouldn't have other super-drugs paid for?

EPO happens to be because of the relationship of the disease. The Federal Government in effect buys about all the EPO that Amgen can sell.

But I see no reason why we shouldn't provide drugs for arthritis or drugs for breast cancer, drugs for testicular cancer, in the same manner.

Do you?

Dr. Breyer-Lewis. Well, yes, Representative Stark. I, in most of my talks, I do a lot of lecturing, and end it with the testimony of the human spirit that my patients provide for me.

It tells me that my responsibility of being blessed with good health is to ease their burden.

And I think, actually, there are so many things that we don't have a way to help, to have things that we can help and not provide them, we're not doing our jobs.

We need to provide them.

Representative Stark. Dr. Shak?

Dr. Shak. As a company, we've had to address this issue for the past 15 years.

And for all of our products, we have had in place a policy that provide our drugs free to all patients who have no ability to pay.

Representative Stark. And you are supporting, I understand, as an industry, some kind of more universal drug coverage.

Dr. Shak. Yes, we are.

Representative Stark. For which we thank you.

Dr. Shak. And goes further to actually look at the total burden of costs and to provide help for those individuals for whom the copayments are excessive.

Representative Stark. Thank you.

Dr. Einhorn. It's very sad when a young person dies prematurely from cancer. But it's catastrophic when you have a promising treatment that you're not allowed to give to that patient.

What happens in the real world when patients come to a hospital and a new drug is just being used that looks very promising in the laboratory, we have to figure out how we're going to pay for that because the insurance industry views experimental therapy oftentimes not just not covering the cost of the drug, which is free, but not covering cost related to any component of the treatment whatsoever.

We have to learn to educate the insurance industry that clinical trials are the only way that progress will ever be made in this disease.

We still see patients who are denied commercially-available standard treatment.

The biotechnology industry provides the drug free in those situations for indigent patients. But we still have to figure out how to cover hospitalization for complications like pneumonia, infections, the use of fluids for nausea and vomiting from the chemotherapy, et cetera, et cetera, et cetera.

We clearly have patients who are denied and we're not able to provide what we feel is the optimal treatment for their diseases.

Senator Mack. Senator Frist?

Senator Frist. Thank you.

Dr. Breyer-Lewis, could you comment on the role of clinical trials? In your opening statement, you pointed out very clearly the importance of having this whole process of Federal investment. Walking us through the beginnings where we see Ms. Fagan and the really miraculous results.

You've been involved in numerous clinical trials over the years and see both the pluses and minuses.

What advice do you have for us in terms of looking at clinical trials and their role from a policy standpoint in the future?

Dr. Breyer-Lewis. I think that it's critical that our wonderful basic research gets translated into helping patients.

In the end, the mission of that work is to help our patients in our society. And that just can't happen unless there are clinical trials.

And I echo Dr. Einhorn's comments that we're faced with increasing challenges in conducting clinical trials now because of the insurance companies and other agencies' unwillingness to support other aspects of their care.

Even when a patient is in a clinical trial that covers all the costs of the trial itself, again, if they get hospitalized or they have a problem that the insurance company deems related to it, there are no funds for that.

And the universities and the academic centers' ability to shoulder the burden of those kinds of unpaid costs is limited, and becoming even less able.

So I think that we need the ability to translate science into things that help patients.

And the only way to do it is clinical trials.

Senator Frist. Comments by other panelists on role of clinical trials? How much the government can afford to invest in clinical trials has to be weighed against all, whether it's reimbursement for particular agents or research, broad basic science research.

It's a trade-off there.

Any other comments or advice that you might have on clinical trials and the Federal Government's approach?

Dr. Bunning. I'd just say briefly that performing the clinical trials is an industry, too.

I know if it's difficult to do the trials here for various reasons, the companies will take them elsewhere.

One of the two drugs that has been approved for rheumatoid arthritis, Arava, I know the Phase 2 trials were done I believe in Czechoslovakia because they felt it was easier to accomplish the trial there.

So this is an industry that I think we'd like to support and keep in our country as we've done a great job on it so far.

Senator Frist. Dr. Einhorn?

Dr. Einhorn. I'm sorry. I was just going to point out that for Medicare recipients, for insurance beneficiaries, that the cost of clinical trials is basically very similar to the cost of standard therapy.

If a patient gets standard therapy that we know to be inferior, it's covered completely.

If we want to use an equal cost experimental therapy, it's not covered at all.

Senator Frist. Ms. Fagan, just one final question. As for quality of life before and afterwards, can you just comment on that?

You've been on dialysis for how long?

Ms. Fagan. Well, the second time around, since 1993.

Senator Frist. And that's peritoneal dialysis?

Ms. Fagan. Yes.

Senator Frist. Which means that you are on it eight or nine hours a night?

Ms. Fagan. Ten hours.

Senator Frist. Ten hours a night every night.

Ms. Fagan. Yes.

Senator Frist. When did you start EPO? How long ago was that?

Ms. Fagan. April?

Dr. Breyer-Lewis. Last spring.

Ms. Fagan. Last spring.

Senator Frist. And compared to two years ago, quality of life is better?

Ms. Fagan. Well, I have a lot more energy now. When I first moved up here, I had a few setbacks that caused me to start becoming anemic.

I was constantly tired. And I was only working a part-time job at that time.

And then when I started working full-time for the state, it just became increasingly harder to actually carry out one full day's work.

They kept drawing my blood and kept telling me I'm anemic. So they told me about the clinical trials going on, starting up, and they asked me if I wanted to be a part of it.

And I told them, yes, please.

But I know what it's like being anemic and having energy now and I'd much prefer having the energy.

Senator Frist. Had you been in any other clinical trials before?

Ms. Fagan. No, this was the first.

Senator Frist. Was it a randomized clinical trial?

Dr. Breyer-Lewis. Yes. We didn't mention that part, but Erin is looking at a trial that is to decrease the number of injections of EPO that are required in patients that take it at home.

It is a randomized clinical trial.

Senator Frist. Thank you very much.

Thank you, Mr. Chairman.

Senator Mack. Senator Kennedy.

OPENING STATEMENT OF SENATOR EDWARD M. KENNEDY

Senator Kennedy. Thank you.

First of all, I want to thank all of our witnesses, particularly the patients. It's difficult for them to talk about their personal experiences.

Perhaps we've listened to this over a period of years and we begin to take it for granted. But I think they really serve an enormously important purpose.

The best way we can really ever thank you is to do something about the message that you give us, number one.

Number two, I want to thank the Chairman for having these hearings.

This is the Joint Economic Committee. As much as Congressman Stark and I work on health, Bill Frist and others, this is the Joint Economic Committee.

I can remember talking with the Chairman when they were looking at the responsibilities of this Committee in terms of looking down the road to the future in the area of biotechnology.

I think there's a great deal of interest, as there appropriately should be, in telecommunications and software and the Internet.

But biotechnology is right up there. For many of us who are interested in progress in those areas realize that this next century is really the century of the life sciences.

This is really where it's at. Its intersection in terms of people and people's lives to me, is really a compelling one.

We have an extraordinary group of people, Mr. Chairman. Arthur Ullian, I've known for a long period of time, is talking about the intersection of biotechnology and industry and the changes that it's had.

It's very profound.

And I see Henry Termeer and Carl Rausch, who are heads of two very, very interesting and important companies.

In my own state, Peter Lynch can talk about investments and where we're going over a long period of time as a nation, as one of our pre-eminent investors.

And Professor Sager, here from Boston University in the area of health sciences.

So this is really—these are extraordinary individuals. I know them well from my own state. They are really the state's men and women of this industry.

I was thinking as we were listening to the answers and listening to this, the fact is, with these breakthroughs, we can have the opportunity to save money on health care.

We think eight or nine cents out of every dollar goes to drugs. It's going to go up to 15 cents. It may go to 20 cents.

But the possibilities of savings in hospitalization or savings in terms of other areas, is profound. It's significant.

We're not dealing with it. We're not dealing with it here in the Congress. That's the kind of issue that we ought to be thinking about in terms of health care. And our responsibilities.

Perhaps not in the Joint Economic Committee. Perhaps in the Joint Economic Committee.

But then when we have an opportunity to look at these, I think the compelling case that's to be made has even additional kinds of persuasion.

I find it persuasive myself now that the guarantee of Medicare is to try and relieve people, particularly elderly people, obviously in terms of their future anxieties, and men and women that serve the country well.

But we left out prescription drugs. Now we know the opportunities that are there and we're reminded about it again.

So I think that the whole lessons of these hearings, Mr. Chairman, are very, very profound.

It's really looking into the future. We have, too infrequently, as committees, really done this. And I look forward to working with you and others, as we try and put these prescription drugs and these catastrophic

drugs which are so essential, making both available and encouraging them, with the kind of investment that you have led in a bipartisan way in terms of NIH research and the R&D credit, all those things make sense.

But we cannot listen to this panel and not know what is out there and the opportunities that are out there.

And I thank Ms. London.

We have made the point about the fictitious Flo and the real Flo. And we've seen the fictitious Flo on television.

We've got the real Flo here. And we thank you very much. It's difficult.

One of my dearest friends who had rheumatoid arthritis had 56 operations.

He just won't quit, like you. He's got an indominatable spirit.

So we thank you very much and I thank the Chair. I'll look forward to getting back here and asking questions of some of the other witnesses.

I thank the Chair.

Senator Mack. Thank you, Senator Kennedy.

Senator Bennett?

OPENING STATEMENT OF SENATOR ROBERT F. BENNETT

Senator Bennett. Thank you, Mr. Chairman. Thank you for the hearing.

Like my colleagues, I want to thank all the witnesses for their candor and their openness.

I used to work for arguably the most eccentric man in the United States. His name was Howard Hughes.

A great deal has been written about him.

One of my assignments was to develop the authorized biography of Howard Hughes, which, given the culture of the Hughes organization, never saw the light of day and probably never was destined to, even at the time I was given the assignment.

Mr. Hughes died without telling us where he put the will.

(Laughter.)

And a lot of folks in Hollywood and elsewhere tried to create wills for him.

But the legacy of Howard Hughes—he created TWA. He created a number of other things—is the Howard Hughes Medical Institute.

And if we had found the will, we would have known that his entire fortune was to be left for medical research.

As it turned out, if I may get autobiographical for just a minute, I lobbied the Congress on behalf of Mr. Hughes to keep a very anxious Congress from destroying the stock relationship between Hughes Aircraft and the Howard Hughes Medical Institute.

We took a considerable amount of abuse for that, but we succeeded. And as a consequence of that, when the Hughes Aircraft was finally sold to General Motors for roughly $7 billion, that amount of money endowed the Howard Hughes Medical Institute with that $7 billion that is going solely for medical research.

And as I say, if we could have found the will, all of the casinos and airline and so on in Nevada would have gone to medical research as well.

The thing I learned in dealing with the Howard Hughes Medical Institute and the amount of money that was going into medical research, and have learned again with another very successful business man of my acquaintance—he's not quite in the Howard Hughes category, but he's considerably more rational—that's John Huntsman, out in Utah, head of the Huntsman Chemical Company, who has just opened a $100 million Huntsman Cancer Center at the University of Utah and told me he's prepared to, he and his family are prepared to put $15 million per year into that facility for a minimum of 20 years, asking if we can get some federal matching funds to try to help that out.

Now I've gone through that facility and recognize the tremendous advances that are being made in cancer research there.

The thing I've learned from all of this exposure, Mr. Chairman, and I'd like any comment from any of the researchers who are here, is that medical breakthroughs are a combination of very hard work and discipline and blind luck.

That very often, the breakthrough occurs when one researcher casually mentions something to another researcher. Some of the best breakthroughs are some of the cheapest because the blind luck happens early in the process.

The most important thing is the attitude and atmosphere of the researchers to explore virtually anything and go virtually any place.

And that one of the most damaging things to research is when some kind of outside agenda is imposed on the researchers.

I'll share with you one thing from the Howard Hughes experience.

The Hughes Medical Institute early on refused to give grants in the traditional way because those who administered that fund, and Mr. Hughes did not himself, said there are—here's the dreaded word—there are political implications in medical research just like every place else.

People maneuver for power and position.

The thing that the Howard Hughes Medical Institute does is fund fellows who work at institutions like the University of Utah or Harvard or wherever all else they are. And as such, they are not employees of the university and therefore, have a degree of independence and can do more freelancing, if you will.

And many, many breakthroughs have come as a result of the work of the Howard Hughes fellows.

The University of Utah, the Huntsman Cancer Institute, the thing that impressed me the most on the tour was the way it was laid out physically.

It was laid out so that all of the researchers were in the same room and there were no cubicles. There were no Dilbert cubicles to keep them from talking to each other.

And the fellow who took me through the tour, who had come to the University of Utah from what he considered to be the leading cancer institute in London, saying I would have a better opportunity at Utah, said, this is fabulous, that you just walk up and down the aisles and people are running into each other with their ideas and their conversations.

And the idea is this is more important, really, than the money, is the kind of culture that has been created there, even though the amount of money is enormous.

Does anyone have any corrections for me on these observations?

I'm here to learn. I don't have any specific questions. I'm just sharing with you this background. I'd be happy for any responses you might have.

Dr. Shak?

Dr. Shak. I would certainly like to build on your comments from a very personal perspective.

Clearly, what's special of biotechnology is not just the technology, that we can do things that we never could do before, but is also the entrepreneurial spirit and the spirit of innovation.

I conceived of Pulmozyme, the first new drug developed for cystic fibrosis in more than 30 years while at Genentech in 1988.

Why did it take 30 years to come up with a drug for that dreaded disease?

I think one was the technology without biotechnology. This kind of approach could never have been made practical.

But the second thing was the environment at Genentech.

Each scientist is given some of their time to be free to pursue their own interests.

And when I came up with the idea for this enzyme, to chop up the secretions in the lungs and to help kids to breathe, I was told to drop everything and to go after it.

So, indeed, I think it is correct that the culture is critical and that investment in that culture and in research is the only way to develop these new breakthroughs.

Senator Bennett. Thank you, Mr. Chairman.

Senator Mack. Thank you.

Congressman Minge?

OPENING STATEMENT OF REPRESENTATIVE DAVID MINGE

Representative Minge. Thank you very much.

We're honored that each of you has come this morning to testify.

Lance, I'm particularly honored that you're here. I use a bike to reach my destination each day for the last 50 years. This morning was no exception.

But I only had to come one mile, as opposed to the distance that you came.

I didn't have all the complications.

(Laughter.)

I'd like to just make a couple of comments on my own and then turn some of these over to you as witnesses.

I come from a state that has a deep and long, rich tradition of medical research and manufacturing.

In Minnesota, we have 800 firms and 20,000 employees making medical devices. And it's second only to California, which does seem to exceed us in population, but we think not in much else. Maybe area.

(Laughter.)

But our commitment in this respect parallels the commitment of the country to try to make sure that the quality of life for people that live here in the United States is evenly distributed, and that those that are afflicted by medical conditions are included in a constant effort to improve and renew what it means to live in America.

Within my own family, my wife was diagnosed with breast cancer. Fortunately, she was not Stage 3. She did not go through quite the same turmoil that you did.

But nonetheless, we did go through chemotherapy and radiation and hair loss and all of the struggle that a person diagnosed with cancer and trying to work through the treatment has to endure.

And I agree, Mr. Armstrong, that one is stronger quite often for having worked through that and made that commitment and survived.

But I recognize from the hundreds of letters that I've had in my office, and talking to family members and friends who are physicians, the frustration of how do we afford this in our society?

And I'd like to ask if there is any way that you as physicians can quantify the cost that we face and then, secondly, any suggestion you have as to how we approach financing it.

Dr. Bunning, I was particularly taken with your comment that you have—did you say 20 patients? Is that per year?

What percentage is that—in this category where you know that there is an effective treatment out there, but the cost is somehow not covered by insurance or whatever program they may participate in.

Dr. Bunning. Well, speaking of the two new drugs that are available, which have been available for about a year and I've accumulated, say ten to 20 in my practice—what percent of my practice that is—it's probably five to ten percent of the patients.

Representative Minge. And if you then include other categories of patients that you have and problems they experience in financing the treatment that's necessary, either for quality of life or just survival.

Dr. Bunning. There are other drugs, there have been other advances in arthritis in the last year or two. We weren't talking about it today, but there's something called the Cox 2 selective ensays, which are basically generalized arthritis pills that work for osteoarthritis.

These have been approved. They're a significant advance. They cause less stomach irritation than the previously available medications for osteoarthritis.

And I've had a lot of trouble getting those approved in the District because D.C. Medicaid won't allow it with just a prescription. You have to go through a lot of hoops to get that.

And again, it's also—

Representative Minge. So would you say maybe 15 percent or 25 percent of your patient mix would end up having—

Dr. Bunning. Trouble getting the medicines? Maybe ten to 20 percent have trouble getting the medicines that are available.

Representative Minge. Would that square with the experience that the rest of you have had?

Dr. Lewis?

Dr. Breyer-Lewis. Yes. But I'd also like to expand on that a little bit.

Diabetes is the most common cause of chronic renal failure in this country. There are 135 million diabetics world-wide currently.

Again, basic research funded by the NIH suggested that a certain kind of drug would slow or prevent patients reaching dialysis.

We conducted a clinical trial that proved that to be the case.

In a simplistic way, if you were two years away from dialysis, taking this drug would make you four years. If you were ten years, you'd be 20 years away.

We then, following the conduct of the clinical trial, we actually engaged the services of an accounting firm to assess the health care dollar impact of that intervention.

And you have to remember that when you save the lives and prolong the lives of chronically ill patients, those patients go on to use up more health care dollars.

That's just an economic fact.

We found that this intervention, which again can be applied to half of the 135 million diabetics in this world, not only saved their lives and kept them off dialysis, an incredible quality of life, prolonged their lives, but saved health care dollars.

And so it isn't always the case, although it is sometimes the case that extraordinary amounts of money need to be spent on small numbers of patients and ends up costing you money.

It is often the case that these interventions can be applied to large numbers of patients and save you health care dollars in the end.

I think that's a really important thing to keep in mind as you approach biotechnology funding.

Representative Minge. Thank you. I see both the red light is on and there's a vote that's been called on the House side.

So I will have to cut my comments short.

But I am interested in the other physicians' views and hopefully, I'll have an opportunity to follow up on that.

And secondly, any suggestions you have as to how we as a society pay for this—should we simply say that we have unprecedented wealth in America and this is a part of what it means, our responsibilities to our neighbors in this setting?

And what does it mean, Mr. Chairman, in terms of the programs that we've constructed?

We have in my area of the country hundreds of thousands of people enrolled in government programs who do not qualify for prescription drugs.

But those that are more affluent and have the opportunity to go to Florida for part of the winter, and they learn that if they only lived in Florida, the so-called AAPCC rate would qualify them for prescription

drugs, for hearing aids, for eyeglasses, and even transportation to the doctor's office, and they can't figure out why they can't get it in Minnesota.

So we've got some things I know that we've got to work through, especially in the Medicare program.

Senator Mack. Sounds like a lot more people are going to be moving to Florida.

(Laughter.)

But, anyway—

Representative Minge. Thank you very much, Mr. Chairman, for holding these hearings and thank you again for coming as witnesses.

Senator Mack. Senator Sessions?

OPENING STATEMENT OF SENATOR JEFF SESSIONS

Senator Sessions. Thank you, Chairman Mack.

This is, as has been noted, interesting that we have such a panel as part of the Joint Economic Committee.

First and foremost, you as physicians and those of you who have suffered from diseases, know that patients' primary goals are to achieve a high level of functioning and quality of life.

The research that so many of you have been involved in does help us as a nation, as part of our strength as a nation, and the entire world, really, in an extraordinary way.

I share with you, Connie, your commitment to doubling the funding for NIH in the next five years. We've made some progress towards that already.

I believe that is an outstanding investment.

I guess I'm thinking here as you, Dr. Einhorn and Dr. Shak and Dr. Breyer-Lewis, and I guess Dr. Bunning, too, had indicated that there are on occasion procedures, therapies that are available that would actually help the patient's quality of life and that would actually be less expensive and save money.

Sometimes that's not approved readily.

Where are those problems?

I think you mentioned, Dr. Bunning, the Medicaid agency here. There's HCFA. There's private insurance companies that resist new procedures.

Where are the bottlenecks and how can we as members of Congress help eliminate them?

Do any of you want to comment on that?

Ms. London?

Ms. London. In my personal case, I have a Medigap policy and I have drug prescriptions partially covered under the Medigap policy. But it does not cover these new drugs.

I called last week and asked why and I was told that because one of the new drugs is self-injectable, it's not covered.

They also don't cover another drug Dr. Bunning prescribed for me, which is a nasal inhalant, which helps bones grow.

For whatever reason, they've just decided on their formulary list to have pills covered. And that's only certain pills.

At one point Dr. Bunning prescribed an antibiotic. They said, no, that's not on our list.

So the pharmacist at Dr. Bunning's hospital, the National Rehabilitation Hospital, had to find another drug. Dr. Bunning could address whether or not that antibiotic was as good.

It certainly wasn't his first choice.

So I can tell you that a bottleneck, at least in my case, is through some of the Medigap very limited drug policies.

Also, it's not a copayment policy. It only pays 40 percent of the retail cost of a drug in most cases. And I can't always get it through a regular drugstore.

So if I run out, I can't go down the street to Rite Aid. I have to get it through their mail order.

Arava, one of the drugs Dr. Bunning talked about, costs $210.08 a month through this limited policy that I have. And the Immunex drug is not covered at all.

So that's one bottleneck.

Senator Sessions. So you would say that some of those decisions are not rational.

Ms. London. I would say, in my opinion and in my experience.

Senator Sessions. Any other comments on how those decision are made, particularly when you've got a procedure that would appear to be in the long run less expensive?

How do we get into these problems?

Dr. Einhorn?

Dr. Einhorn. Well, it's unfortunately illogical to think that logic prevails in the health care industry.

(Laughter.)

It's a sad event for health care providers and patients.

To give you some examples, with Erythropoietin, EPO, which we also use for cancer patients, as well as chronic kidney failure patients, if a patient self-administers a drug, and it's very easy to self-administer, much like insulin for a diabetic, it's not covered.

So they have to go to a more expensive physician's office on a daily basis, in many insurance coverages, including Medicare, in order to get reimbursed for the drug.

If we find an equivalent cancer drug that can be given orally, which is far less expensive for the health care system, Medicare doesn't cover oral administration of some chemotherapy drugs.

And so, we're forced to give more expensive intravenous drugs, which are covered.

It's an illogical type of system.

I think everyone on this panel, and perhaps everyone in Congress, has their own agenda, and I'm no different than anybody else.

But when we ask, how can the country afford, I would ask, how can we not afford?

Senator Sessions. Is there a procedure—where would you make the protest?

Dr. Einhorn. Where do you draw the line in the sand?

Senator Sessions. Who would you complain to? What is the central office that makes those decisions that you could make an appeal to to make this a logical case about?

Dr. Einhorn. Well, that's the amazing thing. It's a little bit like when I was growing up and the war of my generation, the Vietnam War, you never really knew who was in charge and what was going on.

And the same thing is true with the health care industry.

You don't have an idea of who to call, who to contact. You call one person and you get sent to another person, to another person.

And as a patient, and I'm sure all the patients here can attest to this, you finally shrug your shoulders and say, I give up. I can't do this any more.

I'm just going to not take this drug or pay for it myself.

As a physician, it takes an enormous amount of infrastructure in my office just to get claims settled so that we can treat patients with standard, FDA-approved drugs.

It's an insane system that we have right now.

Ms. Boyer-Fortier. Senator Sessions, similar to Ms. London, I am also now a Medicare patient.

One of the things we have is an anxiety come on July 1st every year, when you wait to hear whether your Medicare managed care plan is going to continue to operate in your region.

And of course, because I'm not eligible for Medigap, so I don't have a Medigap policy, I am very fortunate that there is a Medicare HMO carrier here in the region, in the District of Columbia where I live.

And so, I have been getting care.

But it is true. The last antibiotic I was prescribed, they wouldn't pay for. We'd call the doctor. We have to get another one. But it wasn't the doctor's first choice.

But at least I have some prescription coverage.

But come next year, if the Medicare carrier does not decide to continue to stay in this region—and everyday in the paper, you read that HCFA is saying that the Medicare carriers are paid too much. And the Medicare carriers are saying that they're not paid enough.

I actually don't know the answer. All I know is that if I don't have one, there is no way that I could afford without some kind of coverage any of the treatment that I receive on a weekly basis.

And because I still have a lot of disease progression, I will always be on treatment.

I am not in a cure situation.

And so, we've got to do something to deal with the Medicare coverage that we have in this situation.

Senator Sessions. Thank you.

Dr. Breyer-Lewis. I would like to reiterate Dr. Einhorn's comment.

There is no doubt that the infrastructure required in my office now to go through the bureaucracy of figuring out every insurance company, every Medicare carrier—we have Tencare in the State of Tennessee—has a different formulary. The formulary changes monthly.

You almost hesitate to write a prescription because the odds are you're going to get a call back from the pharmacist that that one isn't covered.

These formularies are not being constructed because someone is deciding what are the very best medications for the patient in our health care plan.

These formularies are constructed and they're changing on a monthly or a quarterly basis based on price of drugs and what they're willing to invest in the price of a drug.

The infrastructure costs in our offices aren't added into the health care system. But I promise you, they're an enormous cost. And I suspect

that they're higher than whatever they're saving on the two-cents cheaper antibiotic they switched to on the formulary.

This is an enormous problem.

I think that some—I guess it's your responsibility and I don't envy you it. But the government has to take a role in this. They're going to have to partner with industry in a creative way to solve this problem because the current way it's working isn't working for our patients.

Senator Mack. Again, I thank all of you for your both participation and your comments.

Before I conclude, Arthur Ullian, we'll get to you in just a moment. I appreciate your patience. We're running a little bit behind.

But there is one question that I want to put to Lance, and then I want to make kind of a concluding comment with respect to our discussions.

One of things, Lance, that my wife and I have been actively doing over the years is trying to get out the message of early detection.

I was wondering whether you had any comment with respect to how early you detected your cancer.

Were you aware of changes that were taking place that maybe you should have reacted to earlier? What effect did that have on you?

Mr. Armstrong. Well, my situation—I was almost—I waited as long as I possibly could.

Dr. Einhorn can tell you more because he's seen thousands and thousands of patients.

But I ignored symptoms for months and months and months. Towards the end, they were really extreme and very obvious that I still ignored.

I think we all hate to hear that we're sick. We all hate to go to the doctor—don't hate, but we don't like to go to the doctor.

I wasn't nervous about being a young man with a sensitive issue. That didn't really bother me.

But I was nervous about being a professional athlete and the possibility of losing my career, or being told that I was sick.

So I sort of put that off. I had very extreme headaches and coughing up of blood and blurred vision. This went on and on and on and I ignored all of that.

I thought I had a hard work-out.

Senator Mack. That is a hard work-out.

(Laughter.)

Mr. Armstrong. It's funny now, but at the time, to answer your question, I waited a long time, probably to the 99th percentile.

Had I waited any longer, I don't know if I'd be here now.

Senator Mack. What would you say to men that might be going through the same thing you did?

Mr. Armstrong. Well, that's where we've come so far with an illness like this, especially to awareness and early detection. And even in all cancers, if it's testicular or prostate or breast or colon.

If we can get the message out and teach people what to look for and hopefully provide them with living examples of a cure and life after cancer, then I think that they won't be so hesitant to go to the doctor that first time and get that check-up.

These aren't pleasant. If it's a young man with testicular cancer or a man over 50 going in for prostate screening or colon cancer screening, people dread those things.

But we're talking about life and death here.

So much of what I try to tell people is go get checked out. If you think you have a problem, it's real simple. Just go to your local doctor.

And I think, like Dr. Einhorn said, we have to fund these things. We can use all the cliches and analogies and examples that we've all heard before, and we can have our local charities, our regional charities, or even national charities.

But we want to raise millions of those things. And the key isn't the M word. The key is the B word, and that's billions.

We need, on all levels, on all illnesses, we need to step it up.

I can only speak as a cancer patient and a cancer survivor. It's a deadly, deadly illness. It's almost become an old story.

In 1971, President Nixon said that we were going to cure cancer in ten years.

Well, it's almost the year 2000 and we've had some success on some fronts, but, by and large, the illness is still incurable.

That gets frustrating for people, I know. Like I said, it becomes almost an old story and people—a 70-year-old dies of cancer and they think, well, they just died a natural death. But that's not the case.

We should do more. It's there.

Senator Mack. Well, again, thank you for those comments. I think that's very helpful. I think it reflects probably the attitude of the panel in general.

And again, I thank all of you.

I'm sure for all of you who are listening to this discussion, you can't help but notice the tension that exists. However, there should be no reason in a free society that we don't provide complete medical attention

and treatment, from the surgical to the pharmaceutical for everyone in America.

But I would just say to each of you also that there is a consequence to how we do it.

Dr. Bunning said for example that there are people that come from all over the world. It's not just in your area of specialty, but in so many of the areas of medicine in the United States.

We are the leaders.

So, in some respects, you have to ask yourself, why is that?

We know that a huge percentage of the pharmaceuticals and the biotech that's being created today is happening only in America.

And why is that?

So we've got be very careful about how we try to solve this problem. There's no question in my mind that Medicare and other insurance programs have to include prescription drugs. That's the way we treat people today, not the way we did back in the '60s.

So recognize that there is a tension. I would say that, really, neither side's only interest is to go after the other in making a political point.

And I don't accept the notion that because I am concerned about making sure we keep an environment where we in fact can create the drugs of the future, that I am somehow opposed to finding a way to provide those drugs to those who need them.

So that's the tension that existed this morning. I thought it was very helpful to have it.

Again, I apologize to the other panelists for running a little bit later. Thank you for coming.

This panel is now adjourned. Thank you very much.

(Pause.)

If I could get you to take your seats.

(Pause.)

Again, if you all would take your seats, please.

I want to welcome Arthur Ullian to the podium. It is a distinct honor and pleasure to have you here this morning.

You have been a tremendous advocate for patients of all diseases, committed to the effort to double NIH.

You've been a great spokesman for the issues that we all have a concern about.

And so, I welcome you to the Committee this morning and look forward to your testimony.

PRESENTATION

STATEMENT OF ARTHUR D. ULLIAN, CHAIRMAN, TASK FORCE ON SCIENCE, HEALTH CARE AND THE ECONOMY

Mr. Ullian. I must say that I have great admiration for you and your leadership. And also for the inspiration to put this together.

It's a very different kind of hearing. As all the other members have mentioned, bringing all of the economics into it, as well as the patients.

We really need to look at health care, not as kind of a luxury, but as you'll see as the day unfolds, it's really part of an important progress of moving forward.

I must say that your leadership really requires that you don't step down and retire.

It's boring out here.

Senator Mack. I don't believe that somehow.

(Laughter.)

Mr. Ullian. We've heard from the first panel about the wonderful therapies and cure that our researchers are finding and our hospitals are delivering at an ever increasing pace.

We are moving away from so-called half-way medical technology towards less invasive therapies that let us treat diseases at the genetic and molecular levels before they ever become symptomatic.

With all of these examples and treatment that we've heard this morning, and the ones that we'll see this afternoon at the R&D fair in Dirksen, it's all the more surprising that Congress and the American people have come to view health care only as an expensive burden to the government as well as to the consumer, and not as the benefit that it is.

In effect, it's kind of puzzling that it's one of the few areas of consumption, of the products that we consume, that people almost resent paying for.

Health care actually represents 17 percent of consumption in this country, just slightly ahead of food, 16 percent, housing, 14-1/2 percent, transportation, 11 percent.

And if a sector of the economy was this large and in fact was a burden, it would be impossible for us to experience a three and one-half to four percent growth in the economy.

It's logical, right? It's impossible for that to happen.

What we see actually is that the whole health care industry is a major contributor to this level of growth. And this is what we would like to bring the attention of Congress to.

Given the robustness of the economy, we have to be very, very careful about encouraging policies that threaten the strength of the health care sector before we fully understand its influence on the economy.

Otherwise, we could very easily and unwittingly reverse a very promising trajectory for the whole economy and cause a downward spiral.

Not only does health care delivery system contribute to the growth of itself, but the knowledge it has generated in genomics and cell biology is rippling out to other sectors and other industries in ways that we're just beginning to see.

What is taking place is really nothing less than a medical industrial boom, paraphrase Eisenhower's—

Senator Mack. Military.

Mr. Ullian. Yes. Industries that were seemingly unrelated to the health sciences, just a few years ago, industries such as chemicals and energy and agriculture and cosmetics and others are looking at genomics and cell biology and seeing entirely new areas of application for themselves.

For instance, the chemical companies are replacing multi-step manufacturing processes with faster, cheaper systems that use microbial genomics to convert sugars and other materials into chemicals.

Other industries, such as the plastics, electronics, and computer industries, are finding whole new markets for expansion in health care and health-related research.

These kinds of companies are rapidly merging and acquiring biotech and pharmaceutical companies and blurring the boundaries between these once-distinct areas.

The result is the creation of new sciences, new products, and new economic activity at a huge scale.

It's also giving us a bunch of new words, too—agro-pharma, nutri-ceuticals, cosme-ceuticals.

The computer industry is a particularly good example of the growing demand by health-related research.

For example, a single laboratory in this country will now generate a full terra-byte of data in one year, equivalent to the contents of one million encyclopedias.

Powerful new software and hardware is required to organize and manipulate these enormous and complex databases and is creating a whole new area of employment—the field of bio-informatics.

The computer industry is working with companies like General Electric and will also be called upon to deliver super-enhanced, three-dimensional imaging capacity for use in diagnostics and micro-surgery.

The new technology will allow advances such as the removal of previously undetectable pin-sized tumors.

Today, the growth in the economy is from telecommunications, software, and the Internet. But it is widely agreed that life science R&D will be the next growth area in an increasingly knowledge-based economy.

And unlike an asset and resource based economy, which depreciates and depletes itself over time, a knowledge-based economy actually appreciates and builds on itself in an increasingly rapid pace.

All these fantastic and amazing new products have developed, products which promise us a better quality of life, better health, increased productivity, they've got to have a market place. They have to be able to get it out, because you can't have a seller of all of these wonderful products that we hear about unless you have a buyer.

And the buyer happens to be the health care delivery system, which includes the research facilities, the hospitals, the medical schools, the academic research areas, the physicians and the providers.

But the buyer is in serious trouble because the problem is the $115 billion cuts in Medicare which understandably were passed at a time when we were projecting enormous deficits running out, and which are coming on top of managed care pressure and reimbursements.

We can't let this happen.

It's critical that we take a very, very conservative approach to any action that will further weaken and destabilize the health care system.

The quality of health care and the strength of our economy are the envy of the world. You said that.

But we need to understand that health care and health and growth are dynamically connected and that the connection lies in research, which can continue the stream of innovation, which has brought us so far.

It needs the commitment which I know you have and other members have to doubling the NIH as well as the other sciences.

I thank you, Senator, and I hope you don't find it too boring out there.

Senator Mack. I assure you I won't.

(Laughter.)

Arthur, thank you very much for your testimony this morning. We appreciate your continued effort in this fight to double NIH and all of the other issues related to it.

Thank you very much.

Mr. Ullian. Thank you, Senator.

Senator Mack. And now I will call the next panel.

Mr. Levinson, Mr. Termeer, Mr. Verfaillie, and Dr. Cassel.

And then we will have an additional panelist via the television here shortly.

Before I turn to Dr. Levinson, though, let me turn to Senator Bennett, who wants to make a further comment.

Senator Bennett. Thank you, Mr. Chairman.

I didn't want to prolong the previous panel. But as I listened to the way the conversation turned at the end and the concern about whom do we call to clear up all this mess, I couldn't help but reflect on what I think is an analogous situation with the work we did in the Congress with respect to welfare reform.

We as a nation are a compassionate nation and we want to take care of those who are homeless and those who are economically deprived.

We've had a commitment to do that from the Federal Government for something like 60 years.

We did a pilot project in the State of Utah that I think summarizes what we need to think about with respect to health care.

We took all of the money that was available for welfare and changed the paradigm completely.

The pilot project ran this way. People would come through the door and they would either be treated in the normal fashion or in the new fashion, the experimental fashion.

So it was completely random who got where.

And in the normal fashion, it was, what are you eligible for? And then we'll try and help you. And in the experimental fashion, it was entirely, what do you need?

We don't care about your eligibility. What do you need?

And when this was proposed, the suggestion was made that the experimental panel would be far more expensive than the panel that was based entirely on a tight control of eligibility.

We found that welfare costs in the experimental group went down, as the social worker dealing with the individual no longer said, well, you're eligible for food stamps, but you're not eligible for WIC.

You're eligible for this kind but you're not eligible for that.

It was just, in your situation, what do you need and we will work to provide it.

Ninety-seven percent of the people in the experimental group ended up in jobs. The people who were taking the eligibility, very few of them ultimately ended up getting off welfare and working because the second approach was, what do you need to get off welfare and solve your problems?

I think that has something to tell us.

We've now reformed welfare and we're seeing very significant changes. We still have problems. But I think that has something to tell us, Mr. Chairman, about health care.

Medicaid, Medicare, insurance companies, the whole approach is, what are you eligible for? When the focus ought to be, what do you need?

And I bet we could drive our costs down if we took a whole clean sheet of paper approach. And I know that's very difficult around here. There are so many investments in political agenda on both sides of the aisle.

But if we could just take a clean sheet of paper and say, this is how much money we are spending per person in the United States on health care, and I think that that figure is the highest in the world.

And say, if we spent that much per person on health care, we could afford to say, what do you need, rather than the amount of money that the doctors we're talking about that they are spending on the infrastructure in their office to deal with the paperwork connected with determining what you're eligible for.

So I just had to make that comment and I appreciate your indulgence.

Senator Mack. I'm glad we were able to give you the opportunity to get that off your chest.

An excellent statement, I might add.

Dr. Levinson?

Panel II

STATEMENT OF DR. ART LEVINSON, PRESIDENT AND CEO, GENENTECH, INCORPORATED

Dr. Levinson. Mr. Chairman and distinguished members of the Committee, thank you for the opportunity to testify today regarding the most important topic of biotechnology and its impact on people like you and me.

Your leadership related to issues of innovation, and medical research and development, has been critical to the ongoing development of new life-savings drugs and breakthrough technologies.

We've been asked to show the human side, the human face of biotechnology, and to discuss the challenges that the industry faces.

As the patients this morning I think so eloquently testified, the human face of biotechnology is very real. All the cutting-edge science and innovative technology of our industry is valuable only when it results in the alleviation of human suffering and the overall enhancement of life.

The mission of Genentech and the mission of many of my colleague companies in biotechnology is to use state-of-the-art science to develop breakthrough therapeutics for unmet medical needs.

What is not necessarily always appreciated is the fact that this an extraordinarily research-intensive industry.

The pharmaceutical industry will typically spend maybe between and eight and 20 percent of its total revenues on R&D.

That is actually quite high by high tech standards. Many of the high tech in Silicon Valley, the semi-conductor electronic industry, they'll be spending in the low single-digits on research.

Our company over the last few years has spent no less than 30 and as much as 50 percent of total revenues on research and development.

And that's not that atypical of biotechnology. And I think it's very important to understand the extreme expense rate because this is a very new industry and it is critically dependent upon innovative science.

As a result of this commitment, we have developed breakthrough drugs for the treatment of diseases and conditions such as heart attacks, stroke, cystic fibrosis and cancer.

And with this brief background in mind, I want to just emphasize a few points that I think are particularly important for the industry going forward—federal support for research and development, permanent extension of the R&D tax credit, and the medical innovation tax credit.

Let me start off with the federal support for biomedical research and innovation.

This has been crucial in the past and I believe will remain crucial in the future.

It is not an overstatement at all to say that this entire industry, our company and I would say all the 1300 companies that the Chairman referred to this morning, are here strictly because of the government's commitment to basic science and in particular, the funding of the National Institutes of Health.

The scientific underpinnings of our own industry—namely, the discovery of recombinant DNA techniques that were performed, among other places, at Stanford and the University of California at San Francisco, all was done with the help of federal funding.

It's not an overstatement to say that without that funding, there really would be no biotechnology industry. And I certainly applaud the Chairman and your colleagues' efforts to dramatically increase the funding of the National Institutes of Health.

Moving on to the R&D tax credit, we're strong advocates of a permanent extension of this.

I think that people put forward various arguments. I want to give our perspective, which I think is the industry's perspective on this.

Except for small increases in the past three years, direct federal support for overall research has for the most part been declining for over a decade.

While a long-term commitment to increasing funds available to the Federal Government for basic research is important, maximizing private industry R&D through a permanent R&D tax credit I believe is a necessity.

Numerous academic studies have shown that a permanent R&D tax credit is a cost-effective means of ensuring that high levels of private-sector investment will continue to take place.

And I want to illustrate this with our own example over the last few years of our company.

We have benefitted enormously from the R&D tax credit. We made a commitment publicly some years ago to the effect that we will take every dollar that we benefit by virtue of the R&D tax credit and put that not into the short-term, bottom-line earnings increase, which I think is ephemeral and doesn't last for more than a single quarter, but put that into the hiring of scientists that will be used to develop the breakthrough drugs that will fuel the development of new drugs five and ten years into the future.

I want to just reiterate that again, that it has made a very big difference to us. We have taken the benefit of that credit to hire more scientists, as has the industry.

And I think, going forward, it's going to be essential if this industry is to remain vibrant.

Another example that my colleagues, Dr. Steve Shak, mentioned this morning, it's a very real example. We have heard from one of the patients who benefitted from the use of Herceptin, the first monoclonal antibody for the treatment of breast cancer.

This was a drug that truly in its early stages wasn't at all clear would be a beneficial drug.

And when we were making the decision a few years ago to either spend enormous amount of monies to conduct the Phase 3 clinical trials or not, that decision was truly on the fence.

And I can say without necessarily absolute assurance, but it's my belief that without that R&D tax credit, we would not have conducted that Phase 3 clinical trial for that drug.

Mr. Chairman, I know you have long been championing the cause, and I know that others in the Committee have been long-time supporters of the credit and it's our desire to work with you to make the credit permanent.

A few words on the medical innovation tax credit.

Over the years, the Federal Government has invested billions of dollars to create a biomedical establishment of medical schools and teaching hospitals that have been deemed the finest in the world.

I believe that.

The growth of managed care, coupled with cuts in Medicare payments, threatens the ability of these medical schools and teaching hospitals to carry out their vital social mission of research, training of health professionals, and the provision of indigent care.

The Medical Innovation Tax Credit would establish an incremental 20 percent tax credit for clinical trials performed at medical schools, teaching hospitals that are under common ownership or affiliated with an institution of higher learning, or nonprofit research hospitals that are designated as cancer centers by the National Cancer Institute.

These are institutions, fine institutions, on which we are critically dependent for future health care breakthroughs and health care delivery.

And I think the statement of that particular credit would go a long ways to ensure the continued viability of the academic institutions.

I will close with just a few words on the future of biotechnology as I see it.

The first quarter century of biotechnology has been a period of astounding advances. The next quarter century promises revelation and quantum leaps forward. I think it will make the advances that we've seen in the last 25 years look small by comparison.

While no one can say with certainty, we scientists, and I am a scientist by background, so I speak with, I think, some degree of modest authority here, but I do believe that we can say that there is a distinct possibility that even within the next ten years, we can foresee the

accomplishments of not just treatments, but actually cures for such diseases as Parkinson's, hemophilia, AIDS and cancer.

But there are two things that are predictable as we look forward to the future of biotechnology.

As in the industry's first 25 years, the next 25 years will require Federal policies that are supportive of biomedical research and innovation.

And finally, the industry as a whole will only succeed if we continue to keep the patient, the human face, in biotechnology, first and foremost, in all of our decisions

Thank you very much for the opportunity to speak.

[The prepared statement of Dr. Levinson appears in the Submissions for the Record.]

Senator Mack. Thank you very much.

Mr. Termeer?

STATEMENT OF HENRI TERMEER, PRESIDENT AND CEO, GENZYME CORPORATION

Mr. Termeer. Good morning, Mr. Chairman, and members of the Committee.

I am Henri Termeer. I am CEO of a company called Genzyme, a biotechnology company. It has 4000 employees. It is 18 years old. It works in the area of breakthrough drugs, again breakthrough biologicals in genetic diseases, cancer, heart disease and also Parkinson's disease.

My thinking about, having heard this morning's session and Dr. Levinson's comments, the answer to the question, how can we deal with what we are working with? Why are we as a very compassionate nation having such a difficulty? Must lie in the fact that we are underestimating what is happening here.

This truly is a revolution.

This is not just advances in science. This is a revolution that will treat diseases differently in the future. It will diagnose them differently. It will make therapies much more specific.

The therapeutic index in the future of the new therapies that we are developing will be much greater than the therapeutic index of the therapies that we have experienced in the past.

That's what it must mean.

And there is an increasing problem in trying to reconcile, on the one hand, health maintenance and on the other hand, potentially curing disease, which is a very different level of change.

This change of course is coming at a very good time, very much the right time. This change is extremely needed because we can't afford not to deal with the health care problems that we have around us.

Cancer is over a $100 billion problem. People die.

Alzheimer's is a $100 billion problem in this country. And people die and people suffer. And families suffer.

Cardio-vascular disease, heart disease—over a $100 billion problem. And people still die or have major complications as a result of these problem.

Diabetes—very similar. We heard examples mentioned this morning. Over $100 billion problem.

We now have the possibility and that's, I'm sure, what has driven all of our intuitive feel to make these investments, to start to make a change in these kinds of diseases, which can only be solved with breakthroughs.

They can't be solved with more doctors, with more hospitals, with more caretakers.

They have to be resolved with much more direct curative kind of treatments.

I think there are three challenges in this. And in all three of these challenges, government, Congress, does play a role, an absolutely fundamental role.

You can only play that role, I think, if you look at this as truly a revolutionary change, not as a small incremental change.

We cannot address the effect of this revolution in an incremental sense.

One of them you are addressing and that's extremely impressive. And that's the NIH, the basic research, funding the basic research, funding what has really created these early breakthroughs, these early hints that we can start to attack these diseases.

And it is extremely gratifying and extremely important that indeed, the NIH funding is growing, such that science can stay longer in academia so that it is readier when it starts to move into the transformation phase.

Industry doing basic science is very tough, very tough to finance, the financial with returns required.

So that is excellent.

Industry has reacted to that. Today, we have a biotechnology industry that is spending ten billion dollars on R&D. But it's also losing five billion dollars each year, funded through private capital at this moment.

This is not to be taken for granted. This can't be sustained over time. This must change. This will change.

This will change because the biotechnology industry will have output that is productive and will allow it to fund itself, to sustain itself.

There's a third element here that I think you ought to look at very carefully. That's how do we deal with the academic clinical research centers?

They are critical in the translation and in the training of these new therapies. And they are in a squeeze, an unfortunate squeeze, an unintended squeeze.

But we have to pay attention to that squeeze, indeed, because we will get in deep trouble if we don't.

The second area is risk-benefit analysis.

And here, there are two areas where you will play, must play a role. One of them is the FDA.

The FDA has made tremendous progress recently. But the FDA can't sit on this progress. It is only the beginning. It has to continue to be funded to stay excellent.

We can't have an enormous flow of innovation without the FDA having the funding available to review this and to stay at the highest possible quality and be challenged to also change the way they look at risk-benefit analyses.

And the second area is the discussion of ethics.

The marketplace won't develop unless the consumer is secure in these marketplaces. We will get hundreds and hundreds of bills in many different states, in many different areas of ethical review, like in the genetic testing area, unless we take some thoughtful approaches where we all work together to create an environment where people feel secure to let advances happen.

And the third, this is a long subject. We've talked about it already all morning. And that's access. That's the third area of challenge.

I do not think that we can fit that what's being created into the way that we are doing things.

HCFA is a very good institution, well-intended when it was set up. I don't think HCFA as an institution can absorb the level of innovation that is occurring.

I'll give you an example, and then close, and then maybe I'll pass on.

Senator Mack. Don't use that word, pass on, in this environment. (Laughter.)

Mr. Termeer. The example is the following, Mr. Chairman.

We are developing a product for Parkinson's disease. It's a revolutionary product.

We take fetal porcine neural cells and we introduce them surgically into brains of Parkinson's patients. You need to drill six holes to do so.

We did 12 patients. Some of these patients had a tremendous effect and it was extremely encouraging.

We now have entered a controlled—you may have heard about this, 18-patient trial, nine patients on the control, nine patients with the active ingredient. And next year we will find out—do we have a positive impact?

In the meantime, I wanted to find out, what if we are successful, which is maybe three or four years from now? How will this be reimbursed? How will this be afforded?

Because the objective, the ambition here is to actually cure the disease, to replace the cells that do not work.

I asked a consulting firm to give me advice. The advice came back. It said, this does not fit in any DRG—this cannot be afforded in the current system.

But we have a suggestion.

Why don't you deliver these cells not in one surgery, which clearly is the most practical way to do it for the patient, for everybody else concerned, but in a number of different surgeries so that you can charge the price allowed within the DRG, many times.

It's of course crazy. It's so crazy that you have to laugh about it.

But these discrepancies, these bizarre discrepancies do exist and they're very difficult to change. And they will exist more in the future as we try to fit this revolution, these many new things, into existing systems that can't absorb these changes.

We have to have the courage, I think, to look at many of these, new health medicine, new health care, in a way that's not based on averaging the past.

Thank you.

[The prepared statement of Mr. Termeer appears in the Submissions for the Record.]

Senator Mack. Thank you very much. I believe we're still experiencing some technical difficulties with respect to sound.

Dr. Edelheit, you'll just have to bear with us while we try to correct those.

Mr. Verfaillie, we'll go ahead to you.

STATEMENT OF HENDRICK A. VERFAILLIE, PRESIDENT AND COO, MONSANTO COMPANY

Mr. Verfaillie. Thank you, Mr. Chairman.

I'm Hendrick Verfaillie and I'm president of Monsanto.

Monsanto is a life sciences company that is dedicated to finding solutions to the growing global needs for food and health, utilizing common technology, biotechnology and genomics, across agriculture, nutrition, and health.

As a Belgian who has lived, raised a family, and worked in this country for over 20 years, it's an honor to be here and I am very thankful to have the opportunity to represent not only Monsanto, but also the other side of biotechnology—agricultural biotechnology.

There are three points about the benefits and potential of agricultural biotechnology that I want to share with you.

First, this industry is driven both by our growing technological capabilities and by the growing global pressures to produce more food on less land, and in ways that are more efficient and, especially, more environmentally sustainable.

The current system is just not sustainable.

Secondly, it's directly and dramatically benefitting farmers and the environment.

And third, while the current benefits strongly favor the farmer, we believe that American and global consumers will be the ultimate beneficiaries of this technology.

To put agricultural biotechnology into context, humans have been planting and improving crops for over 10,000 years. Today, almost everything we eat is the result of improved animal and plant genetics over time.

In the last 200 years, we have made a lot of progress. Mendel and Watson and Crick's discovery of DNA has allowed us to learn how biology works and how genes work.

We can now be very precise and efficient in creating new varieties and traits and in how we farm.

Twenty years ago, pioneering Monsanto scientists came to company executives like myself to convince us that biotechnology could create better crops and better agricultural products.

To bring the first crops to market required years of safety and environmental tests, reviews by EPA, USDA and FDA, and hundreds of millions of research and development dollars.

The first biotech crops were planted commercially only three years ago.

We spent 20 years to get to that time. And we spent well over a billion dollars to get there.

Today, we are now creating crops that fight insects and disease and enhance the vitamin and nutrition content of the plants and that will grow under adverse conditions like in arid soils or high salinity soils.

A potent example of the practical power of this technology is Hawaii's papaya crop—once threatened by a deadly virus.

Using biotechnology, papaya plants were genetically improved to resist that virus. And now, this industry, made up mostly of small growers and once at the verge of extinction, is flourishing.

Our industry is being driven by an inescapable need to produce more food and to do so in ways that are efficient and environmentally sustainable.

Six billion people live on earth today. By the year 2025, we expect eight billion. How are we going to feed these people?

Producing feed for eight billion people will require 75 percent more agricultural production. And we don't have any more land to add.

So we need to come with new technologies.

Meeting this challenge, using conventional and traditional agricultural technologies is very problematic.

Without new technologies, we will have to convert ecologically sensitive resources like forests, wetlands, deserts, to grow the food we require, and that is not sustainable.

Biotechnology is the tool that can help farmers to provide the food that the world needs.

Because of the benefits of biotechnology, American farmers are choosing and planting genetically improved crops in record numbers.

As a matter of fact, in '99, 65 million acres of bioengineered crops will be grown, just three years after introduction.

Mr. Chairman, 47 percent of the cotton crop in Florida has been genetically improved, 55 percent nationwide.

The benefits of agricultural biotechnology include use of fewer agricultural chemicals to protect and nurture the crop. Over two million fewer pounds of pesticides were applied because of biotechnology in '99 alone.

This means fewer chemicals in the soil and water.

We use fewer resources, less gas for tractors, chemicals for fertilizer, and so on, the potential for greater yields that the farmers enjoy, and a lower cost, make the American farmer more competitive.

But consumers stand to be the ultimate beneficiaries of this technology.

Genetically-improved high stearate soybeans require no hydrogenation to produce margarine or shortenings and help eliminate trans-fatty acids that contribute to high cholesterol and other health risks.

Over 20,000 children a year are blinded because of Vitamin A deficiency. Genetically improved oilseed plants with higher levels of Vitamin A help eliminate this serious problem.

We have donated this technology to the US AID to get it to the people that need it most.

Reducing mycotoxins. Mycotoxins are potentially hazardous to both human and animal health and are released by a fungus that grows when a Corn Bore invades and damages a corn plant.

Genetically-improved Bt Corn, for example, controls this insect damage and prevents the fungus to develop these harmful mycotoxins.

New products will soon be available that lower blood pressure or that contain antioxidants that may help prevent cancer, as well as products with nutrients to help fight heart disease—particularly important traits as America's population ages and health costs, as we have heard, become more expensive.

The U.S. biotechnology industry and market is by far the global leader. Over 140,000 people are employed in the industry nationwide, and the positive economic impact is great.

It also encourages innovation in the formation of new and emerging businesses. We see new start-ups almost every day.

According to OECD, $9.4 billion research dollars were spent in '97 alone.

We must work together on several key issues, including constantly refining the regulatory process as the technology grows. This is absolutely critical to maintain public confidence.

Research and development tax credits are needed to encourage new businesses and applications.

We must prepare our children with the scientific education and skills needed for the jobs being created.

This industry is still in its infancy. What we can do is unimaginable at this point in time. It is not going to be a small increase. It's going to be a huge step increase.

Public confidence in the safety, science and oversight of these technology is absolutely critical. When that confidence is challenged, as we see today in Europe, issues such as trade and suspicion dominate the public discussion.

We recognize our responsibility to hear both the concerns and to do something about it. We must reinforce the strong independent

scientific verification, including years of testing and regulatory oversight that has made agricultural biotechnology safe, health and environmentally sustainable.

We are eager to work closely with you, Mr. Chairman, and this Congress, to create an environment that will encourage this emerging industry.

Thank you.

[The prepared statement of Mr. Verfaillie appears in the Submissions for the Record.]

Senator Mack. Thank you very much.

Dr. Cassel, if you'll just bear with us, I think that Dr. Edelheit is ready to go. I think that we've corrected the problem.

So, Dr. Edelheit, we look forward to your testimony.

STATEMENT OF DR. LEWIS EDELHEIT, SENIOR VICE PRESIDENT, GENERAL ELECTRIC CORPORATE RESEARCH AND DEVELOPMENT

Dr. Edelheit. Hello?

Senator Mack. It works.

(Laughter.)

Go right ahead.

(Pause.)

Well, so much for high-tech.

Dr. Edelheit. Can you hear me?

Senator Mack. Yes, we can.

Dr. Edelheit. Okay. Thanks. I'm sorry. It's not much of a human face of medical technology here today with all of these videofeed problems.

But thanks for the opportunity to participate.

My name is Lonnie Edelheit and I'm Senior Vice President of General Electric, in charge of research and development.

It's a pleasure to talk to you today.

It's been to me the great things that are happening in medical science today. Fortunately, I've spent most of my career in medical imaging systems. And I have to say that the technology advances that I've seen in the last 30 years are absolutely staggering.

Our ability to see inside the body with sophisticated imaging equipment like CT scanners and MRI and ultrasound and nuclear medicine and pet scanning has profoundly changed medical care, eliminating surgeries and all sorts of things.

Most of this progress has been funded by companies like the General Electric Company and others.

We continue to spend hundreds of millions of dollars a year on medical imaging research. But public policy and Federal funding can play a vital role in the development of biotechnology in the beginning, in the middle, and at the end of this innovation process.

In the beginning of the innovation process, everything rests on fundamental and basic research at the universities and the National Institutes of Health. And I strongly encourage that to be the top priority here.

Everything that we do depends on that and it really keeps our country strong, as well as improving health care costs.

At the end of the innovation process, the issues related to the FDA and HCFA and how technologies actually get into the market place, I think there's a lot of work that needs to be done as we sort through the regulations and risks and speed.

I think we can all work together on that.

In the middle of this process, between basic research and market introduction, I think government can play a huge role. And that's what I'd like to focus on today, similar to the other speakers.

In the final analysis, the only way to get a breakthrough in medical care is by industry producing a product or a service. And industry prioritizes its R&D based on a relatively narrow risk-reward trade-off and probability of success.

It's a great standard for us, but it leaves a lot out. And in partnership with the government, industry can also address national priorities that we might not take on our own—taking bigger risks, speeding up the pace.

Let me give you a couple of examples.

In the last several years, early detection of breast cancer has been a high priority, higher than our market research would have made it.

Fortunately, the National Cancer Institutes, the Army, DARPA, supported some breakthrough research in digital x-ray imaging.

As a result, this year GE and others will begin manufacturing digital mammography systems. I think this is the first major advance in mammography in a generation.

Another example, in the early '70s, when the CT scanner first was developed—I was involved in those early days—the early CT scanners took about four minutes to take an image and it could only be used for the head, which they could keep rigid.

The National Cancer Institute challenged industry to develop a system that could scan in ten seconds or less and image everywhere on the body.

None of us had a clue how to do it. But the program galvanized many companies like GE. We actually didn't win that contract. Somebody else won it and they got into the market place. But we took on the challenge also.

I think this one project jump-started that industry, and that technology, by at least three years.

I think the National Cancer Institute and the National Institutes of Health never took enough credit for that because they got all sort of worried about who did the product and who didn't.

I know, Chairman Mack, you've been to the National Institutes of Health to see cardiac MRI images. We believe that noninvasive cardiac imaging is a huge opportunity to reduce heart disease. And we think that is going to really have a huge effect on health care.

We could be going faster. But, again, the technical risk is too high. And so, we're moving fairly cautiously. But the government could really help speed that up.

Lung cancer screening is another example where we could move faster.

The survival rate right now for lung cancer is 12 percent because we can't see the tumor early enough, we can't see it until it's grown big enough to image.

This summer, researchers at Cornell found that, with some advanced CT scanning, they could find the tumor much earlier and could perhaps significantly increase the survival rate of patients found to have lung cancer.

You may be able to reduce by half the time to develop such a CT scanner. That might save many lives.

The point is industry has to target its resource to a fairly narrow risk-reward window. But Congress and the federal agencies can successfully challenge and fund industry to stretch further and go farther in those areas that you decide are national priorities in health care.

There are many exciting opportunities out there and there's lots you can do to make a difference.

Thank you very much.

[The prepared statement of Dr. Edelheit appears in the Submissions for the Record.]

Senator Mack. Thank you very much.

Dr. Cassel, we'll go to you.

Dr. Edelheit. I can't see at all. I'm sorry.

Senator Mack. Can you hear us?

Dr. Edelheit. I can now, yes.

Senator Mack. Okay. Again, thank you for your testimony. We're going to go to Dr. Cassel next. And then there are three of us here on the Senate side that we'll be raising some questions.

If you can be patient and stay with us for a little bit longer, we might raise some questions to you.

Is that all right?

Dr. Edelheit. Right.

Senator Mack. Okay. Good.

Dr. Cassel?

STATEMENT OF DR. CHRISTINE K. CASSEL, CHAIRMAN OF THE DEPARTMENT OF GERIATRIC AND ADULT DEVELOPMENT, MOUNT SINAI MEDICAL CENTER

Dr. Cassel. Thank you, Mr. Chairman, and members of the Committee. It is a privilege to be here with you today.

And I particularly appreciate the spirit of putting a human face on biotechnology.

What I'd like to do in the next few minutes is put a human face that represents tens of millions of human faces. That is, the face of a successfully aging nation. Or as one of my medical students put it on a T-shirt—Be Old. 35 Million People Can't be Wrong.

(Laughter.)

I'm an internist, a geriatrician and chairman of the Henry L. Schwartz Department of Geriatrics and Adult Development at the Mount Sinai School of Medicine in New York.

I have specialized in the care of the elderly for 20 years, leading academic research and training programs at several major medical centers in this country.

One of my own areas of study is the demography and epidemiology of aging. While it is now common knowledge that Americans are living longer than ever before, most people don't realize that there is still a lively debate surrounding the question of why this is the case.

Advances in biotechnology are central to this discussion.

Life expectancy in the United States has almost doubled within this century alone—more than all other previous history of the human species.

By 1965, the average life expectancy in the United States had increased to approximately 70 years. The framers of the Medicare program assumed that we had probably reached something near the genetic limit of life expectancy.

Shortly after Medicare was implemented, mortality rates for older people began to decline and have continued to do so up until the present time.

I believe, as do a number of other experts in the field, that while advances in life expectancy that occurred in the early part of this century were primarily related to improvements in public health and living conditions, that the advances that we are now seeing are directly related to medical care and interventions.

The dynamics promoting increases in life expectancy are not limited only to the United States.

We should be aware of this.

The United States is approximately number 13 in the world in both average life expectancy and the percentage of the population over the age of 65.

Thus, we can, and perhaps should, look to other countries for examples of how different health care systems and different socioeconomic frameworks have both contributed to successful aging and also helped to cope effectively with the demands on families and on society.

Because the promise of successful aging is now something that most Americans are aware of, we must be realistic about the investment it takes to achieve that promise.

Individual decisions and health behaviors count for a great deal. But, increasingly, medical care provides meaningful advantages in quality and length of life.

As we've heard this morning, in the last 20 years, advances in science and medicine have been unprecedented. Much of this has come from the biotechnology research fields, which are funded both publicly and privately.

In the field of aging itself, dramatic advances, particularly in surgical techniques and pharmacological treatments, like what we just heard about, the promising new treatments for Parkinson's disease, for both common and uncommon diseases, has made it possible for us to prevent many of the diseases and, as importantly, the long-term disability that often occurs with age, such as stroke and heart attack, and ameliorate the symptoms of other chronic diseases to improve quality of life, function, and independence of those who suffer from them.

In this category are very common disorders, such as osteoarthritis, which affects 50 percent of people over the age of 65, diabetes, osteoporosis, Parkinson's disease, and depression.

We now are on the verge of effective medical treatment for Alzheimer's disease, which is one of the greatest threats to successful aging.

Advances in genetic research have helped us to understand telomerase, the enzyme which controls the aging of cells, which may lead to a cure for macular degeneration, responsible for one-third of the causes of blindness in the elderly.

Treatments for cancer have become much more successful in older people, but require expensive adjuvant therapy because elderly people are more susceptible to the side effects of these highly toxic chemotherapy agents.

Surely, dramatic advances will continue and it should be possible to promise these treatments to older people.

The expectation that 80 percent of Americans will live well past the age of 65 is a dramatic success story of our modern civilization, one that we should be celebrating.

Unfortunately, because of the potential financial burdens that are seen, this success story is often seen as a burden and a problem rather than one that offers tremendous benefits to families.

Access to appropriate medical and nonmedical interventions requires flexibility that most current market place insurance models don't offer. And the barriers that are faced were described by many of the patients this morning.

The major problem is affordability and insurance coverage.

Increasing numbers of older Americans do not have medication coverage as part of their Medigap insurance. And increasing numbers of employers are limiting or eliminating supplemental insurance as a retirement benefit.

Most older people are not wealthy and cannot afford out-of-pocket expenses that range from $3000 to $6000 a year or more.

Characteristically, as people age, they may suffer from multiple chronic diseases. As medical treatment for these disorders improves, disability decreases. But this has real financial costs to families.

This is not just an issue of benefits to the elderly. People don't exist in horizontal groups called generations. They exist in vertical groups called families.

So every cost to an older person is a cost to their adult children and to those people's children.

Here's an example.

A patient needing ongoing treatment for hypercholesterolemia to prevent heart attacks, hypertension to prevent heart attacks and strokes,

arthritis to increase mobility, health and productivity, osteoporosis to prevent disabling fractures, diabetes to reduce the risk of kidney failure, blindness and amputation of limbs—it's not unusual to have one person with all of those disorders still quite functional and able to be productive with appropriate medical treatment.

But that person can easily rack up charges of $10,000 a year or more.

So this is not just about a single catastrophically expensive, newly developed drug. But about common treatments for common disorders as well.

In addition, any intervening illness such as cancer, depression, recovery from spine or hip fracture, can lead to thousands of additional costs.

If the older persons themselves are not paying these costs, it falls on their children, who are struggling to put their children through college and raise their families.

Thus, it's essential for us as a nation to find a way to make the advances of the biotechnology industry available to more than just the wealthy among us.

Like Arthur Ullian, I see this as an investment, not a drain on our economy.

Let me suggest that access to effective medications will keep our older population productive, reducing ageism in the work place and allowing people to work longer, which has better effects for their health, as well as better effects for our economy.

Now I am aware that this hearing is not addressed at specific policy strategies to deal with the problems of coverage, access and affordability of medications and other new technologies.

But as a researcher and a physician caring for patients, I do know that it is very important as we celebrate the success of the biotech industry, to realize that if we do not address the affordability question, we may find ourselves in a situation where we can do miraculous, life-saving, life-prolonging, and life-enhancing treatments, but they will only be available to a small number of the population.

The burden of chronic disease, dependency and disability will fall on families and on society if we allow this to happen.

Thank you.

[The prepared statement of Dr. Cassel appears in the Submissions for the Record.]

Senator Mack. Thank you, Dr. Cassel.

I'm going to turn to Senator Bennett first.

Senator Bennett. I'll pass to Senator Frist.

Senator Frist. Thank you, Senator Bennett.

If we look over the next ten years, given the position where each of you are positioned in terms of new drug development and research, if you say that there are going to be, say, 100 new drugs coming through, how many of those will originate in the United States or can be really traced to research here in the United States?

And again, I understand that these boundaries of the United States today, with scientists and communications barriers falling, it's almost impossible to say.

But if you project ahead, 100 drugs, the next ten years, how much of it really comes out of research monies here, private and public?

Any of you can start.

Mr. Termeer. I would guess, Senator, it has to be by far the majority.

The amount of basic research spending in companies outside the United States is a fraction from what it is here.

The biotechnology industry in Massachusetts probably spends more by itself than the whole biotechnology industry of significant countries in Europe, like France.

So it is a matter of starting from the same base. Indeed, the technology is very available in Europe or in Japan or elsewhere.

Then it is a matter of investing in the application of the technology. And that is—where we're way ahead infrastructurally and in terms of actually spending the money.

Dr. Levinson. If I could just make a slight distinction.

Senator Frist. Yes, I'd like to hear from all of you.

Dr. Levinson. Between the biotech industry and the pharmaceutical industry.

I certainly agree with Henri on the biotech side. Probably 90 percent at least in the next ten years of the drugs that will be coming out of biotech will be coming out of the U.S.-based companies.

On the pharma side, there are some very good, big and fine companies—Glaxo, Nevardes—that are European or ex-U.S. based. And I would imagine that 30 to 50 percent of the drugs coming out of the pharmaceutical industry, as a total. And of course, they would swamp the biotech component, at least today. Maybe not so much going forward, would be outside the United States.

Mr. Verfaillie. If you look at the capabilities in biotechnology and especially genomics, this is really very highly concentrated here in the United States.

Even European countries, to a large extent, have made their investments here in the U.S. because these technologies were originally not as well accepted in Europe and therefore, a lot of their research has been established here.

Senator Frist. And what about investments by the federal government in biotechnology, separate from pharmaceutical.

Is our federal investment, as well as you can track it, through NIH and others, significantly higher than the European countries, or Asia?

Again, just to give us a feel for global competitiveness.

Mr. Termeer. It's certainly a feel. This can be figured out very easily. I'm sure somebody will do it as a result of your question.

But there's a very large difference. There are no NIH-like organizations that have as broad a system, keeping so many different researchers competitive with each other to come up with solutions.

It's a much more fragmented approach and the dollars are much less.

I grew up in Holland and I have to admit, too—Hendrick grew up in Belgium—and I'm afraid that, as a European, I have to say that Europe is benefitting, tremendously from the pioneering role and the financing that happens in this country.

Dr. Cassel. Senator Frist, I could also support that by my position on the advisory committee to the director of NIH.

It is definitely true that this country is orders of magnitude great investment in research, and particularly basic research, than other countries, but that other countries are benefitting from it.

I think that really is the fundamental question for us. Is it necessary for us to prevent our citizens from accessing these medications in order to stimulate innovation?

I think that's a really fundamental question.

Senator Frist. Dr. Edelheit, can you hear us?

Dr. Edelheit. Yes. If I could make a point.

Senator Frist. Please.

Dr. Edelheit. I think there's a combination here of government funding basic research and then this huge venture capital industry in the United States, which is also somewhat unique in the world, that leads us, our ability in new industries like biotech, to move much, much faster than the rest of the world.

In a more mature industry, like pharmaceuticals or imaging, then there are very strong global competitors.

Senator Frist. Thank you. Thank you, Mr. Chairman.

Senator Mack. Senator Bennett?

Senator Bennett. Thank you, Mr. Chairman.

My mind goes back to a speech I once heard on the Senate floor by one of our colleagues. And because I'm somewhat critical, I'm going to leave his name off.

But as occasionally happens in the Senate, there wasn't that much going on and he got carried away and spent a lot of time talking. There was no one on the floor.

The reason I heard him was that I was sitting in the chair and had no place to go.

(Laughter.)

He talked about what he thought was the most significant problem facing the world today, which was overpopulation.

And Dr. Cassel, you've gotten into that area when you were saying that people are living longer and that obviously is contributing to overpopulation.

Health care, people are not dying of diseases in their young years. We've all got the statistics you've given us of people whose lives have been saved.

When he got through lamenting global overpopulation, because he still had the floor and still had the muse on him, he then started to tell us about the great humanitarian work that was being done by a group of people close to him.

They were going to Africa and elsewhere to bring medical breakthroughs and helping people survive and live longer.

And I thought, do you realize, statistically, you're on both sides of this issue? On the one hand, you're lamenting that we have so many people, and on the other hand, you're doing everything you can to increase the number of people.

Now, of course, he would argue that and say that the overpopulation problem is caused by lack of contraceptive activities and the position of some people on the issue of abortion.

But, statistically, I think the overpopulation challenge, if we accept the theory that there is overpopulation—and Mr. Verfaillie, you're going to solve that problem. You're going to feed the world and prove Malthus wrong.

But, statistically, I think clearly, the major impact of overpopulation comes from what you people do. As you save lives, then we have more people on the planet.

And it is picking up a comment you made, Mr. Termeer, a revolutionary change, not an incremental change.

And we human beings don't like that. We like to take very small steps into the future and be very comfortable—the old wing-walkers image. As you're walking on the wing on the biplane, don't let go of the wire you're holding on until you have hold of the next one.

You don't just walk down the wing.

And what has happened in the previous hearing we held with the software folks and the hardware folks, revolutionary change, where they have not only let go of the wire, they've changed planes in mid-air. And the kinds of breakthroughs you're talking about have produced those kinds of changes.

The two Europeans here, the green parties in Europe are fighting technology. I refer to them as the modern Luddites who only want things the way they were.

And they're fighting enhanced seeds. And they're fighting things that I think are the solution.

Now, the question is, is there a question in all of this? I'm just ruminating with you and you're all waiting for the question.

Do you, any of you, have any suggestions as to how we can have a paradigm shift in thinking to recognize that the new world that has been invented, from which we cannot now flee, can be portrayed as an attractive one?

Dr. Cassel, I was delighted to hear you say that aging, if we work into the aging situation, this has better effects on our health as well as our economy.

The longer we work, the more we're paying into social security and the less we're taking out of it.

Dr. Cassel. Right.

Senator Bennett. We've got to get people to think in terms of the new paradigm as a pleasant one, as a good one, rather than as something to be terribly feared because, as I say, my feeling here in the Congress is that the luddite feeling is very strong.

The only thing that we like better than things the way they are, is things the way they were.

(Laughter.)

The only thing we like better than Medicare the way it is is Medicare the way it was in the '60s.

And you're telling us that Medicare in the '60s no longer cuts it.

Dr. Cassel. That's exactly right. And the inability to cover outpatient medications is only a small part of that. That's why I used the phrase, flexibility, because I think that we really must look at ways to not have people doing the most expensive thing because that's what Medicare

covers or in other private insurance, but to do the right thing and the most appropriate thing.

We haven't yet really figured out a cost containment strategy that allows us to do that.

There are some ideas, but, unfortunately, health policy too often starts out being budget policy rather than starting from the premise that we have these dramatic new advances in medicine and the capability to keep people healthy.

How do you put that into a meaningful health care program?

So that's where I think the partnership between the providers and the industry has not really been able to work very well because we're all fighting against each other on this cost front.

If we can for the benefit of society and the economy keep people healthy and make it possible for them to work longer, it would be worth the investment of expanding Medicare. And even, dare I say it, reducing the eligibility age.

The last thing, if we're going to delay the eligibility age for social security, the last thing in the world you want to do is delay eligibility for Medicare also because then nobody is going to hire these older workers.

So you really have to think about what's going to keep people active and productive. And then that's the goal that I think we all share.

Senator Bennett. Any other comments?

Mr. Verfaillie. Yes. If you talk about overpopulation, it's really two sides.

If you look in the developed countries, population growth is very slow. It may actually become a problem. For example, in Japan.

On the other hand, where population is very rapid is in the poor, undeveloped countries.

It's a fact that if the economic development of these countries progresses, as we have seen in some of the Asian countries, that the birth rate comes down because they don't have to have ten children in order to have one or two survive.

And so, the projections that have been made about population growth have been coming down because of economic development that has resulted in a slower birth rate.

Senator Bennett. Any other comments or reactions?

Mr. Termeer. I would just amplify Dr. Cassel's comment.

We can learn from the past a few things. We can't rely on the past for the future.

The managed care event a few years ago did reduce static costs in the health care system. But that just—reduced the static costs.

But health care and increasing health, as we improve health care, is very dynamic. It has to be calculated outside the box of the current health care budget.

It has to be calculated in the big picture, as was suggested by Dr. Cassel and yourself and others.

What is the value of that extra life, the quality of life that's being created in economic terms? And I think once we understand that a little better, I think we will become much less afraid of it.

Senator Bennett. Thank you all very much. I appreciate it.

Thank you, Mr. Chairman.

Senator Mack. Thank you, Senator Bennett.

I do have a series of questions that I'll try to run through fairly quickly, just areas of interest.

Dr. Levinson, let me just raise this question with you.

The increased application of biotechnology and genomics in research is blurring the line between traditional drug companies and biotech companies.

Will increased integration between the biotech industries and the pharmaceutical industries make raising capital more or less difficult for start-up or biotech firms in general?

Dr. Levinson. I would say that, if anything, it's going to make it easier.

What venture capitalists want to do is they want to see prospects for return on investment. And if they see, as I think they should see, the prospects of the genomic era facilitating, expediting the process of drug development, it's still in the province, I think, of the pseudo-academic start-up world. It's been embraced, or attempted to be embraced by the pharmaceutical industry, but they tend not to be as nimble and to move as quickly.

There's actually quite a bit of venture capital flow into some of the start-up genomics companies. And actually, some of their market caps now are in excess of one and two billion dollars.

So I think, if anything, to the extent that the capital community recognizes that there are opportunities here, even though they might be seven, ten, 15 years down the road, I haven't seen any deterrent to supply the start-up funds with money.

Senator Mack. Good. This is a question that maybe all of you might want to respond to.

But it seems like—in fact, it was just the other day I heard another report on television about a new product that was being developed that had to do with immunology. But whenever you hear this, again, thinking

of it as a patient, you then are immediately told it may be five, six, seven, eight, nine years before that product makes its way into the market place.

And I guess my question is, one, what are the things that we can do to try to speed that up?

I say that with some hesitancy after the story in the paper this morning with respect to the young man who died in a clinical trial.

I don't mean to make any more of that than just the fact that it was reported. But some people already are raising questions.

So the issue is how can we safely speed up the process of getting that product from the bench to the bedside, as they say, in your trade?

Does anybody want to respond to that?

Dr. Cassel. I have a couple of thoughts and I'm sure that my colleagues here do as well.

And I will leave the FDA out of this because I'm sure that other people will want to comment on the FDA process, which, as you know, is changing.

But we do need to remember also the other lesson from Europe, which is that when drugs are released prematurely, there can be bad consequences as well.

And so, the FDA has served a very good function in the United States in looking for potential adverse consequences very carefully and very rigorously, and particularly with new genetically engineered and other kinds of treatments that we aren't that familiar with, due caution is warranted.

Now having said that, I share the excitement of many people here about the promises of new technologies and new drugs.

I think that one of the most important things we can do to shorten that period, that we need to find out that they are safe as well as effective, is to take much more seriously the need for research and the clinical trials discussion that occurred here earlier addresses that.

If we have to pay—we, NIH, or we, the academic medical centers, or we, the industry itself—have to pay to enroll thousands, tens of thousands of people in these trials, which are really necessary in order to tell what treatment A and treatment B have going for them—and this also gets at the issue of cost effectiveness, and which are really worth paying for.

It is hugely expensive to do that.

Many of these drugs should be tested once they are approved drugs. And yet, insurance companies and managed care companies, if you enroll in a trial, they won't pay for anything that you're doing.

Now you talk about Luddite approaches. If you're not studying the effect, they'll pay for it. If you are studying the effect, they won't.

Same treatment. Now this doesn't make any sense.

So if we had everyone in our country sort of optimistic and engaged, or at least willing to be offered the possibility of entering their treatment for whatever their illness is, into some kind of study, we could be learning an enormous amount more, instead of sequestering clinical research in this smaller area.

Senator Mack. Anyone else?

Dr. Edelheit. May I comment, please?

Senator Mack. Yes, you may.

Dr. Edelheit. I think that if we thought of this like the Defense Department, bringing technology to market, where there was really a partnership between industry, university, basic research and the market place, if you will, things move very quickly.

I think too often in this health care arena, we have a crowd doing basic research and another crowd thinking about bringing a product to market, and in the end of the game, FDA or HCFA. And if we all work together in partnership, I think we could really significantly shorten the period of time that we bring new products to market that I'm familiar with in the imaging system business.

Senator Mack. Very good. I'm going to come back to you in just a minute for a question.

But, Dr. Levinson, did you want to respond?

Dr. Levinson. Yes, a couple of comments. First of all, I want to acknowledge the efforts that the FDA has made to streamline their drug approval process have really made a huge difference.

The approval times in the early '90s were typically 34 or 36 months from the time an application might have been submitted for approval of a new drug to—I think the latest 1998 statistics are 11 months.

That just makes a huge difference.

The industry has always done a very good job at pointing fingers at everybody kind of except themselves in terms of what the problem is.

I think now, since the FDA has been really proactive, it doesn't mean that they can't do more, but I think there are opportunities within the industry.

And one I would point out is in the area of animal models.

Very often, a critical step is how do you really know when you have enough efficacy data for an animal to go into a human clinical trial?

You can spend years and years trying to convince yourself that the animal model might be meaningful. I think the industry and through the

efforts of NIH, as an example, have added an awful lot in recent years and I think will continue to make animal models, for example, like Alzheimer's.

To make a decision to take a drug into human clinical trials for Alzheimer's is a monumental and very nerve-wracking decision because there's really not any good model of Alzheimer's of an animal.

But there's progress being made and the area that you spoke of plays into this because we can now replicate genetic diseases in animals.

I think that, in the future, a real opportunity to cut drug development time will come from enhanced animal models.

Senator Mack. Very good. If I could, let me raise one question with Dr. Edelheit, because I understand you may have to go.

How important is it for private industry to collaborate and share research? How important is it that private industry and the government collaborate on research projects?

Dr. Edelheit. Well, like I was saying, I think the collaboration between universities—NIH, the government, industry—can move things very quickly if we all get our minds around cooperation.

I think that model works very well.

I think between companies in the same industry, it often gets a little tricky. But between industries that aren't competitive, supplier/customer relationships or pre-competitive technology, there's a lot of room for improving that significantly.

And again, I think DARPA has been terrific at that. The NIST ATP program has been terrific at building those partnerships.

I think those are great models for health care, too.

Senator Mack. Okay. Thank you.

Mr. Termeer, maybe this is the question that I'm going to close on because it's already almost 1:00 and I guess we're running about an hour over.

And this touches on the comment that I made at the conclusion of the first panel.

How can we best balance the need to make drugs affordable for patients, particularly seniors, and the biotech industry's need to be profitable so that it can attract the venture capital it needs to sustain research and development?

That's an incredibly difficult question. But that's really where we find ourselves in trying to find some balance that accomplishes all these different things that we want.

So you're all welcome to engage in that discussion if you want to.

Mr. Termeer. As you were suggesting, Mr. Chairman, it's a very, very large question.

But maybe we can comment on what approach can we use. I commented earlier, if we use an incremental approach to try and fit these truly major new developments into the current system, I think we're not going to be successful. It's going to be extremely uncomfortable and there are going to be significant segments in the population that are going to be participating in a very delayed fashion with enormous pressures and all kinds of very uncomfortable ways are going to be figured out to make sure because we are compassionate, that people do get access to the right drug.

But it is not something that will allow this innovation to be pulled into the health care system.

So the current systems do not allow very easily this to happen.

So if we start to view this in a step function way rather than an incremental way, we may have a chance.

And I would make one suggestion, that maybe we should look at what's happening now where we start to truly address the disease at the core because that's where the real opportunities of these technologies are, and where the therapeutic index of the kinds of things that will become available over the next 25 years to the market, is quite different from the way that we've thought about health care in the past.

So maybe you ought to think about this more in catastrophic terms.

There is not a choice. Somebody who has cancer and can be saved, particularly when there is good evidence through good clinical trial work and an approved drug, that a patient can be saved, there's not a choice.

It's not, let's think about it a little bit.

That is different from most things that are currently clogging up the health care system, which is mostly care costs, where we don't really add tremendous incremental value.

It's still very valuable, but a very different dimension of therapy.

I think we need to pull these two apart and think about catastrophic things, differently than the other piece. And maybe that's a start.

Senator Mack. Good.

Dr. Cassel. I would respectfully disagree with Mr. Termeer on this.

I think that care and cure go hand in hand. And especially with an aging society and realizing that chronic illness is actually a success story because it used to be that people died of those diseases. Now they live with those diseases, and live well if we do the best we can.

But in order to have the most prudent, targeted, and cost-effective use of all of the wonderful new technologies that are before us, we must have very intensive care management, for lack of a better word.

You can't just wait until Lance Armstrong starts coughing up blood and shows up in the emergency room. There's got to be a primary care system.

There's got to be people who can pick up signs that something's going wrong early and figure out, is this a medical problem or is this a social problem?

Is the person perhaps not eating?

Is there something that's going wrong in the home?

Is there a depression that is the reason that the individual suddenly appears to be having Alzheimer's disease? Maybe it's not Alzheimer's disease.

Those kinds of questions could end up with a lot of people getting the wrong treatment and getting it too late. If we sacrifice the care function and sort of the basic health care piece—and that's what worries me about some of these models that try to delay the initial coverage until a catastrophic point because if people, in order to save their limited incomes, don't go when they get that first symptom or when they need that preventive care, then I think we're going to end up paying more and our new technologies are going to be less effective.

Mr. Termeer. Let me just say, I absolutely agree with you. I just think they—

Dr. Cassel. That's how they do it in Holland.

(Laughter.)

Mr. Termeer. No, there are lots of problems there.

(Laughter.)

But I think you can look at them. The problem is that we are combining these two things in an administrative sense, and that is problematic.

I'm not excluding one for the other. They need to be looked at separately.

Senator Mack. Well, again, I thank all of you for participating. I will use the prerogative of the Chair to make one last little comment.

It comes out of some hearings that we had in the Finance Committee when we started trying to work our way through issues of Medicare reform or modernization of various aspects of it.

And it touches on the debate that's taking place over prescription drugs.

For years, there were many in the Congress who had kind of an immediate reaction to the idea of whether Medicare should include prescription drugs or not with just kind of saying, no. It costs too much with all the things that go along with it.

But I think that society today, which is reflected in the Congress, recognizes that the delivery of medicine has changed so dramatically in the last 30 or 40 years.

And to not reform or modernize Medicare is just, again, acting as if that change hasn't taken place.

Now here's my point of concern.

We're so focused on the present system, and there are going to be huge efforts to not affect the present system.

In other words, we've made a commitment to a hospital-based medical system that was developed for the old technologies.

The political pressure is going to be to protect that past investment because I can imagine the pressures that every one of us would get. All you have to do is look at the veterans hospital issue and the effort to try and move resources just from one part of the country to another, not necessarily from one form of medicine to another, and you see the political pressures that are there.

My concern is that we could over the years have huge amounts of resources that are tied up in the old technology because we don't have the political will to make the change into the new technology.

So I think that the discussion today has been helpful and I do appreciate all of you participating.

The hearing this afternoon starts at 2:15. Thank you all very much.

(Whereupon, at 1:03 p.m., the hearing was recessed, to reconvene on the same day at 2:15 p.m.)

AFTERNOON SESSION (2:20 p.m.)

Senator Bennett. The Committee will come to order.

Senator Mack has been detained elsewhere and has asked me to chair the Committee in his absence. He will join us as quickly as he can.

We're very grateful to the witnesses that are here this afternoon, not only for the presentations that they will be making, but for their patience at having sat through this morning.

I'm not as apologetic for that as I normally am because I have the feeling that they probably learned as much this morning as we did.

This is a rare occasion in the Senate in that we learned something from some of these hearings.

Very often, that's not the case.

I'd better quit there before I get in any deeper.

Let's welcome the panelists here this afternoon:

Peter Lynch, who is the vice chairman of Fidelity Management and Research Company;

Matt Andresen, president of the Island ECN, Incorporated, and we've had lots of conversations about ECN in the Banking Committee within the last day or two;

Kathy Behrens, who is a member of the board of directors of the National Venture Capital Association;

James Glassman, DeWitt Wallace Reader's Digest Fellow at the American Enterprise Institute; and

Dr. Daniel Callahan, who is co-founder of the Hastings Center.

As those following the hearing will recognize, this morning, our first panel was basically patients and physicians. Our second panel was made up of executives and administrators connected with the delivery of health care and the creation of innovations in health care.

This panel is dealing with those who finance all of this activity.

We heard in this morning's presentation the comment made that among America's advantages around the world with respect to health care, is the fact that we have a pool of venture capital available that is unique to this country and unavailable in other countries.

I've done business in other countries prior to coming to the Senate and I know how true that is. In most of the other countries of the world, such venture capital as is made available comes almost exclusively through the banks, and with that capital comes the kind of bank mentalities and cultural attitudes that sometimes are not compatible with the entrepreneurial spirit.

So, with that, we will start, Mr. Lynch, with you. We appreciate your being here and very much look forward to your testimony.

Panel III

STATEMENT OF PETER LYNCH, VICE CHAIRMAN, FIDELITY MANAGEMENT AND RESEARCH COMPANY

Mr. Lynch. I appreciate that.

This morning, we're talking about putting a face on the health care industry.

And just as some of the other people spoke today, and Senator Mack did, my father contracted cancer when I was seven and died when I was ten.

My older brother got encephalitis when I was two and he's had a difficult last 59 years.

And my mother died of cancer.

So I am definitely interested in curing diseases for my wife and potentially for my children, and for myself and everybody else.

A lot of the things I was going to deal with was treated much more effectively this morning. So I think I might just go into some general points about research and the National Institutes of Health (NIH) and what makes my country different.

There's a chart over here I'm going to get to in a minute.

But I think one number that's fascinating to me, it's very simple to look at is, in the decade of the 1980s in the United States, the 500 largest companies in America eliminated three million jobs.

The 500 largest companies eliminated three million jobs. And we added 18 million jobs in the United States.

In the decade of the 1990s, the 500 largest companies in the United States will eliminate another three million jobs. And we're going to add between 17 and 18 million jobs this decade.

Now contrast that with Europe.

We have about 250, 260 million people. The European Union has about 350, 360 million people.

In the decade of the 1990s, they're going to add zero jobs. There's less people working in Europe in the year 2000 than 1990. Their 500 largest companies are eliminating jobs just like our big companies are to get more efficient. But they don't have the growth in small- and medium-sized companies.

All the growth in America has been companies with 500 employees or less.

So that's what's really made a difference in this country. And a lot of these companies have a technology basis to them. The technology that has been spent by the National Institutes of Health, the National Science

Foundation, the Defense Department in the 1950s, 1960s and 1970s has been one of the legs of this great, great economy that we've had for the last 15, 20 years.

In fact, in the last five years, a lot of average folks, people of less means, have begun to share in it because it's been spread wide around.

And I think this chart here shows a danger zone. This is what U.S. research and development (R&D)—this is funded by the government. This is Federal R&D. And this peaked at around, it averaged around 1.7. It actually got up to 1.9 one year.

But in rough numbers, it peaked in the early 1960s to 1.7. It's now down to 0.7.

So if this was the chart of a company that I owned, I would not want to own a company if it had this kind of a trendline.

This is one aspect of spending that really has a payback. And it has a massive payback in terms of jobs, in terms of taxes paid.

Now offsetting that to some degree, but they're mutually exclusive, spending by businesses has gone from 0.7 to 1.7 percent of Gross National Product (GNP).

Senator Bennett. This chart is just government GNP?

Mr. Lynch. That's just government.

Senator Bennett. Right.

Mr. Lynch. Now the catch is that private industry and public companies have gone from 0.7 to 1.7. That's terrific and that's very helpful. But they're different audiences. They use the kernels of information and all of the basic information that's generated by this.

All the gains made here, like transistors and integrated circuits and the Internet and lots of other things, are generated by spending on pure research by the Federal Government, the National Institutes of Health, is taken by those people and put into applications that lead hopefully to profits down the road.

So that great spending that was done over the last 20 or 30 years has helped us. It's not the total answer. We have a very good venture capital system. We have a very good banking system in America.

They'll lend to people. All they want is the money back. They kind of insist on that. They'll lend to people. Whereas, in Europe, they want to know who your grandparents were, who your parents were.

So we have a good banking system. We have a very good public market here. We've had 3000 come public the last six years in America.

I don't think 3000 companies have become public in Europe since Charlemagne. I think he became king of the Franks in 788.

So it's been a long time.

We have a different set-up here. One of the legs of the stool is this spending by the government. But that's a very scary chart.

I would like to reverse that.

The gap is dramatic. That's something that, if we doubled the National Institutes of Health budget over the next five years, it would turn that upwards. It wouldn't reverse that. It would take a long time to do that. Maybe if we doubled it every five years for the next 15 years, we'd get it back in the right place.

That's an important chart.

One thing that I think shows that is in scientific papers that are filed with patents. When somebody files a patent, they have to get approval. They have to show scientific papers.

Seventy-three percent of the citations come from documents financed by Federal spending, not by industry. These are patents filed by IBM, patents by Xerox, patents filed by pharmaceutical companies.

Seventy-three percent of the citations come from public science, research performed at universities, government labs, and other public agencies financed by the government.

That number is higher than it was before. The citations are higher—they've tripled.

So what we're doing, what Washington is doing, is the best investment we can.

The next chart is the important one—you have to turn it upside down. But it shows some of the great breakthroughs. We listed many of those this morning, things that—I think you may have to turn it the other way.

There's a long list of what—sometimes we take for granted the great things we have, like artificial hips and artificial knees, that have made a difference.

Somebody had to come up with that. The influence of vaccines saves four million lives a year. But people with artificial hips and knees can go back to work.

And the people that are out of work and are chronically impaired, it's a massive number for our economy.

A person that's over 65 that's not under disability spends about the same on health care as someone that's 40. It's really when you get to be disabled, that's when the money really starts to run up.

These are the items that have kept people from dying, keeping from being sick. The list goes on and on. Those are just some of the key items.

And there's more coming. Those are the ones that are in place now, more in the future.

This is a big plus for people—less pain, less grief. But it's productive.

And the final element I want to bring is that this year, biotechnology companies alone are going to pay a billion dollars in income taxes—one billion.

For about 20 of them, almost half of it's going to come from one company, Amgen. But they're paying real income taxes to the Federal Government and their employees obviously pay taxes.

But I think the next two or three years, perhaps the next four years, that number is going to triple.

There's about 30 companies that are in the final stages of clinical trials that are going to have products out there. So I think that billion dollars of income taxes is going to go to three or four billion dollars.

So the money that the Federal Government puts out, they get back in products, lower health care costs, healthier consumers, happier citizens, and cash.

So as an investor, that's what I like.

And statistics, like some of these major studies where you're talking about billions and billions of data points and sending information on proteins, we're talking about billions of pairs in some of these sets that they're trying to study. You need these vast computers.

The vast majority of people wanting this time are being turned down from lack of resources at these central information bureaus because they just don't have the resources.

So as much as we've expanded the National Institutes of Health, there are major projects that are being delayed or totally can't be done because of lack of funding.

So I want to point out from people that I talk to that if this money was put out there, it would be well used.

Thank you.

Senator Bennett. Thank you.

Mr. Andresen?

STATEMENT OF MATTHEW ANDRESEN, PRESIDENT, THE ISLAND ECN, INC.

Mr. Andresen. Thank you.

I would first of all like to commend the Chairman and the members of this Committee for holding these hearings concerning the importance of biotechnology companies and the central role they play in improving our quality of life.

And of course, I appreciate the opportunity to appear before this Committee to testify about my specific views on venture capital, the

biotech industry, and in my mind, their dependence on an efficient market place.

My name is Matt Andresen and I'm President of the Island ECN.

Island is an automated stock market. It's a trading system for equity securities.

What it does is, by serving right now about 207 brokerage firms, it gives these firms the ability to publicly display the orders to buy and sell stocks and to match them.

This is a new business model that was made possible by sweeping Securities Exchange Commission (SEC) progressive regulation in January of 1997. From that time, which we consider to be our birthday at Island, we have gone from no volume to now over 120 million shares a day, accounting for about 12 percent of all the transactions on Nasdaq.

And as we're fond of pointing out, this will all be done, all this information will go across a piece of equipment no bigger than a suitcase.

So we think that this kind of innovation that has been made possible by the SEC's progressive regulation has helped fuel the public interest in the market place.

One thing that we contend at Island is that the tremendous interest, public interest, in the stock market in the last several years is due not only to the fantastic returns that people enjoyed lately because, in fact, these returns can trace their way back to 1991 and 1992, but in fact also to the public realization that they can receive a fair shake in the market place.

In 1995, the Critzy-Schultz study released by Vanderbilt University, the successful billion-dollar class-action lawsuit against the Nasdaq market place, and the SEC's 21(a) report, which detailed abuses of collusion and price-fixing in the Nasdaq market place, all served to expose and undermine the public's confidence in how their orders were being transacted.

With the SEC's regulation, Island has brought true competition to this market place.

Spreads have plummeted. Those are the differences between what people buy and sell to trade the same security. By the NSD's own account, this has saved investors hundreds of millions of dollars.

Of course, we're all aware how commissions themselves have plummeted. The days of having to pay $500 a trade in commission are gone.

You can now choose to pay as low as five, ten, or fifteen dollars.

What this has done is really not only increased people's access to the market, but really increased the number of people who have access to today's market place.

We think there's been a tremendous empowerment of the American people, the ability to participate in their own capital market.

We think this has been a key generator of capital for start-up companies.

We're here today because one of the integral sectors of our economy, technology, is having relative difficulty raising capital. In fact, capital raised through biotechnology, initial public offerings or IPOs, declined 48 percent in 1998.

Now there has been some market improvement so far this year, but, really, we think that one possible explanation is the rash of, as Mr. Lynch already referred to, very early-stage public offerings from Internet and software ventures.

These ventures that don't need FDA approval or other such government approval can raise public money very early.

And what we've really seen is really a seachange in the perception of venture capital. We now see really public venture capital, companies going public and offering ownership to a much wider range of people as would previously have been possible through just simply venture capital companies.

This has certainly been a tremendous boon to the Internet economy, but we must be concerned about the health of the biotechnology sector.

We think at Island that the creation of a very efficient, low-cost and accountable post-secondary market really increased the accountability for this.

We always say that, three years ago, the accountability on Wall Street was you call your broker and you say, jeez, I'd really like to buy Dell. And they say, okay. Where would you like to buy it?

And your answer would be, you're the broker. You tell me. Perhaps your only accountability after you found out it was traded at 38 was to look in the paper the next day in *The Wall Street Journal* and say, hey, it traded between 37 and 39. I wasn't openly misled. It looks okay.

Now of course the conversation with the broker might very well go—jeez, Broker Bob, I placed an order at 38-1/16th and it traded down to 37-31/32nds. My limit order of 37 didn't get hit.

What's going on?

And while the brokers might say that this is certainly the passing of the glory days, it really is the glory days for the American investors, in that they can have the ability to take control of their finances.

This has fed tremendous interest in the markets and really brought tremendous capital to the markets, which has fed the flood of IPOs.

We hope to have the biotechnology industry in the future have just as fair a crack at that.

Thank you.

[The prepared statement of Mr. Andresen and Mr. Andresen's response to Senator Bennett appear in the Submissions for the Record.]

Senator Bennett. Thank you very much.

Dr. Behrens?

STATEMENT OF M. KATHY BEHRENS, MEMBER, BOARD OF DIRECTORS, NATIONAL VENTURE CAPITAL ASSOCIATION

Dr. Behrens. Thank you.

By way of background, I'm managing director of Robertson Stephens Investment Management in San Francisco. I am also chairman of the National Venture Capital Association (NVCA), which is comprised of more than 330 professional venture capital firms which are dedicated to stimulating the flow of equity capital to emerging growth and developing companies.

Also background, I have a doctorate degree in microbiology from the University of California at Davis, where I performed genetic research for six years.

Since that time, I have focused most of my 20-year investment career in the life sciences area, in particular working with biotechnology companies.

In fact, over the past 15 years, I started up biotechnology companies and served on the boards of directors of many of them. These include companies such as Protein Design Labs, which today sells a novel treatment for life-threatening ailments associated with kidney transplantation, COR Therapeutics, which markets treatments for a range of acute cardiovascular events, including heart attacks, Abgenix, Incorporated, which has developed fully human monoclonal antibodies that are being developed to treat bone marrow transplant patients, a range of auto-immune diseases and cancer.

These companies and others in the Robertson Stephens portfolio illustrate that biotechnology is giving new and renewed hope for a wide range of people who suffer maladies across the entire spectrum of diseases.

In fact, without patient investment from venture capitalists, the industry would not exist today.

According to the NVCA statistics, since 1990, venture capitalists have invested over $5.8 billion into over 550 biotechnology companies.

While companies in California, Massachusetts, Washington State, Pennsylvania, and New Jersey have received the largest share of these dollars, biotechnology companies in 34 states have received venture financing.

Venture capitalists invest on average over ten million dollars in each biotechnology company that the industry funds.

This has been very fruitful.

There are currently 79 biotechnology therapeutics and vaccines approved for sale by the Food and Drug Administration. Three-hundred and fifty biotechnology drugs, vaccines and therapies for conditions such as cancer, heart disease, diabetes, arthritis, AIDS, genetic diseases and many others, are currently in clinical trials.

Most of these companies have been financed with venture capital.

However, there are serious and profound problems facing us right now. While the venture capital industry is investing more money in more companies than ever before, the amount of money going into biotechnology as a percentage of the total dollars disbursed has declined.

In 1990, more than $300 million, or eight percent of the total disbursed venture capital dollars, went into biotechnology.

In 1999, that number will likely exceed $1.1 billion.

However, that will reach barely three percent of the total $30 billion that will be disbursed by the venture capital industry this year.

As venture capitalists increasingly pursue Internet deals which have the ability to garner a significant financial return in a relatively short timeframe, the biotech arena, which faces heavy government regulation and requires long-term and more patient investing, naturally suffers.

It is likely that in 1999, fewer new biotechnology companies will be funded by venture capitalists.

Also, privately-held biotechnology companies are having problems securing additional needed money to get through the FDA market approval process.

The market for biotechnology initial public offerings as already described, declined from 22 in 1997 to six in 1998, and 1999 does not look particularly optimistic, either.

To date, seven such companies have come public compared with over 200 initial public offerings for Internet-related companies.

Does the Federal Government have a role in seeing that this trend does not continue?

We believe so.

At this moment, and in this very building, significant debates are taking place regarding health care reform, patent reform, tax policy, immigration policy, and research funding for related fields.

The results of these debates may directly affect the future of biotechnology companies and in turn impact the availability of the innovative products these companies are developing.

As a seasoned venture capitalist, I can attest to the enormous risks biotech companies face in an attempt to bring a product to market.

Health care proposals which impose drug price controls or Medicare drug benefits which provide marginal reimbursement can create a perception or reality that our potential return is limited or at greater risk.

The biotechnology community has for years been asking for patent reform, only to get bills passed in the House and have them not acted upon in the Senate.

The issue is simple—we need to make certain that the new 20-year GATT patent term does not end up shortening the terms of patents when the government causes delays in the issuance of a patent.

Last month, the House passed the American Inventors Protection Act by a vote of 376 to 43. The NVCA hopes that the Senate will also act quickly.

Advances in biotechnology will only occur with a highly educated workforce. Education reform is critical, but it will not occur overnight.

In order to ensure the continued dynamism of the biotechnology industry, Congress needs to increase the number of H1-B visas available. New visas will be available shortly with the commencement of the new fiscal year, but this relief will only be temporary as the number of visas we need to keep our companies running is much larger than the number of visas available in the upcoming year.

The biotechnology industry currently is undergoing tremendous consolidation through mergers and acquisitions, which on the whole is positive for the industry.

Pooling has been the most desired method of structuring acquisitions, but the Financial Accounting Standards Board has proposed eliminating pooling of interest accounting.

By limiting our ability to use mergers and acquisitions as an exit strategy, you may limit our ability to invest in biotechnology.

I understand that the Senate Banking Committee is looking at holding hearings on this issue and I would urge you to support this effort so that you can hear at least the reasons why changes in pooling

accounting could very well limit the amount of capital invested in biotechnology.

It is encouraging that the government is choosing to increase support for life sciences research at a time when the private sector is concentrating its resources in other industry areas.

However, biotechnology requires considerable capital investment, in part, because many disciplines are required to develop these sophisticated therapies.

This trend is accelerating and today, often life sciences expertise must be supplemented with many other disciplines, including information technology, materials science, basic physics, engineering and others.

It's concerning that these cross-disciplinary interactions are becoming critical to advancing new life-saving therapies. Research support has declined in many of these other fields.

This is compounded by the fact that many of these fields were responsible for the discoveries that led to the information technology revolution that's contributing to this robust economy today.

Finally, I want to thank you for holding these hearings. Your effort to highlight the contributions that biotechnology has already created in our society, as well as the problems we now face, will help us all move forward.

Thank you.

[The prepared statement of Dr. Behrens appears in the Submissions for the Record.]

Senator Bennett. Thank you very much.

Mr. Glassman?

I should point out that Mr. Glassman is one of those whose writings I read rather regularly.

We welcome you here.

STATEMENT OF JAMES K. GLASSMAN, RESIDENT SCHOLAR, AMERICAN ENTERPRISE INSTITUTE; CO-AUTHOR, DOW 36,000

Mr. Glassman. Thank you, Senator Bennett.

Thank you for inviting me to give testimony at this very important biotechnology summit.

I am honored to join you today and on a personal note, especially honored to be on the same panel as Peter Lynch, who has been a hero of mine for a long time.

My names is James K. Glassman. I am a resident fellow at the American Enterprise Institute, a Washington think tank.

For six years, ending this past June, I was a regular columnist on financial, economic and political matters for the *Washington Post.*

Next month, I begin a financial column for the *Reader's Digest*, the world's largest circulation magazine.

With *The Economist's* Kevin Hasset, I am co-author of a new book on the stock market, Dow 36,000, which has just been published by Times Books.

Senator Bennett. We will not let Mr. Lynch comment on how likely that number is in the next 12 months.

(Laughter.)

Mr. Glassman. I wish he would.

(Laughter.)

You've heard today about the wonders that the biotechnology industry has already produced and will certainly produce in the future.

I want to talk to you today about one of the elements that's essential to its success, and in fact the survival of that industry.

And that is capital, which is a fancy name for money for investment.

The biotechnology industry needs capital to fund its research and that capital is not easy to come by. You just heard specific testimony about some of the problems that face biotechnology and medical devices and you're going to hear more in the next panel, including testimony about what I believe to be a lamentable lack of understanding both at the political and the venture capital level of where innovation comes from and just how important it is.

But I would like to be much more general.

This is a nation of wonderful entrepreneurs, hard-working scientists, great managers, fabulous ideas. But they can't bring products to the market place without capital.

And where does that capital come from?

Lately, much of it has been coming from abroad. For the past 17 years, while the U.S. has undergone the single greatest round of prosperity in its history, capital inflows for portfolio investment purchases—that is, stocks and bonds—have increased at an average annual rate of 19 percent. That's from abroad.

Foreign direct investment has increased at an average annual rate of 15 percent. Amazing figures. Net foreign inflows to this country—$4.8 trillion over this period.

To put that into perspective, that's two and one-half times the annual federal budget and about half of our annual GDP—a big, big number.

But this strong flow of capital will not necessarily continue unabated.

Right now, the U.S. is attracting capital because of our sensible regulations, decent tax rates, which you'll see in a second, could become more decent, just as regulation could become much more sensible.

But compared to the rest of the world, we're doing pretty well—our low inflation and our entrepreneurial culture.

But Europe and Asia are developing along similar lines. They're beginning to undergo the same kind of managerial revolution that we did in the early 1980s.

In other words, the rest of the world will have other places to invest as time goes on.

Now what about capital from within this country?

There the country is not as bright as it could be. A major source of capital is personal saving, which the Bureau of Economic Analysis defines as the difference between after-tax income and expenditures.

Unfortunately, the official household saving rate fell in May to its lowest level in recorded history—minus 1.5 percent.

The latest number, a slight improvement, is minus 1.4 percent.

To give you an idea of how low this is, in 1974, when Congress created IRAs to help boost the incentive to save, the saving rate was 9.5 percent, positive 9.5 percent.

Why is our saving rate so low?

Well, one immediate reason is a little technical. After-tax income does not include the capital gains that Americans earn on their stock and bond holdings.

But frankly, spending those gains is probably not a very good idea, anyway. If you don't believe me, listen to Peter Lynch.

They should be reinvested.

Also, Americans simply like to consume. It's their money. They should be able to do with it what they want.

Right?

Well, yes and no. Americans are not saving enough. And more importantly, they are not investing enough in biotechnology and in other equities—that is stocks—for two reasons which I believe can be remedied.

The first is the subject of our book, Dow 36,000.

We argue that Wall Street's analysts, media pundits, scholars, have scared millions of Americans away from the stock market with continual talk of bubbles and imminent crashes, talk we have heard all the way

from 1982, when the Dow was at 777, all the way up to the present, with the Dow over 10,000.

These frightening judgments are based on a flawed model of stock valuation, a model that we believe has been repudiated by the facts.

And in our book, we offer a new model and we encourage Americans to invest in the stock market in a prudent, long-term fashion with a sensibly diversified portfolio that may indeed include mutual funds.

The other reason that Americans aren't saving and investing enough is that public policy tends to deter it. And that is the main message that I would like to leave with you today.

Americans are not investing because we have a tax code that encourages consumption over saving. We tax the earnings of corporations, then we tax the dividends they pay, then we tax the income from the dividends and the capital gains that result.

And then, as if that weren't enough, we tax the estates of people with the prescience and the discipline to invest for the long term.

Now what kind of system is that?

Yes, we have IRAs and 401(k)s. But they are burdened with all sorts of restrictions.

And let's not forget Social Security. Including the employer's portion, ten percent of the salaries of working Americans go into a system that produces returns of only one or two percent.

If just a small proportion of that money could go into the stock market, Americans could develop real wealth and get a taste for investing that would certainly lead to a higher saving rate.

In fact, I believe that even President Clinton's USA accounts represent an impressive step in the right direction.

Indeed, as I see it, the most urgent economic issue on the policy agenda is wealth creation—half of Americans have no wealth at all, no significant bank account, no stocks, no bonds, nothing to pass on to their kids, nothing for a decent retirement, other than Social Security.

That is a shame.

For most Americans, the best chance for a comfortable retirement, in many cases the only chance, is stock market investing.

The flip side to this deficiency is the lack of capital that I discussed earlier. In the case of the biotechnology industry, capital saves lives. There is no substitute for it and the need is urgent.

Thank you.

[The prepared statement of Mr. Glassman appears in the Submissions for the Record.]

Senator Bennett. Thank you very much. Dr. Callahan?

STATEMENT OF DR. DANIEL CALLAHAN, CO-FOUNDER, THE HASTINGS CENTER

Dr. Callahan. Thank you.

I won't talk directly about capital investment. I run a nonprofit organization.

But I will say one remarkable thing about the United States is that we have so many nonprofit organizations which other countries don't have and they require a kind of capital investment for people who believe in that sector as well.

I really want to take up the question of biotechnology and the public interest and pick up some themes from this morning.

I think one issue that's going to be important in the years ahead will be the general ethical reputation of a biotechnology industry and the general perception that it is making some very significant contributions to the overall public good.

I think that reputation is going to be affected by two important elements.

First of all, the question of the safety and the social impact of the biotechnology developments. And secondly, to touch on an issue from this morning, the question of the economic affordability and sustainability of what that industry produces.

Let me just say something briefly about the question of safety and social impact.

There are some fears out there in the public about biotechnology, particularly genetic engineering. Some of them are irrational. Some of them are reasonable worries.

As we well know, there's certainly a gap between the United States and Europe on the question of genetically-modified foods, an interesting and significant kind of debate.

In any case, biotechnology is not looked at in quite the same way as many other technologies are.

It seems to touch on something deep in human nature and it worries people, at least many people.

I think the thing we mainly have to fear in biotechnology is that we certainly don't want to see irreversible genetic or other biological changes introduced into human life or animal life or plant life.

We don't want to see, I believe, damaging social changes which might be theoretically changeable, but might change family and child-bearing and other patterns in ways that we would find a threat to some traditional values.

And finally, I think we should worry about changes that might suggest human beings are nothing but machines to be manipulated at will and thus, have a kind of lost sense of our human dignity.

There's a lot to be said on that, but I won't say any more.

I really would like to say a little bit more about the issue of the affordability of biotechnology, at least the products of biotechnology.

I think one thing that Americans don't recognize is that every health care system throughout the world is having some kind of a crisis with health care.

We worry about our lack of universal coverage, the difficulty of paying for it. But it's rather striking that reform efforts are everywhere afoot, regardless of the kind of health care system other countries have.

And one of the major reasons for that is the impact of very expensive technological change and innovation. It's the combination of an aging population, heavily burdened with chronic disease, and a combination of throwing a lot of very valuable but often expensive technologies at those conditions.

The estimate in this country is that some 30 to 40 percent of the cost increase of health care over the years can be traced to technological innovations.

And it's rather striking that when we hear about the supposed atrocities of HMOs and other organizations in not providing pharmaceuticals or other things that people want, that almost all of those debates turn on pretty recent technologies, not technologies introduced 20 or 30 years ago. It's the new technologies that seem to be giving us fits, and the new expensive technologies.

The question, then, it seems to me, is we really see a collision in progress around the world with the health care needs, on the one hand, over against the new technologies to meet those needs. The expenses of those technologies are outrunning either the capacity or the willingness of countries to pay for them.

What are we going to do about that?

And here, biotechnology becomes very important. It's going to be one of the main contributors to the new technologies in the years ahead with medicine. And I think there is a very powerful reason to be concerned about the public interest perspective that the biotechnology industry takes.

One thing we certainly know from the 20th century is there has indeed been a great increase in life expectancy. But the estimate is that no more than 30 or 40 percent of that increase can be traced to

improvements in medical conditions, medical research or health care delivery.

It can be mainly traced to improved public health measures, better diet, change in lifestyle habits, higher education, higher income.

Those are the main things that affect public health far more than even the very best technology.

So, to me, the interesting tension in the years ahead is going to be between the public policy perspective, on the one hand, and say the biological perspective on the other, which is to go after the disease, genetically or otherwise, over against trying to change people's living habits.

And just as sometimes there's talk of an industry-government relationship, it seems to me the great challenge in the years ahead will be to find some kind of good working relationship between the population perspective and the public health perspective and the biological perspective as very much exemplified in biotechnology.

I think the great challenge to the biotechnology industry in the years ahead will be to develop products that are affordable. And that is to say, affordable by the population as a whole and will not create constant fits in the health care systems.

A number of the patients this morning were talking about, were really complaining about, the sad situations they had in trying to get things from their HMOs, and were denied that.

But it takes two to dance in that particular situation. Namely, the question is, well, why weren't they getting it? Is it just nasty HMOs?

Well, possibly. But one reason of course is that the HMOs are trying to live with their own limited resources. They can't give everybody everything that people might like to have and, usually, they are forced to do some rationing and they typically ration these more expensive new technologies.

So I think biotechnology has before it the real challenge of trying to figure out how to really advance scientifically this industry, how to make a powerful difference with health. But how to make a population difference.

It can obviously do much for individuals—relieve pain and suffering, increase life. But how do we make it begin having the kind of effect that, say, the large public health measures over the last century have had?

And that seems to me, if that doesn't happen, then biotechnology is likely not to have the impact that it could have and probably make our health situation worse rather than better.

I think, for instance, right now, we have a very expensive AZT cocktail, very effective in prolonging life of people with AIDS and HIV positive people.

It costs $20,000 to $30,000 a year.

Imagine a kind of Alzheimer's cocktail analogous to AZT, where you have four million Alzheimer's patients who might find suddenly to be confronted with the possibility of also a very expensive disease.

But when you're talking about four million people times $20,000, $30,000, $40,000 a year, you're into very big numbers. And that, I think, would create a kind of terrible medical tragedy and social dilemma in trying to figure out how to do that.

So I suppose the message I would like to leave finally is that biotechnology, as it tries to develop its project, has to really constantly keep before its eyes the question of what is going to be the economic impact of these developments?

Will they be affordable? For instance, I would love to see Medicare provide pharmaceuticals and drugs to everyone. But if we got that and it fell apart five or ten years from now simply because the new technologies coming on the market and the new pharmaceuticals were unaffordable, it would be a short-term victory, but a long-term disaster.

So I will end there saying, what we need is I think a sustainable biotechnology, and that means a biotechnology which societies can afford and which individuals can buy without great pain and suffering and agony.

[The prepared statement of Dr. Callahan appears in the Submissions for the Record.]

Senator Bennett. Thank you very much. I would love to gather this panel around a dinner table with no time limit and let us all have at each other because I think there could be an enormous amount of synergism come out of that kind of conversation.

Unfortunately, we don't have the opportunity to do that. Both Mr. Lynch and Mr. Andresen have to slip away fairly soon.

So let me start, Mr. Lynch, with you.

First, let's go back to your first chart. I'm delighted to see you use this methodology because I have used it in debates about the budget.

If you may recall, some of the debates we have here around the deficit and surplus and so on, show the chart that's been referred to as the hockey stick. It shows the national debt going up like a hockey stick.

Colleagues on both sides of the aisle stand there and point to that chart with shaking fingers and tell us we are on the brink of disaster because the national debt is going out of sight.

And I much prefer to use a chart that lists the national debt as a percentage of GDP—the old question of compared to what?

In fact, the highest point in this nation's history, if I can impose on you a little of my own bias in this area, the highest point in this nation's history for the national debt as a percentage of GDP was 1945, at the height of the Second World War.

And our national debt, instead of going up in that period, as it does in absolute nominal dollars, came down dramatically as a percentage of GDP, bottomed out at around—it was about 150 percent in 1945. It bottomed out in the mid-1970s at around 30 percent and then started increasing and reached roughly double that, around 67 percent in the beginning of the 1990s and has gone down subsequent to that.

So that the national debt as a percentage of GDP is currently going down quite dramatically.

So if you put it in the same framework as your chart, instead of the hockey stick going up, you would show the national debt now turned down.

I think one of the reasons that we have this reaction that you have here is that people are using the hockey stick chart. They're showing the total number of dollars going into R&D and saying, what are you complaining about? The dollars are going up every year.

And the point you're making, if I can—I'm telling you what I hear you saying and I want you to either confirm or correct me—the point you're making is that while they may be going up in absolute terms every year, they are coming down in relative terms, and that that long term is a signal we need to avoid.

Now, have I caught what you're saying correctly?

Mr. Lynch. Absolutely right. And just to add to it a little bit.

There's this issue of investing. I think student loans that the government does has a very good payback. A lot of the money spent in government is a transfer.

This is an investment that has a fantastic return. The problem is you don't know when you're going to get it. And the more important point is that the fact that independent businesses like we've had this morning or will have after this are spending a fortune. They've actually doubled their spending as a percent—they've quadrupled it in absolute terms—they're in a different area. And they're not going to make up for this.

Also in this period of time, you have pure research being done by Bell Labs that is part of a big monopoly. Now Bell Labs is part of Lucent. They're not spending the way they used to on pure research.

Senator Bennett. That's right.

Mr. Lynch. So there's another great pure research house that's doing applied research rather than pure research.

So that's the foundation. It's one of the legs of the stool. I don't know how many legs it has, our stool, but it's one of the more important ones.

The other point is I think the average person in America thinks the National Institutes of Health is part of the United Way. I don't think they really think it's a line item on the budget. I think they think it's a charity.

They don't call their congressman to say, this is really a good thing.

It gets very little credit in the media and the public. I think I'm pretty well read. I didn't know very much about it until three years ago.

Senator Bennett. Yes. Well, you will be happy to know, and I'll make this commercial on behalf of one of my colleagues, Senator Frist's bill which would double federal R&D spending, passed the Senate in August, just before the recess.

It was one of the accomplishments, if I may, of the Republican High Tech Task Force, which I have the responsibility of chairing. Senator Frist is one of the co-chairs.

I believe it still has to pass the House.

But Senator Frist, who has joined us here, even though he's not a member of this Committee, has taken on that particular crusade. And as I say, that bill passed the Senate in August.

So we're doing our best to try to hear you.

Mr. Andresen, you probably followed the hearings in the Banking Committee this week. We had yesterday the chairman of the New York Stock Exchange, as well as the head of Nasdaq.

So I won't rehash that with you. But let's talk about this whole question of—an interesting phrase. You used public venture capital.

Of course, Mr. Lynch has been in this business. And Dr. Behrens has been in this business, too.

But can we expand on that a little bit? Can you give me some statistics?

If I were back in the business that I had been in prior to coming to the Senate of trying to raise money for a little company. And I've done that for a lot of little companies. Not very many of them succeeded.

Fortunately, the ones that did made enough money to repay me for all of the failures of the ones that didn't.

But what percentage do you think now turn to organizations like yours for, quote, public, unquote, venture capital—no background, no earnings record, nothing but a prospectus, that normally would go to a

venture capitalist like Dr. Behrens on bended knee, and now go to their PC and their Internet and do the begging electronically?

Can you give me any sense of percentage of the difference between private placements of venture capital and public placements, and where you think that percentage is going?

Mr. Andresen. Well, Senator, I do not have any specific numbers of that, but I will be happy to get those and submit them for the record.

I certainly can reference the comments by Dr. Behrens about the amount of money that has been poured by the public into Internet IPOs in the last year.

I know that my company itself, although not a public company, has been the recipient of venture capital money. Our core business turned out to be actually profitable in 1998.

However, given, obviously, Chairman Levitt's comments as we all heard last week, there are certainly a lot of new opportunities out there. And obviously, the Senate Banking Committee has been integrally involved in those new opportunities, basically to create for-profit stock exchanges.

We think this presents a tremendous opportunity for Island. We have filed to become a stock exchange, but needed the capital to do it.

We did not pursue the public markets before. We did not feel it was appropriate before our exchange filing. So we went into the venture capital market and raised $30 million towards financing that degree of infrastructure.

I think that one thing that's been very helpful to a lot of companies that are traded on Island, about 3,700 stocks a day transact on Island, is that the ease of entry and exit into the market place by the individual investor.

I think part of that has been a revolution from companies like Fidelity, through mutual funds, but also through self-directed investment, is really the seed of the tremendous influx of capital into the market.

Again, I would assert that much of the increased interest in the market, as you put it, is the people at home with a mouse and a computer screen in the evening, are now participating directly in a way they never did before, both because of the reduced cost and the tremendous emancipation of market information, which companies like Fidelity and Island have been integral in doing.

Senator Bennett. Mr. Lynch, do you see this as cutting into Fidelity's customer base, as everybody says they don't need your advice any more and they'll sit in their own den and make their own decisions?

Mr. Lynch. At one point people bought stocks on their own. At one point, you used to retire and get 70 percent of your last year's salary for the rest of your life. You didn't have to worry about the stock market or the bond market.

Now many companies don't have pension funds. They have early retirements. You have to save for yourself now. You have to say, I have to do it. If I do a bad job of it, I have no pension.

So I think people are making up their mind now. I didn't think about retirement in my 20s or 30s.

People are now doing it earlier on. So they have to learn the information. I think the Internet is a helpful tool for learning information.

Whether they buy stocks directly or they buy funds, in reality, that does not provide, unless IBM does the secondary or a bank does the secondary, it doesn't provide money for new venture capital. It just basically—a person in Arizona sells IBM, a person in Florida buys it, as you know.

Senator Bennett. Right.

Mr. Lynch. We're not in the business of providing money to venture capital, which really creates the growth in the business. These other people and people that follow us are in the venture capital field.

I think this business is just to maybe get the people to buy it a little cheaper.

Senator Bennett. It's true that you're not in the business of providing venture capital. But I can tell you from painful personal experience that if Fidelity decides your offering is a good one, that gives you the Good Housekeeping seal of approval and makes it more likely for other people to buy the stock.

And you did, and I prospered. And then Fidelity decided our stock was a bad one and you sold it, and a lot of other people followed your lead. And I disprospered—or whatever the appropriate alternative word is.

So I think you have a bigger impact on these emerging companies and IPOs than you may be giving yourself credit for.

Mr. Lynch. We're a medium. The money flows to us. Obviously, if there's a strong stock market for 10 or 15 years, a lot of money is going to get to places like Fidelity and then venture capital people have a way to exit, at least when they come public.

We're the natural buyer, people like ourselves.

So the market was 777 in August of 1982. So we've had quite a rally since then. A lot of money has come into the market. So it's helped.

That's why we've had this harvest in the venture capital field. A lot of these companies have come public and done very well, like Staples and Federal Express and Amgen—it's two one-billion dollar drugs. It's been a very successful company.

There's a lot of success stories there and it's helped grow this country.

Senator Bennett. Let me go, Dr. Behrens, to some of your comments.

I don't know if you followed the first hearing that we had when we had two days of this. Bill Gates, Lou Gerschner, people of that stature came in and testified.

We heard much of the same thing from them that we have heard from you, about the need for H1-B visas, about educational reform, and so on.

And many of those things, again, we have passed in the Senate. Unfortunately, a large percentage of them were in the tax bill that President Clinton decided he had to veto, for whatever reason.

And I will not here get into my characterization of his characterization of what that bill really said or really was.

But we have at least passed most of the things that you outlined here in the Senate and they have fallen subject to a presidential veto, which we don't have the votes to override.

I think you will find the members of this Committee anxious to pass them again, perhaps in some form that the President might find a little more acceptable.

Let's bring Mr. Glassman in. I'm not forgetting you, Dr. Callahan, because you're part of this. But we're recognizing that these two may have to go soon.

You talk about the savings rate. Help me understand how that is measured. Let us say that I am a schoolteacher. We talk about putting a personal face on this. I will put a personal face on this.

My sister, who worked for Montgomery County schools for many, many years, did not have much of a pension out of that school system, knew it going in in advance, and knew that she had to invest.

It fascinates me, when I go to my sister's house for dinner and after dinner, she and her husband are nervously looking at their watches because there is a television show they want to watch.

It's, gee, we're glad to visit with you, Bob, but do you mind if we turn on the TV. And I say, not at all. And it's Louis Rukeyser.

My sister, who got her degree from Wellsley in English, and who corrected papers all of her life, is following the stock market very, very assiduously.

Now, she didn't do it with a high savings percentage. She took her lump sum out of what she got from Montgomery County, got a little inheritance from my parents, and put it into the market.

Is that combination considered part of your savings rate, or would that statistically fall out of these discouraging numbers that you've given us?

Mr. Glassman. It would in fact, Senator, fall out of the discouraging numbers.

I think it is important that we don't exaggerate how low the savings rate is. It's a very crude measurement. All they do is take after-tax income—so it would be the salary that your sister made minus her taxes this year—and subtract from that expenditures.

So if, for example, she had some capital gains—let's say she owned stock that went up and she decided to take those gains and use them for expenditures, to buy a new television, then it would appear that she had a negative savings rate, when in fact, let's say she had $100,000 in the stock market and last year, on average, that would have risen by, let's say, 20 percent or $20,000.

Senator Bennett. She'd be delighted.

Mr. Glassman. Her wealth increased.

Senator Bennett. She'd be delighted to hear that she had $100,000 in the market.

She's nowhere near that.

Mr. Glassman. Well, anyway. I don't know how much she has.

But, at any rate, what the savings rate does not measure is the increase in wealth.

And that's why it may be— and I readily concede this—a more pessimistic figure than it is in reality.

But I do think that the trend is not good. I also feel very strongly that most Americans, when they build up extra wealth as a result of the increase in the stock market or the increase in the bond market or the value of their home or that sort of thing, that that's something that they shouldn't fool around with until they retire until they need it.

So it is a good idea to actually save—let's put it this way.

It's a good idea to spend less than you take in, as Mr. McCauver said many years ago.

Senator Bennett. Yes.

Mr. Glassman. And that's a trend that is disturbing. And it's also—my main point is it is a trend that you on Capitol Hill I think can do some things to arrest, to the great benefit of the biotechnology industry as well as to the benefit of Americans.

I just worry about the fact that so many Americans have no wealth at all. And our public policy militates against it.

Senator Bennett. I don't mean to be unduly autobiographical, but you remind me about a comment my mother made after my father retired.

My mother was complaining—we spend everything we earn. We're not able to save any more.

And my brother said to her—Mother, the purpose of saving is to take care of yourself when you're old. I don't mean to offend you, Mother, but you are old.

Start spending it for the first time in your life.

(Laughter.)

That was a very difficult cultural thing for her to understand. A woman in her late 80s, she lived until she was 96, and left, fortunately for me and my sister, a nice little estate. We would have been just as happy if she had spent it. But, culturally, no, no. She had to be saving in her 90s against her old age.

I think, culturally, we don't have that in America today.

Mr. Lynch. I would add, Senator, that last year, 68 percent of Americans own their homes today. It's the highest ever in this country, the second highest in the world.

It's not in those numbers, as Jim knows. If your house goes up in value, it's not in your savings.

If you put a kitchen on, if you put a bathroom on—

Senator Bennett. That's consumption.

Mr. Lynch. That's consumption. Whereas, you probably raised the value of the house somewhat.

So people figure this out pretty fast. It's kind of insulting when you fill out your income tax return. There's a form called unearned income. You have to pay taxes quarterly. You have this thing called unearned income, like you really didn't deserve it.

It's interest on the bank and interest on dividends. It's an insulting preface to the income—you really didn't deserve it.

People pay taxes on their bank account. But if they borrow money to second mortgage and buy a boat with it, it's tax-deductible.

So the system is a little rigged to discourage savings, as Jim and other people pointed out.

Senator Bennett. Dr. Callahan, let's get a little bit back to the biotech industry.

Thank you, Mr. Lynch. We appreciate your being here very much, and your patience.

I got carried away with the excitement of this other area.

You posited the idea of four million people with Alzheimer's and the very, very high cost.

As a businessman, my reaction would be, if you have four million people to spread your R&D costs over, as opposed to 100,000 or so, you can bring that cost down very, very rapidly and make it available at a low cost.

One of the concerns we hear, and this is anecdotal and, frankly, rumor, so I'd like you to comment on it, is that we've run out of low-cost, cheap solutions.

For example, Dr. Lister teaches everybody to sterilize themselves in hospitals and they bring the cost down very dramatically, and that didn't cost him anything.

And that, increasingly, and you may know this as well, Dr. Behrens, from your background, increasingly, I'm told the medical breakthroughs are for a relatively limited population, and therefore, the economies of scale that can be achieved by a drug company spreading the costs over millions of people using the drug as opposed to 100,000 or so, are simply not there.

But the cost of developing the drug was just as high for a population of 100,000 as it is for four million. And therefore, that's one of the reasons why HMOs and others are saying, gee, we're not going to get into reimbursing for these very high-cost items because the population is so small, that we simply can't afford that.

Now, do you have any background to allow you to comment on where that would be?

Dr. Callahan. Well, I think in the first point about the technology, there's no doubt that the cost per unit of a technology would go down if you can spread it over a large population.

But the typical history in health care has been also that you increase the size of the population at the same time. If you bring the cost down, you actually bring more people into range.

Senator Bennett. That's not just health care. That's economics.

Dr. Callahan. And the difficulty in years ahead, the fastest-growing age group in this country are those over 85 who are most at risk of Alzheimer's.

And the estimate is something close to 50 percent of people over 85 will probably have some degree of dementia. And that means the number is going to grow.

We have four million people now with Alzheimer's. That number is going to grow significantly.

So that even if we bring the costs down, we could still have problems.

I guess the main problem, though, is where are we going to start? Are we going to start with the very expensive treatment and hope the costs come down? Or will we find something less expensive?

That to me is the great question.

The problem is of course, interestingly, we do have inexpensive ways of improving health. We could have people exercise more, eat better diets.

The interesting thing is that those statistics are going the wrong direction. We're having an aging population who could clearly benefit from better diets, from better living habits. But exercise is down. Obesity is up.

That's pretty cheap stuff to correct. But we somehow don't know how to do that.

The behavioral changes seem to be much harder, interestingly, than the genetic changes. If we could figure out how to—I think we need a lot more research money on changing people's behavior and understanding human behavior.

There, you would get some genuine large-scale population savings. Just take the exercise on obesity alone. If we could really make inroads there, that would have a significant population health benefit.

But we don't spend nearly so much on that kind of research and we spend an awful lot of money on curing people once they get sick with heart disease and far less on figuring out how to keep them from getting it.

Senator Bennett. Dr. Behrens, you should fund the company that comes up with a pill that makes people want to exercise and eat less.

(Laughter.)

Dr. Behrens. I would like to respond to something that Dr. Callahan said.

There has been a meaningful response in the venture community with respect to managed care and what venture capitalists are willing to finance.

I would add to that the degree of discipline now that goes into assessing potential medical innovation investments and the degree of

discipline required to understand whether those will really become cost-effective devices or therapies or whatever.

And there's no doubt that that's had a consequence on the level of funding in the venture community.

So managed care is a very real consideration in making investments today in the medical field.

A much higher level of scrutiny as well before companies bring these products to the FDA for approval.

It's still a fairly inefficient system. We still don't all budget as well as we would like to. We still don't all predict as well as we'd like to.

So I don't want to suggest that therein lies the answer to a perfect system.

But I will say that the other area that Dr. Callahan has referred to has been much more troublesome. Encouraging or financing opportunities that change human behavior are not well received. And I think there's a simple reason for that. The record is just not very good. It's pretty hard to change.

Senator Bennett. Well, the Chairman has arrived, which injects a note of discipline into this activity.

So may I thank you—

Senator Mack. Before you do that, may I mention something?

Senator Bennett. By all means. I'm through.

Senator Mack. I just want to ask Jim Glassman a question on the capital side of this.

Financial innovations such as Nasdaq have played a crucial role in the growth of the U.S. high-tech industry. Do you see future innovations that could encourage or cause even greater flows of capital to help again these new technologies, these new start-up companies?

Mr. Glassman. Oh, absolutely. We just had Matt Andresen here from Island, Senator, who described ECNs, which are basically electronic networks which will soon be electronic exchanges.

I think we're going to see more and more of that.

And the other thing is I think we're going to see more investing by more Americans.

This is certainly something that should be happening. And if there are public policies that can encourage that, it will be all to the good, both for individual Americans and for the companies that they finance.

But I think the technology is certainly there.

One of the things that's happened is that the friction, the extra little cost of buying and selling shares of stock is now getting squeezed and squeezed out of the system.

That's great. That's great for companies. It's great for consumers and investors. I think we're just going to see more and more of that.

I can't predict exactly what it will look like in five or ten years, but it will look a lot better than it does now.

Senator Mack. Because of time, I'm going to let it go at that and maybe we'll chat some more about it. But I apologize for not being here. But, again, because of conflicts, I just couldn't do it.

Thank you for your participation.

Senator Bennett. Thank you very much. We'll move to the next panel.

We welcome Dr. John Kim Niparko, who has a very special guest he would like to introduce to the Committee and through the television, I guess to the rest of the world.

Dr. Niparko, tell us this extraordinary story.

PRESENTATION

DR. JOHN KIM NIPARKO, PROFESSOR OF OTOLARYNGOLOGY; DIRECTOR OF OTOLOGY, NEUROTOLOGY, AND SKULL BASE SURGERY, THE JOHNS HOPKINS HOSPITAL ACCOMPANIED BY: RACHEL NOBLE, FOUR-YEAR-OLD COCHLEAR IMPLANT RECIPIENT AND JULIE STEINBERG, HER MOTHER

Dr. Niparko. Yes, thank you, Senator Bennett, Senator Mack. It's a pleasure to be here this afternoon.

Part of our obligation to the children of our nation is to provide the tools to children with disabilities that will allow them to engage in the world around them and experience life to its fullest.

We have a growing opportunity to offer such tools to children that are born without hearing.

While I know the focus of today's discussions has to do with biotechnology, in fact, biotechnology is ultimately about people.

Some of these people are very young and can have high hopes and dreams that are virtually without limits.

With me today is Julie Steinberg and her daughter Rachel. Rachel was born with an invisible handicap, and as a consequence of a simple twist of genetic fate, she was born without hearing.

In the past, that handicap would have threatened her ability to hear speech and environmental sounds. She would have missed out on the sensations that make up so much of a child's experience.

Rachel's loss also would have meant that without intervention, her mother and dad would never hear their daughter's voice.

And as we can see right now, that's not a problem.

(Laughter.)

But Rachel's handicap also would have threatened her acquisition of one of the most human of all behaviors, and that is spoken language.

We can think of language as a window on our thoughts, enabling us to report facts, to express our opinions, our emotions and to exchange information with the hearing world around us.

With a severe hearing loss like Rachel's, aggressive strategies are needed for a child to develop functional language. These strategies now employ a revolutionary development in communication technologies, the cochlear implant.

The implant is a remarkable blend of digital circuitry and information processing and the device represents an alliance of strategies of processing information that use both manufactured and natural neural circuits, enabling the hearing pathway to respond to sounds of the environment, to voiced sounds, and to provide a very sensitive level of hearing.

I'd like to roll the tape to demonstrate to you just how the cochlear implant works.

(A videotape is shown.)

Dr. Niparko. Thank you. The advent of the cochlear implant has called attention to the support that is needed for a meaningful and timely exchange of information between the innovators of digital technologies and those of us in the university centers of research and health care.

And just as access to key technologies is critical to the care that I provide, information regarding the consequences of deafness is key to those who, for example, can design a chip that can encode the tremendously complex information that is contained in speech.

I congratulate this Committee in its decision to explore programs that might expand such interactions.

At Johns Hopkins, we've learned that in an experience of over 200 children, that a focused program of rehabilitation in combination with innovative technologies like the cochlear implant can provide the gift of language to profoundly deaf children.

I know that Rachel and her family can express this much better than I can, and I'd like to introduce Julie Steinberg and her daughter Rachel to make some comments.

Ms. Steinberg. Senator Mack, Senator Bennett, members of the Committee, thank you for the opportunity—

Senator Bennett. Could you find another implant and get close to the microphone?

Thank you.

Ms. Steinberg. Thank you for the opportunity to be here.

Rachel and her family are grateful every day for the technological magic that has given her the tools to be like any other child.

I apologize for her interruption of some of the proceedings, but I think that also illustrates the point that she listens and she hears and she wants to talk, just like any other child her age.

Senator Mack. Her reaction was to let her talk.

(Laughter.)

Ms. Steinberg. Thank you. Just a little bit of background and then I think we will give Rachel a chance to talk.

Rachel goes to a mainstream nursery school with children her age. She comes home. She tells us about her day.

At her last birthday party, she sang to herself. She argues with her parents and she tries to boss her brother.

She's just like any other little kid because she has the benefit of this wonderful device and she also had the benefit of some wonderful training to learn how to use the device to listen and to speak, just as we do naturally.

Ms. Noble. It's my turn?

Ms. Steinberg. It's your turn.

Dr. Niparko. If we can perhaps show you a little bit of what Rachel likes to do.

Rachel, do you have anything that you would like to say?

Ms. Noble. Yes.

Dr. Niparko. Please, go right ahead.

Ms. Steinberg. Go ahead.

Dr. Niparko. Why don't you start by telling them your name.

What is your name?

Ms. Noble. Rachel.

Dr. Niparko. And Rachel, how old are you?

Ms. Noble. Four.

Dr. Niparko. And how did you get here today? Did you come by boat?

Did you take a boat to get here today?

Ms. Noble. No.

Dr. Niparko. How did you get here?

Ms. Noble. A train.

Dr. Niparko. A train. Okay. Very good. Rachel, I'm going to try something with you. We haven't really done this before.

Do you know what this is?

Ms. Noble. Yes.

Dr. Niparko. What is this? It's a beeper.

Ms. Noble. A beeper.

Dr. Niparko. Right, a beeper. I'm going to make it make a sound and you tell me, you raise your hand when it makes a sound. Okay?

Ms. Noble. Okay.

(Beep.)

Dr. Niparko. Listen carefully, okay? You tell me when it makes a sound.

(Beep.)

Ms. Noble. Now.

Dr. Niparko. Very good. That's a simple demonstration. I think that what's most important is that she has built on that sensitive level of hearing to incorporate this very complex human behavior known as language into her daily activities.

Ms. Steinberg. Rachel, what else would you like to say? Do you know where we are?

Ms. Noble. At Washington.

Ms. Steinberg. Where do you live?

Ms. Noble. Columbia.

Ms. Steinberg. Where does Daddy work?

Ms. Noble. At Baltimore.

Ms. Steinberg. Where do I work?

Ms. Noble. At Washington.

Ms. Steinberg. That's right. How do I get to work?

Ms. Noble. With a train.

Ms. Steinberg. You got it. Can you drive?

Ms. Noble. No.

Ms. Steinberg. Why not? Why can't you drive?

Ms. Noble. I don't know.

Senator Mack. How old was Rachel when—

Dr. Niparko. Rachel received the implant at age—

Ms. Steinberg. 18 months.

Dr. Niparko. 18 months. And she is now—

Ms. Steinberg. She's now four.

Ms. Noble. I'm four.

Ms. Steinberg. The thing that is most key to know about Rachel is that her hearing loss was picked up as part of the universal infant hearing screening program at the hospital where she was born.

So we knew when she was two weeks old that she was profoundly deaf and that we would have to do something, either through visual enhancement, auditory enhancement, or both, in order for her to develop language.

And because we had that early notice, we were able to jump on it immediately. Rachel got hearing aids when she was four months old.

And when she wasn't getting enough proper hearing through the hearing aids to develop language with hearing aids alone, we knew we needed to look at another option. And that option was the implant.

The early part kicks in yet again. Because she was identified early, because she got hearing aids early and we knew early that she would need something more, she got the implant at what was then considered early—18 months. She was implanted in November of 1996, two days before Thanksgiving.

Senator Mack. How long has this product been available?

Dr. Niparko. This product was first introduced in the United States in the early 1970s.

It has evolved rather quickly, however, to the sophisticated kind of device that Rachel is using just over the past six or eight years.

Much of that work has been done in the United States, although I would add that Australia and Germany are spending tremendous resources on developing similar devices for deaf individuals.

I need to share with you that this is not cheap technology. An outcome like Rachel's comes neither easy nor cheap.

However, the cost of her intervention, the technology and the rehabilitation, is less than one year in the school for the deaf in the State of Maryland.

And so, the cost benefit, cost utility ratios are very favorable with this kind of intervention when applied at an appropriate time.

As Julie mentioned to you, in fact, Rachel's early intervention in our experience seems to predict a very natural sort of outcome, such that she may well be indistinguishable from her hearing counterparts in a mainstream classroom.

Senator Bennett. Will the implant have to be replaced as she grows?

Will it be too small?

Dr. Niparko. No. In fact, the inner ear is adult-size at birth. And so the need for reimplantation for that reason is minimal.

Senator Frist. First of all, I appreciate you all being here. I think of all the things that we've heard and seen today, this to me most dramatizes the importance of the overlap of technology.

Looking at your video, and then seeing the reality, as we look to the next 10 years or 15 years, I'm absolutely convinced that the great breakthroughs are going to be on the overlap.

And although we focus today a lot on the biotechnology breakthroughs, if you look at the implant, the overlap of engineering feats of basic physiology overlap with electrical and mechanical engineering. The bio-compatibility of having an implant like that, which is an issue that we deal with in many committees. The relative risk of kicking in the immune response.

All of that demonstrates to me the importance of our broad investment and not that we target because it's very tempting for us to target a specific disease, as if we know what the cure of that disease is going to be.

Where, in truth, it's going to be at the overlap of various disciplines that we invest in. The great breakthroughs, I believe, are going to occur in that overlap.

So I very much appreciate you coming by. I really feel it's going to be that integration of engineering, bio-engineering, physics, chemistry, bio-compatibility.

Your background is what?

Dr. Niparko. I'm an otolaryngologist. I'm an ear surgeon. My background is in behavioral psychology in my undergrad days. I was rather intrigued by the options that were becoming available to prosthetically restore activity in the hearing pathway.

We've known for a long, long time that early exposure to sound is critical to developing language.

Senator Frist. Were you a principal investigator on this device?

Dr. Niparko. No. I have to admit that I'm one of about 300 that have contributed to the development of this device over the past 25 years.

Senator Frist. And you're at Johns Hopkins now?

Dr. Niparko. I am, yes.

Senator Frist. How many people right now are working with you as a team in terms of development of this device?

Is it just you or is there a group?

Dr. Niparko. No. We have a clinical team of approximately 15 people and a research team of seven.

I think what you're seeing in Rachel is the product of a very, very successful collaboration between industry and the university settings.

In fact, however, a lot of new technology is on the bench and probably could be applied to patients in the near future. But the system as it is now creates in effect a bit of a Valley of Death, where initial great ideas simply aren't getting to the stage of good prototype that's likely to be applicable to large populations.

[The prepared statement of Dr. Niparko appears in the Submissions for the Record:]

Senator Frist. The reason I mention this again is that there is a great tendency among us, me as much as anybody else, to invest just in the pure life sciences.

When the budget is tightened, we're watching spending, there's a tendency to increase some areas of science, based on personal experiences, and forget that areas like engineering really are not emphasized very much in the National Institutes of Health.

If we overemphasize certain areas, not recognize that broad research and development is going to have these spin-offs, I think we'll be putting our investment in any of these single entities at great risk.

Senator Bennett. Knowing what I do about attention spans, I think we've probably exhausted Rachel's.

Ms. Steinberg. Very likely.

Senator Mack. We have a little gift for Rachel for doing such a great job.

Rachel?

Ms. Steinberg. Thank you.

Senator Bennett. My wife would not forgive me if I didn't suggest that Rachel is a good candidate for the Suzuki music program. My wife is a Suzuki music teacher and she has some four-year-olds.

Ms. Steinberg. We just started piano lessons, but I don't think you could say it's the Suzuki method.

Senator Bennett. I can go home and report that I properly have done my duty.

Ms. Steinberg. Thank you.

Senator Bennett. Thank you very much for coming.

Ms. Steinberg. Thank you.

Senator Frist. Thank you very, very much. We appreciate it.

Senator Mack. Can we get the last panel to come forward, please.

We welcome all of you to our last panel of our hearing today, both those who are here in person and those who are joining us via the television screens.

Thank you for your patience. It's been a long day, and some of the panels have run over.

But thank you very much for taking the time to be here. I think, if we will, why don't we start with Mr. Fritzky.

PANEL IV

STATEMENT OF EDWARD FRITZKY, CHAIRMAN AND CEO, THE IMMUNEX CORPORATION

Mr. Fritzky. Well, thank you very much.

Good afternoon, Mr. Chairman, and members of the Committee. My name is Ed Fritzky and I'm chairman and chief executive officer of the Immunex Corporation, an 18-year-old biotechnology firm in Seattle, employing more than one thousand people.

At Immunex, our focus in science and our passion is the human immune system, which impacts almost every function in our body.

It is an honor to discuss our contributions to creating the future of medicine with you today.

Our research has produced several scientific breakthroughs, helping tens of thousands of people who suffer from cancer, rheumatoid arthritis, and we believe new therapies in development for multiple sclerosis, asthma, heart disease, stroke, and osteoporosis.

The most recent Immunex discovery to be approved by the FDA is a product called Enbrel, a self-injectable biotechnology product.

Enbrel treats rheumatoid disease, a condition affecting more than two million Americans, adults and children. Rheumatoid disease can be tragic, potentially crippling, disabling, painful, eating away at healthy joints, robbing its victims of productivity and their ability to move freely.

A typical person with severe rheumatoid arthritis can take up to three hours just to get out of bed in the morning.

That's a devastating disease.

More than any other product in my 26 years in this industry, Enbrel illuminates the human face of biotechnology.

Before Enbrel, people with rheumatoid disease relied on traditional chemotherapy drugs and steroids, with all of their side effects.

In the end, painful and expensive surgeries are the only alternatives for many, as we heard this morning. And now Enbrel gives rheumatoid arthritis patients new hope.

Enbrel targets the cause of the disease. Enbrel today is helping thousands of people in America walk again, enjoy life more, move away from wheelchairs, and return to work.

Here are a number of faces, people who have told us we have our lives back because of Enbrel.

In my written testimony, I introduce to you Kaitlyn, a nine-year-old rheumatoid patient who had tried all traditional therapies.

After years of painful suffering, Kaitlyn was prescribed Enbrel. Now, for the first time in her life, Kaitlyn is able to chew her food, brush her teeth, and sleep peacefully. She is even starting to walk on her own.

Thanks to Kaitlyn's courage and the scientists at Immunex who discovered Enbrel, miracles are taking place like this all over America.

I'd like to introduce you to another very courageous person, Judith Levinson, who is with us today.

An accomplished writer who loves poetry, her struggle with rheumatoid disease left Judith so crippled, that she couldn't even tighten her grip enough to put pen to paper.

Enbrel is helping Judith overcome her rheumatoid disease, getting her back her energetic and her active life.

Mr. Chairman, with your permission, I'd like to submit for the record two of the many unsolicited letters we have received from patients.

Senator Mack. Without objection.

Mr. Fritzky. These personal stories are the most powerful testament to the impact of biotechnology and to Enbrel in particular on their lives.

At Immunex, we treasure people like Kaitlyn and Judith and the thousands of others who benefit from Enbrel.

But we are concerned for a very specific group of people who cannot benefit from Enbrel today. They are people who rely solely on Medicare.

Since Enbrel is an easy-to-use, self-injectable, it is not covered under the current Medicare statute.

Can you imagine if Enbrel were less easy to use, a hospital therapy, for instance, it would be covered by Medicare?

We are proud of how we developed Enbrel. We developed Enbrel to be easy for patients to use at home, not requiring costly and burdensome hospital visits for dosing, or costly hospital staff time for administration.

Ironically, the convenience and cost-saving properties of a self-injectable product like Enbrel are the exact traits that disqualify it from Medicare coverage.

Yet, Medicare will cover a similar therapy which is administered by IV in combination with a chemotherapy agent in a hospital setting.

It defies common sense to exclude Enbrel or any other less invasive and less expensive therapy that treats the same disease.

A legislative solution to this flaw in Medicare law has been crafted. It is a change that Congress can make this year, regardless of the broader Medicare reform debate now going on.

I am encouraged by the efforts of Senators Gordon and Murray to urge the Senate Finance Committee to address this issue.

Additionally, just last week, Representatives Dunn and Inslee introduced H.R. 2892, the Access to Innovation for Medicare Patients Act, which would correct this problem.

Passage of this legislation will allow access to self-injectable biologics, help people on Medicare who live in country areas who have difficulty accessing treatment simply because they don't live in a big city or near a hospital center, provide an incentive for biotechnology companies to develop in-home, self-injectables, versus today's situation, where there is a Medicare reimbursement incentive to develop health care staff-administered biologics.

And this legislation is fiscally wise, potentially saving valuable Medicare dollars, yet delivering what patients prefer.

So I encourage each of you to support this legislation.

Thank you for the opportunity to be here today and your dedication to public service.

[The prepared statement of Mr. Fritzky and accompanying letters appear in the Submissions for the Record.]

Senator Mack. Mr. Fritzky, thank you very much.

I think, with your indulgence, we'll go to Dr. Carbone and Dr. Smith, who I gather are speaking to us from Vanderbilt University.

Is that a pretty good guess on my part?

(Laughter.)

Whichever of you wants to go first.

STATEMENT OF DR. DAVID CARBONE, DIRECTOR, EXPERIMENTAL THERAPIES, VANDERBILT-INGRAM CANCER CENTER

Dr. Carbone. This is David Carbone. I was asked to speak to you a little bit today about the application of biotechnology to cancer therapy.

Maybe I should start out by saying that I had the personal opportunity to experience the old style of cancer therapy when I underwent surgery, chemotherapy and radiation, for a lymphoma and am now in that category of people called cancer survivors.

Those drugs, even though they're effective, were all designed by the random screening of chemicals, scraped from funguses and tree bark and bacteria in the past.

And by virtue of that, they have a lot of toxicities that are associated with them as well. They can kill normal cells and make people sick, as well as killing cancer cells.

Biotechnology today is in the process of developing a huge array of new compounds that directly target specific processes of cancer that are different from the normal cells. The idea is to generate therapies that are specifically toxic to cancer cells instead of to normal cells.

An example of that in my lymphoma would be the antibody CD-20. My lymphoma was CD-20 positive and this is a totally nontoxic molecular biology agent that's been recently developed targeting my type of lymphoma that will probably see broader application in the future.

At our university and many others, we're studying a variety of other molecular biology therapeutic approaches targeting tumor blood vessels, targeting signal transduction in tumor cells that activate tumor cells to grow, targeting tumor suppressor genes that normally tell normal cells to stop growing when the tumors are affected.

I'm running gene therapy trials that are designed to reintroduce some of these tumor suppressor genes. We have tumor vaccine trials targeting the mutated gene products, of ras and p53 in tumors.

Again, I think in the future we'll see a lot of new therapeutics that are nontoxic and more effective.

That's all I had intended to say, unless there are questions. And Dr. Smith can follow up.

Senator Mack. Yes. Why doesn't Dr. Smith go ahead and then after the rest of the panel has made their presentations, we'll get into a few questions.

Dr. Smith?

STATEMENT OF DR. JOSEPH A. SMITH, CHAIRMAN, DEPARTMENT OF UROLOGICAL SURGERY, VANDERBILT UNIVERSITY MEDICAL CENTER

Dr. Smith. My name is Jay Smith. I'm a urologic surgeon and consequently, I treat prostate cancer primarily. This is a disease which I think emphasizes both some of the real successes of biotechnology mergers and treatments with surgeons and other individuals at

universities, as well as some of the challenges that exist. Because we've gone probably as far as we're going to be able to go with conventional surgery and radiation in treating prostate cancer. But, on the other hand, we continue to use these as our primary form of therapy, and we still run into too many men where these treatments fail and we really have no other therapeutic options.

And even today, despite the advances that have occurred, we don't have any therapeutic options that work.

We've done a gene therapy trial here at Vanderbilt. It's one of the largest gene therapy trials that exists in men with prostate cancer. And we learned a lot with that trial.

But it was a phase one trial, a phase one/phase two. That means it was intended primarily to gain information without any real pretense this was going to be a trial that would actually help the patients.

So we're still in the information-gathering phase with these gene therapy trials. We're still running into lots of roadblocks. We have no method for systemic delivery of the gene therapy.

And so, again, lots of promise with what already has happened, but lots of challenges that still face us.

Senator Mack. Okay. Well, thank you very much. Again, we will come back to you all a little bit later.

Mr. Rausch?

STATEMENT OF CARL RAUSCH, CHAIRMAN AND CEO, BIOPURE CORPORATION

Mr. Rausch. Thank you, Mr. Chairman, and members of the Committee.

Thank you for allowing me to speak about an oxygen therapeutic or an alternative to red blood cells.

Founded in 1984, Biopure is a biopharmaceutical company of about 175 people located in Massachusetts, New Hampshire and Pennsylvania.

Although we are small by comparison to some of the other speakers, our technology could change transfusion medicine worldwide.

Our patented method for purifying and modifying proteins has allowed us to create a new class of biopharmaceuticals called oxygen therapeutics. These products are often called blood substitutes because they can replace red blood cells and deliver oxygen to the body's tissues.

Oxygen therapeutics have overcome many of the medical, economic, and logistical problems associated with blood transfusions and could have an enormous impact on patient outcomes.

In the United States, more than 12 million units of blood are donated and approximately four million patients receive blood transfusions.

It has been estimated that as many as 50 percent of these patients risk death if left untreated.

Ninety-five percent of us will require a transfusion by the time we are 75. Each year, there are regional shortages in the blood supply which may soon become a national problem.

In a recent public health service meeting, testimony was given projecting a shortage of up to 300,000 units in the United States next year. The new FDA donor deferral could increase this shortage to 550,000 units.

Biopure's oxygen therapeutic addresses availability issues because it can be stored at room temperature, has a two-year shelf life, and it is compatible with all blood types.

Blood is a product with inherent and unavoidable risks. Although it is licensed by the FDA, it does not have the same standards for purity or effectiveness as a pharmaceutical product. It is classified as a biohazard and it is unique among health care products in being shielded from most product liability laws.

In contrast, pharmaceutical manufacturers like Biopure are responsible for producing products that adhere to FDA requirements.

Donated blood also carries risk of emerging pathogens, for which no test exists.

For a historical example, today, nearly four million Americans are affected with Hepatitis C virus, many unknowingly. These infection-related illnesses represent a significant economic health care burden.

Biopure's oxygen therapeutics address these issues.

A GAO report in 1997 found that eight out of 10,000 units of blood pose a potential serious risk to the recipient. Red blood cells have been associated with substantially greater infection, such as pneumonia, resulting in increased hospitalization costs of approximately $14,000 per patient.

This is not reflected in the unit price of blood.

The impact of oxygen therapeutics upon transfusion medicine is being proven in the veterinary field.

In 1998, Biopure's Oxyglobin became the first FDA-approved oxygen therapeutic.

Since then, the product has provided safe and effective treatment for anemia in dogs and has helped save the lives of hundreds of animals.

Biopure's similar oxygen therapeutic for humans, Hemopure, is being evaluated in a pivotal phase three clinical trial. It has received fast-track review status in South Africa and is being evaluated for the treatment of heart attacks, stroke and tumors to increase the responsiveness to chemotherapy and radiation.

With a two-year shelf life and room temperature stability, the product consists of a purified bovine hemoglobin that has been chemically modified. It is compatible with all blood types and releases oxygen immediately upon administration—red blood cells do not.

Hemopure can address critical care conditions and emergency situations where immediate oxygen support is vital. It could facilitate military preparedness and improve medical care in remote geographic areas world-wide.

For example, during Desert Storm, it cost our military almost one million dollars a day to maintain a state of blood preparedness.

The United States' leadership position in biotechnology will only be maintained if economic policy fosters this new technology, like the oxygen therapeutics.

Biopure has raised $300 million to fund development of this technology. And like all companies, we must show a return to our investors.

To achieve a fair return and make this product available to the health care system, we need a true understanding of the overall blood cost in patient management.

The government, with its payment system and Medicare, cannot be a bystander. Reimbursement policies for blood have not significantly changed for more than 20 years.

Yet, 11 new tests and multiple new procedures have been mandated to improve the safety of blood.

With oxygen therapeutics now possible, it's time for reimbursement guidelines to reflect the total cost of blood infrastructure and use.

In addition, the U.S. Patent Office should consider lengthening patent protection coverage so that it compensates for the lengthy FDA process.

In closing, I'd like to put a face on our technology in describing a compassionate use case.

A 21-year-old woman was suffering from a severe anemia. Essentially, her immune system was destroying her own red blood cells. At one point, her red cell volume dropped to four percent, a level which will not sustain life. It's far below the normal 40 percent.

However, this patient was administered Hemopure, and was stabilized until she overcame the disease. She was discharged from the hospital.

This significant case demonstrates the impact Hemopure can have when blood is not available or cannot be used.

With proper economic policy, Hemopure will support the world's growing blood requirements.

Thank you.

[The prepared statement of Mr. Rausch appears in the Submissions for the Record.]

Senator Mack. Thank you very much.

Mr. Dollens?

STATEMENT OF RONALD W. DOLLENS, PRESIDENT AND CEO, GUIDANT CORPORATION

Mr. Dollens. Thank you, Mr. Chairman. I appreciate the invitation of the Chairman and the Committee.

I am Ron Dollens, president and CEO of Guidant Corporation. I also happen to be chairman of the Health Industry Manufacturers Association this year.

Our company is a two and one-half billion dollar organization with approximately 7,000 employees, headquartered in Indianapolis, Indiana. But most of our people are in the Minnesota and California areas, with facilities in Texas, Puerto Rico, and Ireland.

We are a five-year-old split-off of Eli Lilly and Company's medical device businesses.

To start with and state the importance of innovation in technology in our business, I would represent that 60 percent of our sales since we've been a company five years ago are products that were less than 12 months old.

So our velocity of product development is a critical success factor in this business.

Guidant focuses entirely on the treatment of cardiovascular patients. Half of our business is around the treatment of arrhythmias of the heart. We make pacemakers for slow heartbeats. We also were the first to commercialize a therapy, an implantable defibrillator to treat fast heartbeats, which are potentially an arrhythmia dysfunction for people that have had or are prone to sudden cardiac death.

We've also designed and manufactured and marketed globally the early and continuing advances in angioplasty for treating coronary artery disease, the predecessor to myocardial infarctions or heart attacks.

We also provide 40 to 50 percent of the coronary stents for patients around the world. This year alone, that will include 400,000 patients.

Again, stents are those metal scaffolds that are within the coronary arteries to hold the artery open.

Today, before the conference, we displayed the next technological advancement in terms of treating coronary artery disease.

It is the application of site-specific radioisotopes or radiation to minimize the reclosure or restenosis, regrowth of plaque in the coronary arteries.

Our technology demands are heroic.

When we design an implantable defibrillator, we don't have the luxury of plugging it into a wall socket. It has to be implanted in the patient, sense the activity of the heart 24 hours a day, and deliver an electrical charge to stop a lethal arrhythmia and bring the patient back to life.

And, yes, it needs to last five to seven years on its own battery.

As you can imagine, when we design the integrated circuits and the accompanying software, our demands may be greater than all other applications of this technology.

And did I mention that the patient and their doctor expect this product to work on a regular basis?

Also, today's product is one-fifth the size and approximately the same cost.

As a company, we also put great demands on the field of material science, both polymers and metallurgy.

During angioplasty, the catheters incorporate thin films that are inflated to pressures of 300 pounds per square inch inside your coronary arteries.

There is an expectation that during this dilatation, there will be no ruptures, leaks, and nothing will be left behind.

This technology has advanced substantially in the last five years and the prices are less than half they were in 1990.

As we remind ourselves, though, our greatest contributions are not in the advancement of the technology, but the treatment of approximately six million patients by Guidant products in the last five years.

How has this activity been encouraged?

For a company to spend not only 15 cents out of every sales dollar on research and development, but to spend every dollar of profit after taxes that we have earned for technology acquisitions and development, which in our case is $2.4 billion over the five years that we've existed.

And yes, go ahead and pay 35 percent in taxes on these earnings.

At the same time, the investors in this company have rewarded that activity by increasing the value of the firm by 15 to 16 times in five years.

All of these efforts are directed towards treating the leading cause of death in the U.S., while providing a constantly improving value formula.

By value formula, I mean increased functionality, increased reliability, decreased cost and the chance to prolong life.

As a personal example, we all saw the death of George C. Scott in the last couple of days and he died of an abdominal aortic aneurism.

You may have also read yesterday, we had a product approved for the treatment of that disorder.

When Mr. Scott was diagnosed, the only option was to have open surgery, which meant they opened up the abdomen, separated all of the organs, went back to the aorta, filleted open the aorta, and sewed in a graft.

In the system that got approved yesterday, that graft is available on a catheter, it's delivered up through the aorta and it's delivered from the inside out.

It has a third of the cardiovascular events, half of the respiratory events, the length of stay in the hospital is one third of surgery, and the same outcome.

One can ask, how can a society be so astute as to encourage this great activity?

I would submit that we have leveraged the entrepreneurial initiative, the market-based competitive environment that works so well in the U.S. across many sectors.

Will it continue?

I state that it is fragile. There are no guarantees. Our ability to attract financial capital and as importantly, and not unrelated, to attract the intellectual capital, is dependent on this company and this industry remaining an interesting business.

Public policy has great influences. Public policy actions that increase uncertainty and decrease predictability or interfere with the patient choice and clinician input on the values of technology will eliminate the innovation and inventiveness in the industry by causing the fleeing of capital and talent to more interesting opportunities.

Public policy in areas of tax, in areas of immigration—there is an absolute war for talent, especially around software and software engineering.

Public policy around regulation—the FDA has made great strides in the last five years and we are currently focused on a huge concern around reimbursement in HCFA.

Companies make decisions to invest in R&D based on understanding such public policy implications as regulatory processes and reimbursement processes.

If I look at 1993, where the FDA was being nonresponsive and we were talking about a Clinton health care plan that limited people's choice, we saw very little capital formation and very little capital appreciation.

In 1996, the FDA was making some great strides in terms of product approvals. We had 65 initial public offerings that year.

In 1999, with concern about Medicare reform and reimbursement, there have been zero IPOs in the medical device sector.

Policy has far-reaching implications. Our current greatest concern is Medicare reform and HCFA reimbursement policy.

If HCFA establishes itself as the monopolistic purchaser of products and services and squeezes the provider and suppliers and eliminates choice, we do have the opportunity to destroy where we are going, which is the future of novel therapies.

We can decide if where we are today is good enough. I believe our public constituencies will vote for continued evolution of therapies.

Thank you for the opportunity, Mr. Chairman.

[The prepared statement of Mr. Dollens appears in the Submissions for the Record.]

Senator Mack. Thank you, Mr. Dollens.

Let me explain the situation.

We have two votes, the first of which has been called. There's about eight minutes left.

I suggest, Bill, if you want to, you run and make the first vote. I'll stay here and listen. When you come back, that way, we can try to keep this going.

Dr. Rathmann?

STATEMENT OF DR. GEORGE RATHMANN, CHAIRMAN, ICOS CORPORATION

Dr. Rathmann. Yes. I'm George Rathmann. I was cofounder of Amgen in 1980. Amgen was referenced by Peter Lynch as a big taxpayer, one of the few biotechnology companies that is because of two very successful products.

In 1990, I was a cofounder of ICOS, a small company in Seattle. It focused on inflammation, although we found ourselves, because of the fortuitous nature of research, coming into a lot of other areas.

I thought I'd cover three areas.

One is, keep in mind that the United States is number one in biotechnology. It's wise to remember the clues that put us there and they are clues to what we should be doing in this Committee and for the future.

The second item is what has been achieved? I won't go through a lot of the things that have been covered already, but maybe some personal angles that I think are important.

And finally, the issues from the future, which includes some thank yous to many of the people in this room from the congressional side.

If we ask why the United States is number one, it is pretty clear and you've heard a lot of it today. It is the academic power of this country, which is the fundamental basis.

Our higher education leads the world. The basic science, the technology transfer, and the infrastructure has all been made possible by that work largely supported by the government.

The risk capital system and the entrepreneurial mode in which this industry has developed has certainly been important. And the broad base of viable public markets has made a lot of it possible.

In 1994, with the threat of price controls just referred to, the price of many of our stocks dropped by 80 percent. ICOS dropped by 82 percent during that year.

It looked as if we might not be able to complete a financing.

About three or four weeks ago, there was a hearing with respect to the issues of what Medicare changes might mean. Our stock dropped 20 percent in about a week.

So the linkage has never been so clear as to the need for a free market with respect to how products will be priced. At least pricing has to be looked at with some concern about the enormous risk that this industry encounters and the enormous costs before a product can be launched.

And the third thing in this country that's been wonderful is the government's role.

The patent decision in 1980 by the Supreme Court and subsequent patent decisions and the speeding up of the Patent Office have been very important. The awareness of the world-wide implications of patents have been very important.

Of course, the government's role in academic support was mentioned. For the most part, the tax structure in this country is more favorable than in many countries in the world. Our regulatory processes,

although cumbersome, have been satisfactory and, with improvements recently, I think that they're looking pretty good.

So, basically, those are the things that we have to keep in mind as to why the United States is number one.

There should be no doubt in anybody's mind. It is number one. It stays number one. It has weathered many periods in which people have suggested that the Japanese are taking over, the Europeans are going to take over, and so on, that has not happened and it shouldn't happen if we keep those things in mind.

Now a lot of things have been mentioned about what's been achieved.

The ones that I am most familiar with are, of course, Amgen's products.

In the case of anemia, the anemia drug which during a lifetime might involve administering as little as about one aspirin tablet of erythropoietin throughout a life. Yet, it will prevent the anemia which plagues all dialysis patients, or almost all of them.

And in fact, this drug has represented savings in the blood system of hundreds of thousands of units every year.

Amgen's other product deals with chemotherapy, the prevention of infections during chemotherapy. Death during chemotherapy treatment is largely associated with infection loss and that product has been very important.

Some of the personal side of that is when you meet people that have had these drugs, even though I've been away from Amgen for eight years, they say, you brought me my life back.

Ed mentioned the same thing with respect to arthritis.

We have a personal instance in arthritis because we're both in Seattle. One of our scientists has been unable to work as a scientist. We gave her another assignment because the dexterity of her hands was almost gone.

Within about three weeks on Enbrel, she is able to handle anything with the same dexterity as she had before the disease.

So these are remarkable changes.

When we look ahead, one of the questions that's come up here, who's going to pay for it all?

We're paying for it today. Henri mentioned the fact that we're spending $100 billion here, $100 billion there. As Dirksen used to say, that can be real money.

It turns out it's $540 billion a year in the U.S. in 1996, for diseases that essentially represent an unmet medical need.

Unfortunately, many of them are tied to the aging population, which we know will drive that number within the next few years to over a trillion dollars.

We're seeing an escalating cost of just what we're doing today with the diseases that we haven't handled very well.

We are looking through biotechnology. We look at molecular mechanisms. We have a way of getting in there early, possibly preventing some of the consequences before some of these enormous costs.

I think that the outlook is certainly pretty promising for not only some of the progress that we've seen, but you're going to hear a lot more about cancer and aging and obesity and prevention over the next few years.

That's where the action is going to be. We've already seen the breakthroughs with respect to cancer. We've seen the fact that there are molecules that we used to assume caused cancer which now we realize, those are mutations of molecules that prevent cancer.

And that's going to be used. It's going to be—P-53, for example—there is a marvelous breakthrough that we have actually been learning more and more about for quite a number of years.

Now if we look at the current status and the future in terms of the government's role, first of all, we need to thank you for your work in NIH funding, FADAMA reform, securities liability reform, biomaterials liability reform, extension of the R&D tax credit and capital gains.

We have many things to be grateful for—the expansion of H1-B visas and the permanent orphan drug tax credit. I think Senators Bennett and Frist should receive some accolades for their medical confidentiality contributions. It's been very important there.

So we think that there are some ongoing needs of all of these in these same areas. The work is not done. The work is not done with the FDA. The work is not done with—certainly, it will never be done with respect to the research funding levels that could continue to be improved to great benefit.

And I think with the right actions by this Committee, we'll maintain the leadership of the United States and we'll also perhaps have successfully put a human face on biotechnology, which will assure us of a promising future.

Thank you.

Senator Mack. Thank you very much.

Dr. Sager?

STATEMENT OF DR. ALAN SAGER, PROFESSOR OF HEALTH SERVICES, BOSTON UNIVERSITY, SCHOOL OF PUBLIC HEALTH

Dr. Sager. Thank you, Mr. Chairman. Good afternoon.

My name is Alan Sager. I'm a professor at the Boston University School of Public Health.

I'm honored to be here today.

We have all heard great things today. But biotech has a second human face—the face of people who cannot afford even today's medications.

What's the problem?

Seventy million Americans have no insurance for prescription drugs. Many additional people have terrible coverage.

And these are the economy's fat years, as Joseph kept reminding Pharaoh.

The industry acknowledges that Americans pay the world's highest prices for most drugs, even though we are one-third of the world market, one-third of the purchasing power.

Uninsured Americans pay still higher prices. Fully 17 percent of all Americans and more than two out of five uninsured Americans reported not filling prescriptions for financial reasons.

U.S. prescription drug spending per person this year will become the highest in the world. We will be number one—probably a little over $400 per person. And the total will be around $120 billion.

The drug cost problem will worsen. Drug spending in the United States has been rising three times as fast as overall health spending and, as we've heard, over a thousand new biotech and other drugs are reported to be in the pipeline.

To finance all the drugs that work for all Americans, and to protect biotech research specifically, we must examine the prescription drug industry generally and look at why medications aren't affordable today.

U.S. drug prices are high because our government does not protect us from the world's drug makers, letting them charge what they wish here.

This year, Americans are paying at least $16 billion extra for prescription drugs, which is a hidden subsidy to the world's other wealthy nations.

This is the least well-targeted foreign-aid in the history of the world, more than double the foreign aid you vote as bilateral aid to threatened nations or starving nations.

The drug-makers paralyze government action to protect us by claiming that today's high prices and profits are legitimate products of a

free market, that high drug prices and profits are needed to finance vital research, and that any restraint on prices or profits here will collapse the drug-makers' financial house of cards.

Not so.

This decade, the drug-makers' returns on equity were over double the average for all U.S. industries. Such returns are not justified by a genuine free market.

Instead, they derive from simple anarchy, anarchy produced by a combination of market failure with government inaction.

That is why the Pharmaceutical Research and Manufacturers of America (PhRMA), the drug-makers' trade association, spreads a fog of fear, PhRMA's fog of fear, to try to paralyze government action to preserve their pricing power.

If the looming cost and coverage crises intensify, a future Congress, spurred by anger and fear, might legislate harsh price and profit controls which would gut needed research.

We will have continued re-enactments of the 1993 and the 1999 drug stock price dips until we fix these problems.

What solutions are possible to make medications affordable and to protect returns on equity in biotech and pharmaceutical research generally?

Well, the manufacturers and others hyperoptimistically hope that new drugs will reduce total spending. I expect no more than a one-time saving at best.

No matter, we must plan against the contingency that costs will actually rise as we develop new medications.

By any sound standard, we already spend enough in the United States to buy all the drugs that all Americans require. So the first challenge is to protect all people without spending more money.

And the second challenge is to pay enough to the drug-makers to protect all needed research and to attract all needed capital.

Internationally, the step is clear. We have to negotiate a drug price treaty. All wealthy nations have to pay the same prices.

Domestically, I see only two alternatives. The first, we could fight for years over drug prices and profits and coverage while people suffer and drug stocks and biotech stocks go up and down erratically, in a very unstable and dangerous environment for all of us.

Or we could make a package deal that has to include, I think, at minimum, two essential pieces and four or five others as well.

The two essential pieces: first, all payers and drug-makers negotiate returns on drug-makers' equity, returns adequate to finance research and to attract and retain all needed capital.

But on top of that, developers of effective new medications that really make an advance would earn premium profits. There would be a clear reward for doing better, not for raising the prices on drugs like lithium, which has been around for decades, the price of which in the Boston area has almost trebled in the last few months, I've been told.

There would be justifiable profits that really promote and reward decent and valuable innovation.

Second step: in exchange, drug-makers produce enough medications to fill all prescriptions that American physicians write.

The cost of increased production would be low. That's because drug-makers typically face tiny marginal or incremental costs to make more of a given drug once they cover the high fixed cost of research and setting up the manufacturing plants.

We've estimated incremental costs at only about five percent of average retail prices.

That's a great positive sign, a great source of optimism.

In conclusion, winning affordable drugs in ways that stymie research is dangerous. But developing useful drugs that people can't afford is tragic.

Americans can't keep subsidizing other wealthy nations. Moderate action and compromise today will protect both American patients and vital research tomorrow.

I hope that you'll be able to lead us in that direction and thank you very much.

[The prepared statement of Dr. Sager appears in the Submissions for the Record.]

Senator Frist. Thank you, Dr. Sager.

Let me begin with a couple of questions, and you're free to comment on each other's responses as well, because a number of interesting concepts have been mentioned, as well as different approaches.

Mr. Fritzky, on Enbrel, it has been incredible for the people that I've talked to in terms of the effects and how people's lifestyles are changed. You mentioned that, which is so common when you talk to people with debilitating arthritis, how they can't move the neck, how it takes literally 30 or 35 minutes just to get out of bed. Being in that position, this has revolutionized their lives, all of a sudden being able to immediately get out of bed.

It's so dramatic when you hear it.

And then we have the cost issues. The people who come to see me very quickly go directly to the cost issue and then what we're concerned about is how you spread this new technology to other people. If you're one of the fortunate few in terms of the overall population who have access, you don't have the barriers that most do.

How long did it take you to develop a drug like Enbrel?

Mr. Fritzky. Well, it was discovered in the late 1980s and approved by the FDA.

By the way, thanks to FDA reform, in just five months, and it was approved in November of 1998, so about ten years to reach market.

Of course, when you think of the ten years, that's once the discovery was made. It doesn't count all those years that researchers were looking at the root cause of rheumatoid arthritis and what may cause it, what might be the genes that turn it on, turn it off.

So all that preliminary work may have taken 20 years to get to the point that we reached about ten years ago with the discovery.

Senator Frist. Now let me also come in because Mr. Dollens mentioned it as well.

Food and Drug Administration modernization. We hear how government doesn't do a very good job in these things. I'll have to say, having participated in the debate over modernization, the bipartisan efforts, it has been amazing. It was just two and one-half to three years ago, especially in the device, but also in the pharmaceutical field, the improvement.

The Food and Drug Administration is a strong, respected organization around the world. People have so much respect for it. But as we all know, things can always be improved. A number of people who are on this Committee, and other committees, worked together with academia, industry, private sector, public sector. I think great strides have been made, as several of you have mentioned.

In terms of reimbursement, how much does a month's supply cost?

Mr. Fritzky. A thousand dollars.

Senator Frist. A thousand dollars. And that's an injection, once a week?

Mr. Fritzky. It's basically taking a shot like this, a subcutaneous injection, just like an insulin shot, a little needle stick under the skin.

Senator Frist. How often?

Mr. Fritzky. Twice a week.

Senator Frist. Twice a week. A thousand dollars. To come up with that thousand dollars a month figure—and again, I'm used to this

because in transplantation, as you know, the immunosuppressors are very expensive.

To come up with that thousand dollar charge, do you actually sit down and say, we've got a patent. This is how many years we put into it. This is where we can competitively position this.

How exactly do you come up with a thousand dollars?

Mr. Fritzky. Traditionally, in the biotech area, we see charges in about this arena. And the reason that we do is that these drugs, the cost structure of these drugs, the manufacturing, they're basically really human proteins. The cells to make, ultimately, to produce these human proteins take literally weeks.

And the people that are involved in manufacturing are really scientists.

So aside from the product development expense, the manufacturing cost structure of these newer biotechnology drugs is such that they're several thousand dollars a year to manufacture.

Senator Frist. So three years from now, what do you think the price will be?

Will it come down or plateau off?

Mr. Fritzky. Well, it's a very difficult question to answer because the product has just really reached the market in the last eight months.

Senator Frist. So here we have a revolutionary drug. It took ten years, went through all the hoops, a lot of risks taken. Here we have a great drug, revolutionary, it does the job. Yet, it's very expensive. You gave us these two alternatives, international and domestic.

We've got to sit up here and figure out how to get this drug out, distribute it, because it's so unfortunate that we can't give it to everybody.

What do we do?

Dr. Sager. First of all, we do work to bring down the manufacturing costs.

And we know that they almost always drop over time. Often very substantially.

Second, throughout the drug industry, opportunities to cut wasteful marketing and advertising costs should always be seized upon because these can be a quarter to a third of a given company's costs.

Third, we need much better evidence for physicians, objectively compiled and disseminated, about which patients really need this medication, which patients can make do very well with something that's much less expensive, so that we spend money carefully.

But I think for $120 billion, we can find room, which is what we're now spending on medications.

Senator Frist. I guess that's my question.

Dr. Sager. Find the room.

Senator Frist. You made the statement—I'm not sure that I agree with it, that given the money that we spend today, it's enough. This drug really didn't replace anything.

This drug is, or you can say that it replaced aspirin and some cyclosporin, which is expensive, and Immuran, the usual treatments. But not at this expense.

Therefore, as these new breakthroughs come, or as Dr. Smith and Dr. Carbone mentioned, I think there are going to be some new drugs which incrementally are going to cost more.

And that's why I still have questions about your statement.

Dr. Sager. I think you're right. I think we may have to look elsewhere within $120 billion for the savings. The medications that are misprescribed, all of the drug interactions that follow because people are shopping at four different pharmacies by price to try to cover their costs, so no one pharmacist is checking for drug interactions.

The wasted medications, the physicians who are flying with maybe not the most up-to-date information.

Senator Frist. Some people say that 50 percent of all prescriptions today are unnecessary. If you look at antibiotics, I guess that would be another example.

Dr. Sager. And half of all physicians have less pharmacological knowledge than the other half, the ones below the median.

And so, everyone can be brought up to a higher level of performance. I don't single out physicians, of course.

Senator Frist. Better not. Please not.

(Laughter.)

Mr. Fritzky, Medicare doesn't usually do out-patient prescription drugs; very, very rarely do they. Some say it wasn't intended to and that's where a lot of the debate is.

Of the 60 percent of Medicare recipients who do have some prescription drug coverage today, how many of those would be covered for Enbrel?

Do you know?

Mr. Fritzky. I'd say about a third to a half.

Senator Frist. So about a third of the 60 percent on Medicare—

Mr. Fritzky. Would have some coverage. I think—

Senator Frist. Go ahead.

Mr. Fritzky. Excuse me. Just on the point of Medicare today, Medicare would, if we had produced Enbrel, which we could have, as a

biological to be used in the hospital or with a physician's intervention, or with a nurse's intervention, it would be Medicare-reimbursed.

But we chose to make it as friendly for the patient to use as possible. And also the health care system.

So right now, Medicare basically will reimburse. If you've got an IV infusion requiring a physician of a similar product in a hospital, Medicare would pay for that today.

Now, patients who have Medigap policies, many of them, while they have some coverage, don't have complete coverage.

So even that third to a half is probably a little too high when you really look at the patient's adequate coverage versus what they're coming with out-of-pocket.

Senator Frist. Well, the purpose today is not really to talk specifically about prescription drug coverage, but I think from my standpoint, there are huge savings—the cost of in-patient versus out-patient care. A drug like this would keep people out of the hospital. It gives back quality of life.

But it really does mean we have to look at where those savings might be by modernizing the overall rigid system.

It's a debate that's going to go on and I appreciate everybody's perspective.

Let me turn to Dr. Smith and Dr. Carbone. Can you hear me, Dr. Smith, Dr. Carbone?

Dr. Carbone. Yes.

Senator Frist. Right now you talk about specific genes. As we unleash these three billion bits of information over the next two or three years, what implications does that have in terms of expanding research?

Dr. Carbone. I think it has a tremendous impact. We're studying the genetic changes involved in lung cancer in our laboratory.

The genome project has dramatically facilitated that process.

We identified, for example, a new translocation in lung cancer that has activated a new gene involved in cellular differentiation and represents a potential target for therapeutics.

We accomplished the localization, identification and cloning of this gene in a matter of a few months with a single post-doctoral fellow by virtue of the fact that the whole region had already been sequenced in the genome project and all we had to do was look on the Internet and pull down the sequence.

We knew exactly what the map of the region was. I think it will dramatically accelerate our ability to discover new drug targets and learn

more about the biology of cancers in general, even though only a fraction of the genome is available at the current time.

Senator Frist. Dr. Smith, three to five years from now, how much of your therapy for prostate cancer will be genetic, or genetically-derived in some form?

Dr. Smith. I think that the issue is so complex, Senator Frist, that three to five years from now, I suspect that there will probably be a distinct minority.

I'm not sure that the breakthroughs that we need are actually going to occur with regard to gene therapy within the next three to five years.

But as you already mentioned, things like the genome project are what are going to move us along because this is so complex, you can't think just single genes without knowing the context of the whole and move on to any therapeutic advances.

So within the timeframe you mentioned, I'm not real confident that we'll see breakthroughs. Much beyond that, though, I think they're going to occur.

Senator Frist. Let me just ask the panel to address an issue that seems pretty clear to me.

The vaccine development today, if we look world-wide, as I mentioned earlier today, 5,000 to 6,000 people in a continent like Africa die every day of AIDS, malaria, tuberculosis.

There is not much of an incentive for our pharmaceutical biotech, or biopharm companies to get involved because there is no bottom line for them.

What potential role do any of you see for government to maybe provide such incentives to interact with the private sector to encourage such development?

There's some goodwill and humanitarian feelings on the part of everybody. The bottom line, for those of you who are in business, what does it take in terms of offering an incentive?

What's the potential? What incentives could the government give? It's not going to happen otherwise, I'm afraid. Or it's going to happen a lot longer.

Dr. Rathmann. It strikes me that you're dealing with foreign-aid. If you made the foreign-aid tied to the ability to buy certain health care-approved medicines and thereby created a market that requires very careful consideration.

When Amgen was started, we were put together in part by a scientific advisory board.

We had at least six programs in mind that would affect developing countries' diseases.

It was literally impossible to imagine the financial cost to us and how we would ever get it financed.

If there were a well-documented appetite, financial appetite in the country, then I think you could be quite assured that someone would want to fill that appetite. Malaria, without a doubt, hundreds of millions of people around the world.

But I think if it's foreign-aid, I kind of agree with the idea that you don't burden our health care program with forcing people to try to supply goods or services where they're not going to get reimbursed.

That's just another type of foreign-aid. I think you ought to address it as foreign-aid. I think it's very important foreign-aid.

Senator Frist. Any other thoughts?

Mr. Fritzky. I would add that I believe that the same incentives that were talked about by others on the panel, research tax credits, and had been discussed today, are the incentives that are going to be important to the continuation of vaccine research.

We're a company that has lost over $250 million and have made a profit of less than $10 million.

So when you subtract the 250 and the ten—and we're considered a successful company in the industry. And of course there are companies investigating technologies, vaccine therapy, gene therapy, how to get to the function of genes as was discussed through the TV hook-up that we have.

So all of these areas, including what we're doing, are highly speculative and may not have a reward.

So I would say that it's basically the same incentives that we've talked about that would be common between these various areas— very speculative research, long-term pay-offs, you're not sure if you're going to have a market.

Senator Frist. Thank you.

Mr. Chairman?

Senator Mack. Well, again, I thank everyone for their patience and especially for having what we had to do here this last half-hour.

I was supposed to be at another meeting at this point. But I must tell you that I felt compelled to come back to close the hearing and to thank everyone.

But, Dr. Sager, I can't let you go without me at least responding. I do not want to hear a response from you. This is a statement on my part that's a prerogative of the Chairman.

The kinds of gibberish that I heard you speaking a little bit earlier are the kinds of things that, frankly, encouraged me to enter politics back in 1982.

That somehow, not to recognize that, in health care in America, we have people who come from all over the world to get to the best health care system in the world.

We heard someone mention a little bit earlier this morning, or earlier this afternoon, 90 percent of the biotech products are being produced in America, and that's going to stay that way for a long, long time.

A majority of the pharmaceutical products are developed in America.

You have to ask yourself, why did that happen? Could we go to the approach that Dr. Sager mentioned? Of course we could.

But I would say to people, you ought to understand what it is you're giving up in order to do that. And I think the costs frankly are way too high.

You may not like it, but in a free market, capitalist system, which I happen to love, we are able to provide this leadership, and we are in fact able to give hope, not just to Americans, but hope to people around the world.

Could we be doing better in seeing that more Americans participate in the miracles that have been created?

Yes, we can.

But I'm not going to do it at the expense of cutting off America's future. And again, all you have to do is look at the numbers.

In fact, I remember when the Clintons went after this industry back in 1992. And I will tell you the thought that occurred to me.

This sounds like the 1960s all over again. Do you remember one of the first things that John F. Kennedy did? He went after the steel industry for a price increase.

What happens when you start to manipulate prices at the government level? You're going to get less of it.

And all you have to do is look at the record. We heard this morning a substantial reduction in the capitalization or the value of the companies as a result of what the Clintons did back in 1992, 1993.

Then all you have to do is look at the facts as far as investment in research is concerned. You can run all the way through the 1980s to the present time.

There's one very, very glaring change in the investment made in the pharmaceutical industry. Up until 1994, 1995, the amount of research

from year to year increased by ten percent or more every year from 1980 to 1999, with the exception of two years, 1994 and 1995.

What happened was that the market heard what was being said. And whether you like it or I like it, the market said, we're not real sure about the future of this industry based on what's going on now.

We had a reduction in the investment in the future. We had a reduction in the value of those companies. And I think that's a tragedy.

Now, Dr. Sager, you had an opportunity to make your statement. I came back to make mine.

And so, I think it is a tragedy that America is kind of told that you fellows are engaged in manipulating the way I think, that we're engaged in foreign-aid—I mean, that's a great buzzword. Anyone who's been in politics for a while. Go back into your town meetings. You want to infuriate anybody? Imply that we're engaged in foreign-aid.

Well, I am proud of what you all do and I hope that you will keep it up.

And I now conclude the hearing. Thank you very much.

(Hearing adjourned at 4:45 p.m..)

OPENING STATEMENT OF SENATOR CONNIE MACK, CHAIRMAN

Good Morning and welcome to the Joint Economic Committee's second high technology summit, "Putting a Human Face on Biotechnology."

Oftentimes when we discuss high technology—especially here at the Joint Economic Committee - we tend to speak in numbers. What is it's effect on the economy? On the financial markets? How can we continue this growth? All very important and relevant topics. But the technological revolution we are experiencing is touching all of us in a much more personal way: by improving the quality of our every day lives.

Today's panelists of industry leaders, innovators, and heroes are here to help us understand what Washington can do—and what it shouldn't do - to ensure that the biotech industry continues to thrive and develop products that will improve our standard of living.

U.S. biotechnology is clearly outpacing the rest of the world. Our biotech industry is about five times larger than Europe's. There are almost 1,300 U.S. biotech companies that employ more than 140,000 people in high wage, high value jobs.

As I see it, we've entered the era of the Innovation Economy -- a system in which we see as never before the value of the idea. Today, more than ever before in our history, brain power is being valued as the engine of economic growth.

I view this new economy as a kind of continuum—a logical progression rooted in the freedom that sets our country apart:

Freedom leads to Knowledge...

Knowledge leads to Innovation...

Innovation leads to Capital Formation...

Capital Formation leads to New Products...

New Products lead to New Jobs.

It's a virtuous cycle which has produced immeasurable blessings for men and women all around the world. It has lifted millions out of

poverty. It has stretched the limits of human achievement. And it will generate benefits tomorrow that we cannot begin to comprehend today.

And it doesn't just generate wealth—it generates progress.

As a case in point, consider biotechnology.

When it comes to strengthening government policy to advance medicine and science, let me declare my special interest right up front. I'm a cancer survivor. I'm a big believer in the miracle of modern medicine.

However, I know that for every medical miracle there are one hundred failed efforts—promising ideas—that just don't pan out.

Are they wastes of time and effort and R&D? No—not if each failure narrows the search for science and knowledge. Making miracles is hard work—expensive work. That's why we need a system that recognizes the interplay between our market economy—free enterprise -- and the pursuit of medical knowledge.

To show you what happens when we don't—when we flirt with policies that punish market incentives instead of promote them, think back to the beginning of the Clinton presidency. President and Mrs. Clinton attacked free market pricing for pharmaceuticals. Gordon Binder, the CEO of Amgen, recently testified before this committee that because of the President's price controls threats, R&D spending stagnated. The free market re-assessed what these biotech companies would be worth under a Clinton-style national health care system, and their value plummeted. $100 billion just vanished.

Now, you may be thinking, that's too bad if you're a stockholder in a big drug company. But the fact is we're all stakeholders in modern medicine's search for cures for our most deadly diseases. And that $100 billion dollars would have supported untold numbers of research projects into Alzheimer's and AIDS and cancer or cardiovascular research—research that might have saved someone's life—maybe your own.

Washington needs to maintain policies that give the strongest possible support to innovation. President Clinton received a tax package last week that included a 5 year extension for the R&D tax credit, and he vetoed it. It was the longest extension of the credit since it was enacted. The President has squandered an important opportunity to help these companies innovate.

In addition, I am strongly committed to double funding for the NIH over the next five years. Medical research is the key to saving lives. We

are on the verge of discovery in so many different areas of disease. It is crucial we provide our scientists with the tools necessary to continue the tremendous advances being made in biomedical research. My wife is alive today, my daughter is alive today, and I am alive today because of the many advances made in cancer research and early detection.

I believe it is the freedom that we enjoy as Americans that help bring us the and products that change our lives. The vitality of high-technology in this country reflects our economic freedom.

And it is our freedom that allows each and every person to innovate—to exercise the entrepreneurial spirit that turns innovation into jobs and GDP.

That's the genius of free enterprise.

That's the genius of America.

For more than two centuries it's what helped make America the envy of the world. Now, as we approach the new Millennium—it's what will make the next century a new American century as well.

PREPARED STATEMENT OF SENATOR ROBERT F. BENNETT

Good afternoon. I am very happy to play a part in this Joint Economic Committee high-technology summit. As my colleagues know, I am very involved in high-tech issues because this industry is creating the growth and jobs that our economy will rely on in coming decades. The JEC staff has put together an excellent group of witnesses today, and I look forward to hearing the testimony of this panel.

This panel will focus on the innovative role that financial markets have played in the success of the U.S. biotechnology industry. I want to thank our witnesses for taking the time to share their knowledge with us so that we can keep the U.S. at the leading edge of medical innovation. We will hear first from Peter Lynch, Vice Chairman of the Fidelity Management and Research Company. He will be followed by: Matt Andresen, President of Island ECN, Inc.; Kathy Behrens, a member of the Board of Directors of the National Venture Capital Association; and James Glassman, a Fellow at the American Enterprise Institute.

America's efficient and dynamic capital markets have been crucial to the success of many U.S. high-tech industries including biotechnology. The large and unique U.S. venture capital markets, in particular, have helped fund the R&D budgets of hundreds of small biotechnology firms. These firms often perform years of research before they are able to bring a successful new drug to market. Infusions of cash from venture capital companies make up for a shortfall of revenues in these young research companies.

In 1998, $1 billion of venture capital investment flowed into the biotechnology industry, and over $2 billion flowed into other medical technology companies. This vital flow of investment money has been described as an "umbilical cord" of support for the biotech industry--an umbilical cord which is very sensitive to potential policy changes such as increases in taxation or regulation.

In addition to venture capital, the public equity markets--particularly NASDAQ--have given U.S. technology companies a big edge over foreign companies. Initial public offerings (IPOs) have provided young medical technology companies with the funds that they need to expand and innovate. We will hear today how further financial market innovations, such as electronic stock exchanges, will provide even more efficient flows of investment capital to high-tech start-ups.

It is very important for Congress hear from experts in this areas to make sure that the United States stays at the leading edge of biotechnology and other medical fields. I look forward to hearing you testimony.

TESTIMONY OF

CAROLYN BOYER-FORTIER

BEFORE THE

JOINT ECONOMIC COMMITTEE

WEDNESDAY, SEPTEMBER 29, 1999

(145)

I am Carolyn Boyer-Fortier and I would first like to thank the Committee for giving me the opportunity to share with you my experiences as a breast cancer patient. I would also like to thank everyone at Genentech who literally have made it possible for me to be here today. Their dedication to developing new treatments for breast cancer and other life-threatening diseases is greatly appreciated by all of us.

I was first diagnosed with Stage 3 breast cancer in 1993. For those unfamiliar with the four stages of breast cancer, stage 3 and 4 diagnosis mark a very advanced stage of the disease. My doctors recommended immediate surgery and aggressive chemotherapy, which was the standard treatment at the time. The chemotherapy was quite toxic to my system and I was deathly sick, suffering from severe dehydration and nausea, shingles, and infections, for the course of the nine-month initial treatment. It took about a year to fully recover from the effects of both the surgery and chemotherapy, and I was able to return to work full time as a tax counsel.

I was disease-free for another year, but in 1996 was diagnosed with metastatic disease – that is breast cancer that has spread to distant organs. We discovered widespread metastatic disease in my liver and multiple sites in my bones, both of which are common areas for breast cancer to spread. Unfortunately, there is no known "cure" for metastatic breast cancer and patients are faced with decisions on how to best treat the disease, knowing that none of the treatments are likely to significantly prolong their life.

My doctors were understandably reluctant to predict how long I would live, but I understand that statistically a patient with metastatic disease in the liver has a survival expectancy of 18 to 24 months. And the usual treatments were again brutal to the patient's system – autologous bone marrow transplant and aggressive chemotherapy among them.

While we were deciding how to proceed, one of my doctors found that Genentech was conducting a Phase III clinical trial of a monoclonal antibody, then called her2-neu. We referred to this as a "smart bomb" because it attacks only the cancer cells and is therefore not nearly so toxic as conventional chemotherapy. Upon testing, we found that I scored the highest possible level and was therefore eligible for the clinical trial. This was the bad news/good news – bad news because it meant that my disease was more aggressive and chemotherapy resistant but good news that I could possibly get the drug in the trial.

I was most fortunate to have been randomized to receive the drug – I began treatment with herceptin and Taxol, an approved chemotherapy, in July 1996. I am very pleased to report that the adverse side effects of Herceptin are minimal. In September 1996, I did develop the congestive heart failure previously mentioned, but we have managed to keep that under control with the usual heart medications. I also encountered

the usual side effects of Taxol – hair loss, neuropathy in the hands and feet, nausea, and fatigue -- but these side effects have become fairly routine and my family and friends are accustomed to the "bald" look.

Most importantly, even with frequent treatment and a real uncertainty of how long I would be able to hold on, I was able to maintain a quality life style. With some limitations on my sports activities, I was able to work part-time, socialize, travel, play golf, and otherwise live a very full and contented life.

It is now 39 months since I started taking herceptin and the disease in my liver is still under control. I refer primarily to the metastatic disease in my liver because we thought that was the more life-threatening condition. I have greatly exceeded the statistics on survival time with metastatic disease in the liver and I am convinced that herceptin, combined with my desire to have as much quality time as possible, is the reason. I have the unusual situation of having considerable progression of the disease in my bones, even though the tumors in my liver have responded to treatment. So we are continuing with herceptin and other conventional chemotherapy to try to control the pain and disease progression in my bones.

Again, I am truly grateful to Genentech and to all of the cancer drug research and development programs that promise so much for cancer patients. I am grateful for the quality of life that this new treatment provides and for the promise of the "management" of a disease that it is, at present, considered terminal. I hope that "smart bomb" anti-cancer drugs such as herceptin will take cancer patients away from the cold brutality of chemotherapy treatment and move us towards a treatment that specially targets the cancer without targeting the whole person.

We do not have time to recount all the wonderful moments I have had in the last three years – celebrating weddings and birthdays, Thanksgiving and Christmas with family and friends, summers at the beach, and trips to fabulous golf resorts. - But life is never so precious as when you live on borrowed time. I have been so fortunate to borrow more - and I am looking forward to celebrating the new millennium with my family and friends.

PREPARED STATEMENT OF STEVEN SHAK, M.D.

STAFF SCIENTIST,
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GENENTECH, INC.
BEFORE THE
JOINT ECONOMIC COMMITTEE
REGARDING
"PUTTING A HUMAN FACE ON BIOTECHNOLOGY"
WEDNESDAY, SEPTEMBER 29, 1999

I. WHAT IS BIOTECHNOLOGY?

The founders of Genentech, Robert Swanson and Herbert Boyer, had a simple but revolutionary idea in the early 1970s – that new laboratory techniques to sequence, cut, and splice DNA might make it possible to manufacture normal human proteins and use them as drugs. It was this insight that led to the founding of Genentech in 1976 and, ultimately, the launch of the biotechnology industry.

As a research physician in an academic medical center, I was fascinated by this new scientific field called DNA technology, then being pioneered at Genentech. This fascination, coupled with the desire to make a real difference in the lives of patients, brought me to Genentech in 1986 and inspired the two projects with which I have been most involved at Genentech – the development of *Pulmozyme* for the treatment of Cystic Fibrosis and *Herceptin* for the treatment of Breast Cancer.

Art Levinson, Genentech's President and CEO, will provide an overview of the industry later in this hearing. From the perspective of a researcher, I'd like to "Put a Human Face on Biotechnology" by discussing my experience with *Pulmozyme* and *Herceptin*.

II. WHAT IS *PULMOZYME* – A BIOTECNOLOGY PRODUCT FOR CF PATIENTS

Pulmozyme was the first new drug developed for Cystic Fibrosis in more than 30 years. CF is the most common lethal inherited disease and affects more than 20,000 kids and young adults in the United States.

Kids with CF have airways clogged with thick secretions and persistent lung infections. They struggle to breath every day.

At Genentech, a significant portion of a researcher's time is his or her own, to pursue any project the researcher believes is promising. I wanted to pursue a new way of treating this terrible disease.

I discovered *Pulmozyme* in 1988 by asking a simple question: What substance is in those thick airway secretions that make them so thick? If I knew what the substance was, maybe I could clone and express a human protein which, when inhaled, would chop up those secretions and make it easier to breathe. Using technology developed at Genentech, I cloned and expressed a natural human enzyme, *Pulmozyme*, that dramatically reduced the thickness of CF airway secretions.

Testing *Pulmozyme* required almost six more years of work involving more than 1,000 Genentech employees and many CF care professionals throughout the United States. More than 1,O00 kids and young adults volunteered to participate in human tests. In October of 1992, we finally saw the results of the Phase III clinical trials. The results indicated that *Pulmozyme* could be inhaled safely and was effective – increasing lung function, improving quality of life, and reducing infection and hospitalizations. *Pulmozyme* was approved by the FDA and, subsequently, by more than 50 other countries. It is now used throughout the world in thousands of kids and young adults as part of standard daily therapy.

How important was biotechnology to the development of *Pulmozyme*? All important. While the drug is present in all human blood, it is present in such small amounts that the collection of a unit of blood from 1,000 blood donors would only produce enough *Pulmozyme* to treat one child with CF for one day. *Pulmozyme* would not exist without biotechnology developed at Genentech.

How important is *Pulmozyme* to patients? I will never forget the day I heard one of the first brave young women who inhaled *Pulmozyme* say –"After taking *Pulmozyme* I feel now when I take a deep breath, the air goes all the way down to my toes!!"

III. WHAT IS *HERCEPTIN* – A BIOTECHNOLOGY PRODUCT FOR BREAST CANCER PATIENTS

Herceptin is the first monoclonal antibody developed for the treatment of breast cancer. About a third of women with breast cancer

have tumors with a specific genetic alteration leading to overproduction of the HER2 growth factor receptor on the surface of their cancer cells. Overproduction of HER2 drives the cells to grow more rapidly and to spread sooner to other tissues.

Genentech scientists performed a dramatic feat in the laboratory. All of us have cells in our body that produce antibodies that fight infections. Antibodies are the reason why we get chicken pox only once and not again and again. Genentech scientists designed and engineered a human antibody to bind to HER2 on breast cancer cells to brake their growth.

I had the opportunity to lead the *Herceptin* clinical trial program. We performed clinical trials in almost 800 women with metastatic breast cancer, involving more than 150 academic investigators in 14 countries. The list of successful efforts to date in metastatic breast cancer is unfortunately very short.

The results of the *Herceptin* clinical trials greatly exceeded all of our expectations. In particular, the combination of *Herceptin* with chemotherapy was especially effective, leading to a 25% increase in survival. Some side effects were observed, most importantly, cardiac dysfunction when used in combination with a known cardiotoxic drug. However, most women were able to take the weekly infusions without the kind of side effects – hair loss, infection, nausea, and vomiting – that are commonly observed with cytotoxic chemotherapy.

The success of *Herceptin*, I believe, signals an end of the beginning in our fight against breast cancer. We can understand what makes a cancer cell grow uncontrollably and design, with biotechnology, new drugs that target the cancer.

I should stress that without the R&D tax credit, it's very likely this incredible success story – a story which marks the beginning of a new era of treatment for all kinds of cancers – would not have occurred. While the results of the *Herceptin* clinical trials were ultimately extraordinary, our initial results after Phase II clinical trials were not nearly as robust. In short, this is a project that was "on the bubble". For projects "on the bubble", the additional revenue made available by the existence of the R&D tax credit is the difference between termination or completion. In this instance, it was the difference between termination and an advance of tremendous significance to patients.

IV. THE PROMISE AND CHALLENGE OF BIOTECHNOLOGY

I am one of the many scientists who work at Genentech. We know the challenges of drug development and the odds of success. We know that most of our ideas in the laboratory will never satisfy our criteria for human testing. We know that, of the small percentage of those drugs that are tested in humans, only about one in five are successful. Nevertheless, we have a strong belief in the power of science and biotechnology. We thank you for your leadership in creating and preserving an environment that supports this critical research.

What does *Herceptin* mean to patients? We should listen to one woman tell her story.

PREPARED STATEMENT OF ERIN FAGAN, KIDNEY TRANSPLANT PATIENT

Hello, my name is Erin Fagan and I am from Nashville, Tennessee. I am a patient with the Vanderbilt University Medical Center. I have had a history of end-stage renal disease which began after having pneumonia when I was 13 years old. I began dialysis shortly thereafter. I received a kidney transplant att the age of 17, which was rejected 8 years later.

I have been and continue to be a peritoneal dialysis patient, meaning I am on dialysis for ten hours every night. Early this year I was suffering from severe tiredness and had become very anemic. At that time my Vanderbilt doctor, Dr. Julia Lewis, felt I was a good candidate for EPO and in April of this year I began receiving EPO treatments. At first I was receiving an EPO injection three times a week, Nut I am now getting a full dose injection once a week.

I feel great, and even better, I am able to hold a full-time job with the Tennessee State Department of Labor and Workforce/

I work as a secretary in the office of Employer Services. I also work several nights a week at the Bluebird Café—a famous Nashville restaurant where you can hear local songwriters and singers.

I know today's hearing is looking at how biotechnology drugs can change people's lives. All I can tell you is how important this drug has been for both my health, and my ability to work, and I am very excited about a future that will be less disruptive because of my illness. Thank you for inviting me to join you today, and I want to day a special hello to my Senator, Senator Bill Frist.

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TESTIMONY OF

ARTHUR D. LEVINSON, Ph.D.

PRESIDENT AND CEO

GENENTECH, INC.

BEFORE THE

JOINT ECONOMIC COMMITTEE

REGARDING

"PUTTING A HUMAN FACE ON BIOTECHNOLOGY"

WEDNESDAY, SEPTEMBER 29, 1999

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Mr. Chairman and distinguished members of the Committee. Thank you for the opportunity to testify today regarding the most important topic of biotechnology and its impact on people like you and me. It is truly an honor to testify before you today. Your leadership on issues related to innovation, and medical research and development has been critical to the on-going development of new life-saving drugs and breakthrough technologies.

Without your commitment to such important policy initiatives as funding for the National Institutes of Health (NIH) and permanent extension of the research & experimentation tax credit (commonly known as the research and development tax credit), many remarkable products would not be made available to those in need.

The subject of today's hearing cuts to the core of what the biotech industry is all about. As Carolyn Boyer and Lance Armstrong's testimony demonstrates -- the human face of biotechnology is very real. All the cutting-edge science and innovative technology of our industry is valuable only when it ultimately results in the alleviation of human suffering and the overall enhancement of human life.

Our mission at Genentech is to be the leading biotechnology company, using information and human genetic engineering to develop, manufacture and market pharmaceuticals that address significant unmet medical needs. We are committed to working with patients, families, providers and payers to improve patient care.

At Genentech we say that we are "In business for life". Our commitment to this is reflected in our history – a history that marks the genesis of the biotechnology industry. Genentech's founders, Herb Boyer and Bob Swanson, were the first to conceptualize the process of cloning human proteins for the purpose of manufacturing life-saving therapies. In 1976, Genentech was founded as the pioneering biotechnology firm with research and development, manufacturing and sales capabilities. By the early 1980s, Genentech had developed and licensed the first two products of biotechnology – recombinant insulin and alpha interferon.

As a testament to our commitment to saving lives, Genentech is among the most research intensive companies in the world. In 1996, we invested $471 million, or 49% of our income, on research and development. We reduced that amount to $396 million in 1998, or 34% of income, partially because investors are hesitant to support one-half of income going

to research. But research is our lifeblood. It gives life to the ideas we test to treat serious, unmet medical needs. Our strong portfolio of products is a direct reflection of the ideas our scientists have brought from the lab to the patient. And, as evidenced by our robust pipeline, I firmly believe the best of our science is yet to come.

In an effort to further our commitment to our patients, Genentech devised a "Single Point of Contact" (SPOC) program to assist patients and their physicians in gaining reimbursement for their care. In addition Genentech instituted our own "Uninsured Patient Program" in 1986 when we marketed our first product, *Protropin*. The program provides free drugs to patients ensuring that a lack of financial resources will not prevent anyone from gaining access to our products..

With this brief background in mind, there are a few issues on which I wish to focus today, particularly: federal support for research and development, permanent extension of the R&D tax credit, and the Medical Innovation Tax Credit (MITC).

Federal Support for Biomedical Research and Innovation is Crucial. The scientific underpinnings of the industry itself – namely, the discovery of recombinant DNA technologies – was developed in the 1970s at

Stanford University and the University of San Francisco with the help of federal funding.

As the industry has matured and grown, the ability of the federal government to either constructively nurture or inadvertently harm the industry has increased commensurately. The Joint Economic Committee (JEC) – particularly in hosting the national high technology summit earlier this summer – has played an enormously important role in highlighting some of the critical ways the federal government can advance our country by creating a more supportive environment for high-technology.

Permanent Extension of the R&D Tax Credit. Except for small increases in the past three years, direct federal support for overall research has, for the most part, been declining for over a decade. While a long-term commitment to increasing funds available to the federal government for basic research is important, maximizing private industry R&D through a permanent R&D tax credit is a necessity. Numerous studies have shown that a permanent R&D credit is a cost-effective means of ensuring that high levels of private-sector investment will continue to take place.

A short-term extension of the credit is clearly preferable to allowing the credit to lapse, however the lack of permanence severely compromises

the effectiveness of the credit for the biotechnology industry. With biotechnology R&D programs often planned five to ten years in the future, uncertainty regarding the credit can prove detrimental. The industry is required to work under the assumption that the credit may not be in effect for the entire life of the research project, which in turn means less revenue can be committed to R&D. And, this translates into fewer scientific discoveries -- fewer therapies like *Herceptin.*

Returning to our theme of "Putting a Human Face on Biotechnology", this uncertainty regarding the credit has profound implications for the patients since our industry spends much of its revenue on R&D. This uncertainty may necessitate a small firm furloughing scientists engaged in promising research. For a large firm it may mean making the hard choice to terminate or curtail a significant project. Either way, patients lose. I dare say that without the R&D tax credit, *Herceptin* simply would not be a reality. Mr. Chairman, you have long been the champion of this cause and I know that others on the Committee have been long time supporters of the credit. It is our desire to work with you to make the credit permanent.

Medical Innovation Tax Credit (MITC). Over the years, the federal government has invested billions of dollars to create a biomedical establishment of medical schools and teaching hospitals deemed the finest in

the world. The growth of managed care, coupled with cuts in Medicare payments, threatens the ability of these medical schools and teaching hospitals to carry out their vital social mission of research, training of health professionals, and the provision of indigent care.

The Medical Innovation Tax Credit would establish an incremental 20 percent tax credit for clinical trials performed at medical schools, teaching hospitals that are under common ownership or affiliated with an institution of higher learning, or non-profit research hospitals that are designated as cancer centers by the National Cancer Institute (NCI). This credit would partially offset the roughly 30 to 50 percent greater cost of doing clinical trials at these institutions. It would encourage biomedical firms to do clinical trials here in the United States while providing a revenue source for medical schools, teaching hospitals, and NCI-designated cancer centers. Clinical trials at these crown jewels of our health care system have dropped from 82% of clinical trials in 1985 to an estimate of 27% in 1996.

This narrow credit is designed to complement the R&D tax credit and has been scored by the Joint Committee on Taxation as having negligible cost so long as the R&D credit is in effect. The legislation – H.R. 1039 in the House and S. 1010 in the Senate – has attracted strong bipartisan sponsorship and support. Mr. Chairman, thank you for your vital leadership

on this important issue. I know others on the Committee are co-sponsors of this legislation, and we appreciate their support and efforts as well.

The Future of Biotechnology. The first quarter century of biotechnology has been a period of astounding advance. The next quarter century promises revelation and quantum leaps forward. The industry is on the cusp of major breakthroughs, breakthroughs that would have been the stuff of science fiction – not science – a few short years ago.

One example of where Genentech is headed in the future is our use of computers and the new technologies of bioinformatics to search large databases of information to advance our own research and medical science. Genentech's Secreted Protein Discovery Initiative (SPDI) builds on our world-class expertise in cloning and expressing genes from the human genome that encode proteins. SPDI focuses -- through the brilliance of computer technology -- on identifying the minority of proteins that are most likely to be of therapeutic interest. And because SPDI is just that -- "speedy," it has dramatically enhanced our scientific capabilities and is leading to new candidates for research. For example, SPDI has already helped identify proteins that may be useful as cancer therapies through a process called "apoptosis," which means the genetic programming of the death of cells or, in the case of cancer, tumor cells. This technology would

not have been possible 5 years ago. Both the Human Genome Project and the increases in computational capability through smaller, more powerful computers make bioinformatics work. Both the Human Genome Project and the advances in computer capability rely on federal research as the platform for future breakthroughs.

Our pipeline is very exciting and robust. In addition to apoptosis, we are making headway on an advanced form of our original product, *tPA,* which is effective in the treatment of heart attack and stroke victims. We are also moving forward with research on a product designed to block the cascade of health problems associated with asthma and other allergies, and are in the process of testing *Herceptin* on other forms of non-breast cancers as well as on earlier stages of breast cancer.

As I hope I have illustrated for you today, the biotech industry holds tremendous promise for the future and lives of so many patients facing serious illnesses. Our resolve to better their lives is unwavering, even in the context of an unpredictable financial and regulatory environment.

However, two things are predictable as we look toward the future of biotechnology. As in the industry's first 25 years, the next 25 years will require federal policies that are supportive of biomedical research and

innovation. And finally, the industry as a whole will only succeed if we continue to keep the patient – the human face in biotechnology – first and foremost in all our decisions.

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Testimony of

Henri Termeer

President and CEO, Genzyme Corporation

before the

Joint Economic Committee Summit

"Putting a Human Face on Biotechnology"

September 29, 1999

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Good morning. It is my privilege to be here with you today to discuss the future of biotechnology and its potential to improve human health. My name is Henri Termeer and I am the CEO and President of Genzyme Corporation. Genzyme is an integrated health care company which focuses on the development of innovative products and services for unmet medical needs.

The goal of the biotechnology industry is to allow people to live longer, healthier, and more productive lives.

CHALLENGES

In my view, there are three major challenges, which we must meet in order to develop the innovative health care products that will allow our society to reach these goals. These involve research and development, the risk/benefit analyses necessary for marketing approval, and the integration of new technology into the health care system.

Research and Development

The first challenge is research and development, which provide the intellectual foundation upon which the biotechnology industry is built. Both the federal government and the private sector deserve high marks for the quantity and quality of R&D that is flourishing in this country. One only has to look at the

demonstrable progress that has been made in terms of improving morbidity, mortality and the quality of life:

- Deaths from heart disease, the number one killer in the United States, declined by 36 percent between 1977 and 1999.
- During the same period, death rates from stroke decreased by 50 percent.
- Vaccines now protect against infectious diseases that once killed and disabled millions of children and adults.
- Congress's passage of the Orphan Drug Act in 1983 has led to the development and approval of over 170 products now on the market to treat rare diseases. During the decade prior to the incentives created by that law, fewer than 10 such products had been developed.

These accomplishments would not have been possible without the commitment of both the private sector and the federal government to research and development. This year, while the National Institutes of Health (NIH) will spend $14 billion for basic research, the U.S. medical technology industries will spend collectively approximately $30 billion in their own efforts to translate this new knowledge into products that provide meaningful clinical benefits with respect to a wide range of devastating diseases. It is also of enormous significance that there is a strong bipartisan consensus to double the NIH budget within the next five years. This will help to create the necessary intellectual resources for continued industrial growth, not only in terms of new knowledge, but also in the training and nurturing of the new scientists whose work will serve

as the foundation for not only twenty-first century medicine, but also twenty-first century economic growth. The U.S. is the world leader in biotechnology and our competitive advantage consists primarily of the talented people whose intellectual capital is this industry's greatest asset.

Risk/Benefit Analyses

Our second challenge is establishing a process within the Food and Drug Administration (FDA) that will make appropriate risk/benefit analyses. This is essential in order to realize the ultimate purpose of biomedical research and development: to allow new therapies that will improve patient health to become available to the patients who need them, when they need them. There has been some very significant progress toward this goal within the past few years.

The Congress and FDA have made major advances in improving the approval process for drugs and biologics with the goal of getting needed therapies into the hands of patients as soon as possible. The passage of the Prescription Drug User Fee Act in 1992 enabled FDA to hire enough reviewers so that the agency can complete its review of priority products within a six-month time frame.

The social and medical dimensions of AIDS compelled the FDA to be creative and to initiate the accelerated approval process, which has gradually expanded to facilitate more rapid development and approval of new drugs for cancer, multiple sclerosis, and other serious or life-threatening diseases. The remarkable success of that program has led the Congress to codify and expand

this effort with the "Fast Track" program for breakthrough drugs. This program also formalizes certain FDA "best practices," such as rolling review, so that sponsors can begin submitting portions of their applications before other portions are complete. Enactment of the Food and Drug Administration Modernization Act, as well as the many interim steps FDA initiated during that debate, is already beginning to pay dividends in terms of patient care.

While we have made major strides in addressing the risk/benefit questions, our thinking needs to continue to evolve. For certain diseases, society must decide if it is willing to assume greater risk in return for earlier access to potentially beneficial new drugs. This is especially important with respect to certain types of diseases, such as those for extremely rare conditions, because it simply may not be medically or financially feasible to do things the old way. The truth for many diseases today is that we need to differentiate between information that we *need* to know before treating patients versus information that it would be *nice* to know.

Integration of new technology into the health care system

Our third—and perhaps greatest—challenge is the need for our health care delivery system to be prepared to integrate the innovation we are trying to create. Unfortunately, we are much further away from meeting this challenge. If we do not move ahead in integrating technology into our health systems, patients will be denied access to significant therapies and important innovations will be lost, or even worse, never developed. Our coverage and reimbursement systems are

not equipped to welcome new technology; indeed, they are designed to discourage it. Technology has sometimes been viewed as a problem, rather than as a solution, to human suffering.

Today, if new drugs or procedures cost more than existing therapies, they may be denied to patients even if they can save both lives and money in the future. Prompt FDA approval of a product does not guarantee that patients will have access to it. In some cases, insurers are labeling new products as experimental—despite FDA's approval—and asking for additional efficacy studies, longer-term outcome data, or data obtained outside of a clinical setting. As a result, FDA's impartial determination that a product is safe and effective for its intended use can be disregarded by health plans whose own evaluation of the product is far less extensive or independent. This situation is ironic given the resources and effort that the Congress, FDA, and NIH have expended in trying to make therapies available more quickly.

There are three technology integration situations with respect to the Medicare program that I think are illustrative:

In-Patient Hospital Prospective Payment System. The prospective payment system for in-patient hospital care is designed to contain Medicare's hospitalization costs, a result that has clearly been achieved when evaluated on a cost-per-admission basis. Unfortunately, short-term savings do not necessarily produce long-term savings, especially in a system in which hospitals can keep *all* of the short-term savings they produce, but none of the long-term savings.

This is exactly the situation today under Medicare's in-patient prospective payment system (PPS). Hospitals are paid the same amount, regardless of the quality of care they provide and regardless of whether their patients must be readmitted for further treatment at additional cost. PPS provides hospitals with an incentive to reduce the costs associated with a single episode of care, but no incentive to pay for breakthrough treatments that, while initially expensive, will reduce the need and costs associated with future medical services that are thereby avoided.

Moreover, the term "prospective payment system" is misleading, given that payments are based on retrospective criteria—namely, historical costs of old treatment modalities, many of which are only marginally effective.

Consider, for example, a $200 medical device that reduces the incidence of surgical adhesions. Adhesions, a common complication of surgery in which scar tissue forms between internal organs and/or tissues, can result in bowel obstruction (which requires emergency surgery to correct) and can make subsequent surgeries more difficult, risky and costly.

Under PPS, hospitals have no incentive to use such a device, because while the cost of the device must be absorbed by the hospital out of its fixed payment, the long-term financial benefits of fewer subsequent surgeries (and reduced attendant medical costs), would accrue solely to the Medicare program and not to the hospital. In no other system do we expect investments to be made without offering any possibility of ever recouping that investment.

HCFA's Proposed Prospective Payment System for Out-Patient Hospital Departments. The Health Care Financing Administration has recently proposed a prospective payment system for procedures performed in a hospital outpatient department. It is conceptually based on the same averaging concept discussed above for in-hospital payment.

But it is also based on the assumption that the use of drugs and biologics in that setting are "usually provided in connection with some other treatment or procedure." This assumption is clearly erroneous, at least with respect to biotechnology drugs for cancer, genetic diseases, and other serious and life-threatening diseases. The fact is that, in today's medical world, many patients go to the hospital solely to receive intravenous injections of the medicine that keeps them out of the hospital for more invasive, and less effective, surgical treatments.

It is absolutely clear that the proposed hospital outpatient system would not provide adequate payment for most biotechnology products. In the short run, inadequate payment would impede patient access to new technologies, as hospitals seek to control their costs by providing cheaper—and less effective—treatments. Hospitals will not be able to offer their Medicare patients access to the most current life-saving technologies because of the financial risk to the hospital. In the long run, inadequate payment would undermine the incentive to invest in the development of such treatments.

This policy is particularly troublesome because the concept of prospective payment is based on identifying categories of care, measuring the average cost

of treating a patient in each category, and providing payments based on this average cost. Perhaps this type of cookie-cutter medical system made more sense when it was first developed a generation ago, when physicians provided a "standard" treatment to many patients with similar symptoms.

But we are entering a new medical millennium, a period in which science is beginning to allow us to differentiate among patients who used to be medically indistinguishable from one another. Breast cancer is an excellent example; biochemical tests now allow us to distinguish between different forms of this disease and identify which women are most likely to benefit from a particular drug, such as Herceptin. Or, consider Genzyme Tissue Repair's treatment for torn knee cartilage; it is truly unique because we actually grow more of the patient's own cells for reimplantation into the injured knee. The treatment consists of living cells, not chemical substances, and it is custom-made, not mass-produced.

The biotechnology industry foresees exponential growth in our ability to develop far more effective, individualistic and specific therapies than are available today as the Human Genome Project and other basic research programs provide us with the ability to differentiate patients—and therefore appropriate treatment—based on differences in genetic mutations and/or expression between individuals who suffer from the same symptoms. The Human Genome Project and similar research that is being performed in both academic and commercial laboratories may give us the opportunity to truly customize medical care. This makes it particularly ironic that HCFA is moving in

the opposite direction by proposing that patients and their diseases be classified into ever-narrower classifications.

We currently put round pegs in square holes, but only because we cannot see the differences between the two. As we begin to see these differences and to better understand the diversity of the human condition, we need a health care system that facilitates the use of customized treatment regimens, not one that ignores it.

Medicare's Coverage Policy. While the Medicare Program is in the process of revising its arcane and cumbersome coverage process for certain new technologies, there are no early signs that it will be a great improvement. After a company and the FDA spend years and millions of dollars reviewing a product for safety and efficacy and determining which populations it is appropriate for, the FDA's decision that the product can be marketed is in no way binding—and does not even provide a favorable assumption—in the Medicare program for those categories of product that the program covers. Better research and faster FDA approvals are meaningless if Medicare denies beneficiaries access to the cutting-edge therapies they need to live longer, healthier, lives. Given the rigor of FDA reviews of new drugs, biologics, and medical devices, it seems appropriate to question Medicare's processes for determining coverage. One potential solution would be to permit Medicare to automatically cover FDA-approved products for their approved uses, unless they are statutorily excluded from coverage (i.e. hearing aids and most outpatient drugs), until such time as

Medicare determines that such coverage is inappropriate because another product or treatment is clinically superior. This approach effectively shifts the burden of proof, so that new treatments could be seamlessly moved from clinical trials to FDA approval to Medicare coverage.

CONCLUSION

We have made tremendous progress in the health care field. But there is still much more to be done. There are 20 million people who are afflicted with some 5,000 rare or "orphan" diseases. Cardiovascular disease is still the leading cause of death in this country. Cancer is still the second highest cause of death; stroke is still the third highest.

Old approaches to drug discovery --- based on chemistry and a "trial and error" methodology --- have largely reached a plateau. New approaches are now being used to develop innovative solutions based on biotechnology and computer technology to improve human health. We need to maintain an aggressive focus on R&D in both the public and private sectors. We need to continue to innovate the regulatory systems so that they provide the appropriate balance of risk versus benefit to all Americans. Most importantly, our reimbursement systems need to be harmonized with our R&D efforts and our improved product approval systems. Every year innovation puts more pressure against the growing trend in this country of "first dollar" coverage. This is not a tenable long-term situation if we are to reach our potential to ameliorate serious diseases and to improve the quality and productivity of life.

Biotechnology offers us a new set of tools to achieve scientific breakthroughs that will allow us to actually reverse morbidity and ultimately cure man of some of these diseases. New therapies are in the pipeline that could completely change treatment for certain diseases and disorders: gene therapy to cure diseases such as hemophilia and diabetes, treating coronary artery disease by promoting coronary blood vessel growth, and curing Parkinson's disease by injecting dopamine-producing cells into the brain.

If the 1990s are the golden age of biomedical research, then I believe that the twenty-first century will be the platinum age.

MONSANTO
Food · Health · Hope™

Testimony of
Hendrik Verfaillie
President of Monsanto Company
St. Louis, Missouri
before the Joint Economic Committee
September 29, 1999

Mr. Chairman, Members of the Committee.

Good afternoon, I'm Hendrik Verfaillie and I'm the President and Chief Operating Officer of Monsanto Company. Monsanto, headquartered in St. Louis, Missouri, is a life sciences company committed to finding solutions to the growing global needs for food and health by sharing common forms of science and technology among agriculture, nutrition and health.

As a Belgian who has lived, raised a family and worked in this country for over 20 years, it is a high honor and privilege to be here today, and to represent both Monsanto and the biotechnology industry.

I want to share with you our vision and experience about the benefits and potential of agricultural biotechnology. There are three points about agricultural biotechnology that I wish to make:

- First, it is directly and dramatically benefiting farmers and the environment.
- Second, the industry is driven both by our growing technological capabilities and by growing global pressures to produce more food on less land and in ways that are more efficient and environmentally sustainable.
- Third, while the current benefits strongly favor the farmer, American and global consumers will be the ultimate beneficiaries of this technology.

Let me begin by putting agricultural biotechnology into context. Since humans first cultivated seeds, over 10,000 years ago, they have tried to improve plants. Though it has taken hundreds of years and hundreds of plant generations, farmers have always bred new plant varieties and created new foods and crops that deliver greater yields and more useful traits. Almost everything we eat today is the result of this successful process of improvement over time -- a successful process, albeit one that is hit and miss one.

However, from Gregor Mendal in the 18th century through the discovery of DNA by Watson and Crick in the 20th century, we have continued to learn a great deal about biology and how genes work. As a result, we can now be more precise and efficient in how we create new varieties and traits, and how we farm. Rather than rely on randomly crossing thousands of genes in the hope of creating a new, desirable trait -- or possibly and unknowingly creating a harmful trait - we can now be far more selective for the specific traits we want.

For example, we can take a single gene that expresses a desired trait - like disease resistance -- and place that gene in a seed, creating a plant better able to defend itself.

Biotechnology is a technology of information management, taking the information contained in the genetic sequence of a plant and expressing it in new and beneficial ways. Crucially, it is unlike any other high-technology on the market today. A new computer chip or software program, for example, can come to the market and recover the development investment relatively quickly. Biotechnology innovations take considerably more time.

Over 20 years ago, pioneering Monsanto scientists convinced company executives, like me, that biotechnology could create better crops and agricultural products. To bring the first crops to market required years of safety and environmental tests, reviews by EPA, USDA and FDA, and hundreds of millions of research and development dollars

Just three years ago, we finally brought the first genetically modified plant to market - a herbicide resistant plant that makes it easier for farmers to manage weeds and increase yields in a less labor intensive ways.

Today, as a result of our growing understanding of biology, new agricultural products offering greater consumer benefits are delivering an ever-wider range of benefits more efficiently and without environmental harm.

For example, we can produce crops that fight insect and bacterial pests; that enhance the vitamin and nutrition content of the plant; and, that grow in adverse conditions - such as in highly salinated ground or arid conditions.

One potent example of the practical power of this technology is Hawaii's papaya crop - once threatened by a deadly virus. Through biotechnology, papaya plants were created with a gene able to resist this harmful virus. Today, this agricultural industry, made-up mostly of small growers and once on the verge of extinction, is beginning to flourish again.

American farmers are choosing and planting biotechnology improved crops in record numbers - over 65 million acres nationwide.

In fact, Mr. Chairman, 47% percent of the cotton crop planted in Florida is genetically improved insect resistant seed - 55% percent nationwide.

If you could walk through the fields with me you would see a remarkable change in the way America farms. You would see farmers using fewer agricultural chemicals to protect and nurture crops - this means fewer chemicals in the soil and in the water runoff. In fact, recent studies by the USDA and other organizations show that, nationally, farmers used over 2 million fewer pounds of agricultural chemicals.

You would see farmers using fewer natural resources - gas for tractors, chemicals for fertilizers - to grow and harvest crops.

And, you would see farmers reaping greater yields and potentially earning more profit even in a slow farm economy.

The reason these benefits are so important has much to do the forces that are driving future development of agricultural biotechnology.

Currently, 6 billion people live on Earth. In twenty five years, there will be eight billion. These are people that must be fed. Additionally, they deserve the same things that we want for our own children -- something more than mere subsistence -- a chance to grow, thrive and contribute.

To feed this additional two billion people will require global agricultural production to increase by 75% percent.

Today, nearly six million acres of arable land - an area about the size of South America - are farmed to feed the world. Experts agree that this is about the limit of available arable land. Feeding the world in 2025 will require almost 15 million acres - about the size of North and South America combined.

Meeting this challenge with conventional and traditional agricultural technologies is problematic. Creating the arable land we need to grow sufficient food will require consuming sensitive ecological resources such as forests, wetlands and deserts. For most people, this is not an environmentally sustainable solution.

Or, we can try to bring new tools - like agricultural biotechnology - to bear.

Biotechnology is not a solution in and of itself - government and market reforms, better processing and distributions efficiencies, as well as empowerment of local farmers are all critical. However, without the benefits of plant biotechnology, the challenges ahead will be difficult - both for the United States and for the globe.

Today, American farmers play a critical role in feeding the world. Nearly 17% of global food exports originate here. Their importance will not diminish in the future. It will only increase as more efficient, effective and safe production methods are needed.

I opened my remarks speaking about how American farmers benefit from agricultural biotechnology. But, it is consumers who are the ultimate beneficiaries.

The benefits of the first generation of agricultural biotechnology products accrued mostly to farmers - and I would argue that that in of itself is a good thing. However, next generation of products - those beginning to come on-line today - will directly benefit the health, nutrition and well being not only of American consumers, but global consumers of American agricultural products.

Let me cite some examples:

- high stearate soybeans -- no hydrogenation is needed to produce margarine or shortenings, and it eliminates transfatty acids and lowers cholesterol and other health risks.

- oilseed plants with elevated vitamin levels - such as vitamin A - that will help prevent millions of cases of blindness and reduce infant mortality resulting from vitamin A deficiency.

  We are proud of this achievement and it is so important that we donated the technology to the USAID and other development groups so that it gets to people in need as soon as possible.

- Bt Corn for controlling Corn Bores and -- according to Iowa State University -- reducing mycotoxins. Mycotoxins are potentially hazardous substance to both human and animal health, released by fungus that grow where Corn Bores damage the corn plant.

Additionally, products will soon be available that lower blood pressure or that contain antioxidants that may help prevent cancer as well as products with nutrients to help fight heart disease - particularly important traits as America's population ages.

Further, as our abilities and understandings improve, we will be able to provide health and nutritional products tailored to meet a consumer's special diet and nutrition needs.

The US biotechnology industry and market is, by far, the world leader. With over 140,000 people employed in the United States directly in biotechnology, it is not just an industry of the future, it is an industry of today. The positive impact of this growth can be felt nationwide. For example, in Massachusetts, we recently created a genomics alliance between Monsanto and Millennium Pharmaceuticals Inc. This venture will apply

genomic technologies to the discovery and development of plant and agricultural products. It will also encourage innovation and new, emerging business. Today, this project employs over 100 people in high-paying, high-tech jobs. Tomorrow, the benefits of the discoveries made by this alliance will benefit the economy of the entire region and country.

However, it takes 8 to 10 years to bring a new product to the market and the investment is substantial. Research and development costs are growing - over $9.4 billion in 1997 alone (according to the OECD).

To maintain global leadership, industry, consumers and policy makers must continue to work together. Regulatory processes and programs must be constantly revisited and refined as the technology changes and progresses. Research and development tax credits - which you, Mr. Chairman have so admirably championed - should be extended to encourage further growth and innovation, new ideas and new businesses across the industry. We must also prepare our children with the scientific education and skills needed for the jobs being created.

Today, as we speak, this industry is still in its infancy - there are possibilities and benefits that we've not yet contemplated. There are also challenges - both of public acceptance and understanding – that we've not yet met.

Public confidence in the safety, science and oversight of this technology is critical. When that confidence is challenged - as we see today in Europe - issues such as trade and suspicion dominate the public discussion. We recognize our responsibility both to hear the concerns that are raised about this emerging technology and to act to meet those concerns. We also must reinforce the strong scientific verification - including years of testing and regulatory oversight - that has made agricultural biotechnology safe, healthy and environmentally sustainable.

Working together with Congress, our customers, consumers and scientist we are committed to cement the exemplary health, safety and environmental record achieved by our industry.

The challenges are many, yet we are proud that the benefits of this technology - for both farmers and consumers - are both clear and growing. And, also, as a leading pharmaceutical manufacturer, Monsanto joins with our colleagues at the table to underscore the potential and power of pharmaceutical biotechnology to improve human health through better drugs and medical treatment.

We are willing and eager to work closely with you, Mr. Chairman, and with Congress to create an environment that will encourage and enhance this emerging industry.

I look forward to answering your questions.

Lewis E. (Lonnie) Edelheit
Senior Vice President- Corporate R&D
General Electric Company

Joint Economic Committee
September 29, 1999

My name is Lonnie Edelheit, and I am the Senior Vice President, Corporate Research and Development at the General Electric Company. I've spent my career as a researcher focused on GE's medical systems business, as the manager of GE's CT scanner business, and as the founder of my own venture-capital backed medical technology company, so it's a pleasure to be here today to talk about the great things happening today in medical science.

The technology advances I've seen in my 30 year career are truly staggering, and have stimulated terrific growth in the industry. Ten years ago GE Medical Systems had sales of $2.5 billion and employed 8,000 people. Today, GE Medical Systems employs 20,000, and has sales of more than $6 billion.

When I began at GE in 1969, x-ray was our most advanced form of medical imaging. Disease in the human body was often identified through painful and invasive surgery, compounding the trauma of being ill with the challenge and ordeal of getting an accurate diagnosis. Mortality rates for diseases like breast cancer were exceptionally high because detection was only possible when physical symptoms appeared – a point that was often too late.

Today, our ability to see inside the body with sophisticated imaging equipment like MRI, CT, ultrasound, and digital x-ray has profoundly changed the pattern of medical care - and greatly improved the survival rates for people with diseases like cancer and heart disease. Leading cancer officials have suggested that our ability to detect cancer earlier through imaging may well be the single greatest practical advance in America's 25-year war on cancer. Moreover, the digital revolution has allowed great improvements not only in our diagnostic equipment, but also in the productivity of doctors, nurses, and hospitals.

Tomorrow holds even more promise. Non-invasive surgery is developing rapidly, with techniques that use focused ultrasound energy under MRI guidance, and others involving the use of optical fibers and microwave surgery. Brain function imaging, using MRI, is already being used to plan neurosurgery. In the future, it may help us understand tragic diseases like Alzheimer's.

Most of this progress in medical imaging has been funded by companies like GE, and industry continues to spend hundreds of millions of dollars on medical imaging research each year.

But public policy and federal funding play an absolutely vital role in the development of bio-medical technology at the beginning, middle, and end of the process, and that's what I'd like to talk about today.

At the beginning of the process **our work rests on the huge body of fundamental and basic research** conducted in universities and funded largely by the federal government. Industry cannot do this, yet advances in basic research are vitally important to the country as a whole. In addition, by focusing on universities, we continue to educate and train the engineers and scientists who have made our country and our industries so strong.

In the middle of the process, **federal funding for research on national priorities affects what industry prioritizes.** This is an important phase in the because in the final analysis, the only way any new basic research or technology breakthrough gets into the market to touch people, is by

industry producing a product or service. On our own, we in industry prioritize research that we can get to market with modest risk and a strong probability of return. This means that good ideas with above average risk, or with less certain returns, are often postponed or overlooked in favor of other opportunities. But with the government, industry can also address national priorities, reach farther and take bigger risks, and speed up technology development.

Consider breast cancer, for example. In the last several years, the government made early detection of breast cancer a high priority, as an important way to save women's lives. As it happens, the mammography machine business had not changed for years. As a result, it has been difficult for industry to justify enormous additional investments to advance mammography technology. Enter the National Cancer Institute, the Army Breast Cancer Research Program, and DARPA's medical technology program. These programs supported substantial new investments in breakthrough digital X-ray and mammography technology. The result? Pending regulatory approval, several companies, including GE, will bring to market digital mammography systems – the first major advance in mammography technology in a generation. Without a major boost and push from the government, and the research program to back it up, this would have taken years longer.

Industrial R&D tends to operate within a fairly narrow risk/reward window. Government programs can turn a porthole into double doors.

In the medical technology industry, government support at key moments has encouraged industry to take on high-risk, high-payoff opportunities – opportunities that mean challenging accepted technological limits. In the early 1970s, the first computed tomography ("CAT scan") machines required four minutes to make an image of the inside of a patient's head. The government challenged industry to make an image anywhere in the body in less than ten seconds, and created a program to support research on the best ways to achieve that goal. No one had a clue how to do it, but the program galvanized a lot of companies, including GE. Even though GE failed to get that particular contract, we took on the challenge and achieved the goal anyway. I estimate that this NCI program jumpstarted the industry, making us move about three years faster than we would have otherwise. Many lives were saved as a result. I don't think that the National Cancer Institute has ever taken enough credit for the critical role it played in the development of this life-saving technology, in part because they did not recognize the effect the program had in spurring competition among the medical companies.

Many opportunities to make a difference like this exist today. Chairman Mack, I understand you've been to NIH to see a cardiac MRI system, which we are helping to develop. Non-invasive cardiac imaging is a huge opportunity – and a great example where development could be going faster. Lung cancer screening is another.

The survival rates for patients found to have lung cancer today is only 12%, largely because we can't see the cancer with our current imaging technology until it has progressed too far. This summer, researchers published a study showing by using specialized yet affordable CT imaging to find the lung cancer early, we may be able to dramatically improve lung cancer survival rates. Modest federal funding could reduce the time it will take to get such a system to market by half.

Finally, at the end of the process, **FDA regulation and HCFA reimbursement have a significant effect on which technologies make it market to benefit patients, and how fast.** And unfortunately, while we have ample evidence of the value of industry and government working cooperatively in the development of breakthrough technologies, government regulatory procedures frequently frustrate our efforts to make those very technologies available to benefit patients.

FDA product review and clearance is working better than it has in years – but chiefly in clearing the backlog of "substantially equivalent" technologies. Current resources and policies are not designed to keep up with the technology explosion and digital revolution. This problem will likely intensify in the years ahead.

With the help of Congress, industry and the FDA must work toward developing a better way to approve breakthrough technologies that combines the speed and relative simplicity of the 510(k) process with the benefits of a PMA. Also, we must continue to press the Agency to implement, in a timely way, important reforms like international harmonization and third party review, that were part of FDA reform.

The FDA approval process, combined with a cumbersome and out-dated decision-making process for reimbursement, has slowed significantly the introduction of breakthrough technologies, often available to patients and their physicians in Europe, Asia, Canada and South America far ahead of the US. PET technology (positron emission tomography) in particular is an example of how both FDA and HCFA policies, though well-intentioned, have prevented US patients from getting access to vastly superior cancer detection technology. We know Congress is working on improving the HCFA reimbursement process. We must stress however that FDA approval and HCFA reimbursement are very different processes and must be kept separate. However, they should be conducted in a parallel fashion to help practitioners use the best technology for patients as soon as possible.

In summary, the future will bring even more remarkable advances in medical and bio-medical technologies than we have seen to date. These advances will bring life-saving benefits to thousands of people and continue to spur the U.S. economy. Government decisions -- to support fundamental research, to spur R&D in priority areas, to regulate products, and to permit reimbursement for their use -- collectively have a dramatic impact on what new technologies are developed and brought to market, and how fast. But as the pace of technology development continues to accelerate, government must adapt its traditional approach to funding and regulating this promising technology . Otherwise, the very policies that have done so much to promote technology innovation and the economic vitality of the medical imaging industry may well begin to constrain it.

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Testimony for the Joint Economic Committee of the U. S. Congress
Biotech Summit
Wednesday, September 29, 1999

Christine K. Cassel, MD
Professor and Chairman
The Henry L. Schwartz Department of Geriatrics and Adult Development

I am an internist, geriatrician and Chairman of the Henry L. Schwartz Department of Geriatrics and Adult Development at the Mount Sinai School of Medicine in New York. I have specialized in the care of the elderly for twenty years, leading academic research and training programs at several major medical centers. One of my areas of study is the demography and epidemiology of aging. While it is now common knowledge that Americans are living longer than ever before, most people do not realize that there is still a lively debate surrounding the question of why this is the case. Advances in biotechnology are central to this discussion.

Life expectancy in the United States has almost doubled within this century alone. By 1965 the average life expectancy in the United States had increased to approximately seventy years old. The framers of the Medicare program assumed that we had probably reached something near the genetic limit to life expectancy. Shortly after Medicare was implemented, mortality rates for older people began to decline again and have continued to do so up until the present time. I believe, as do a number of other experts in the field, that while the advances in life expectancy that occurred in the early part of the century were primarily related to

improvements in public health and living conditions, the advances we are now seeing are related to advances in medical care.

The dynamics promoting increases in life expectancy are not limited only to the United States. The United States is approximately number thirteen in the world in both average life expectancy and in percentage of the population over the age of 65. Thus, we can look to other countries for examples of how different health care systems and different socioeconomic frameworks have both contributed to successful aging and helped to cope effectively with the demands on families and on society.

Because this promise of successful aging is now something that most Americans are aware of, we must be realistic about the investment it takes to achieve that promise. Individual decisions and health behaviors count for a great deal, but increasingly medical care provides meaningful advantages in quality and length of life.

In the last twenty years advances in science and medicine have been unprecedented. Much of this has come from biotechnology research funded both publicly and privately. In the field of aging itself dramatic advances, particularly in surgical techniques and in pharmacological treatments for common and chronic diseases has made it possible for us to prevent many of the causes of disability that

occur with age, such as stroke and heart attack and ameliorate the symptoms of other chronic diseases to improve quality of life, function and independence of those who suffer from them. In this category are disorders such as osteoarthritis (affecting 50% of people over the age of 65), diabetes, osteoporosis, Parkinson's disease, and depression. We are now on the verge of effective medical treatment for Alzheimer's disease, one of the greatest threats to successful aging. Advances in genetic research have helped us to understand telomerase, which may lead to a cure for macular degeneration responsible for one-third of the causes in blindness in the elderly. Treatments for cancer have become much more successful in older people, but require expensive adjuvant therapy because the elderly are more susceptible to side effects of highly toxic chemotherapy agents.

Surely, dramatic advances will continue and it should be possible to promise these treatments to older people. The expectation that 80% of Americans will live well past the age of 65 is a dramatic success story of modern civilization, one that we should be celebrating. Unfortunately, because of the potential financial burdens this success story is often seen as a burden and a problem rather than as one of the tremendous benefits to families.

The major problem is affordability and insurance coverage. Increasing numbers of older Americans do not have medication coverage as part of their Medigap insurance. Increasing numbers of employers are limiting or eliminating

supplemental insurance as a retirement benefit. Most older people are not wealthy and cannot afford out-of-pocket expenses that range from three to six thousand dollars a year, and sometimes more for some of the newer medications on the market. Characteristically, as people age they may suffer from several chronic diseases. As medical treatment for chronic disorders improves, disability decreases. But this has real financial costs to families. A patient needing ongoing treatment for hypercholesterolemia (to prevent heart attacks), hypertension (to prevent heart attacks and stroke), arthritis (to increase mobility, health and productivity), osteoporosis (to prevent disabling fractures), diabetes (to reduce the risk of kidney failure, blindness and amputation of limbs), may easily rack up charges of $10,000.00 a year. Any intervening illness such as cancer, depression, recovery from spine or hip fracture can lead to thousands of dollars of additional costs. If the older persons themselves are not paying these costs it may fall on adult children who are struggling to put their children through college and raise their families. Thus, it is essential that we as a nation find a way to make the advances of the biotechnology industry available to more than just the most wealthy among us.

I am aware that this hearing is not addressed at specific policy strategies to address the problem of coverage, access and affordability of medications and new technologies. As a researcher and physician caring for patients, I do however know that it is very important as we celebrate the success of the biotechnology industry to realize that if we do not address the affordability question we may find ourselves in

a situation where we can do miraculous life-saving, life-prolonging and life-enhancing things, but they will only be available to a small number of the population. The burden of chronic disease, dependency and disability will fall on families and on society if we allow this to happen.

Statement of Matthew Andresen

President, The Island ECN

Joint Economic Committee

September 29, 1999

Chairman Mack and Members of the Committee:

I commend the Chairman and the Members of the Committee for holding these timely hearings concerning the importance of biotechnology companies and the central role they play in improving quality of life. I appreciate the opportunity to appear before this committee to testify specifically concerning our views on venture capital, the biotechnology industry, and their dependence on an efficient marketplace.

INTRODUCTION

I am Matthew Andresen, President of The Island ECN ("Island"). Island is an automated trading system for equity securities. It gives brokers the power to electronically display and match customer orders in equity securities. We function as a pure auction market -- directly matching buy and sell orders. Island was founded approximately three years ago with the intent of providing all market participants - from individual investors to large financial institutions - with the ability to execute securities transactions on a level playing field, at an extremely low cost and without the presence of intermediaries or dealers.

On an average day, Island will trade over one hundred and twenty million shares – approximately 12% of the transaction volume on Nasdaq. All this will be done on a single computer about the size of Chairman Mack's podium. In the previous three months, Island traded over six billion shares worth almost $400 billion.

We are here today because an integral sector of our economy, biotechnology, is having difficulty raising capital. In fact, capital raised through Initial Public Offerings or IPOs declined 48 percent in 1998. Although there are recent signs of improvement, more needs to be done to ensure the health of the biotechnology sector. As a secondary market for publicly traded equity securities, Island plays an important role in strengthening the equity markets and facilitating the raising of capital. Island's goal is to create a secondary equity market that is transparent, fair, and efficient. The achievement of this goal will increase investor participation and, in turn, the amount of capital available to fund biotechnology as well as many other types of companies.

With respect to strengthening our capital markets, Island would like to take this opportunity to applaud Chairman Levitt's recent remarks. We believe that his visionary speech points the way toward significantly improving our capital markets and ensuring the United States' continued leadership into the new millennium. The plan would create competition between markets and allow innovative new markets to compete on a level playing field with traditional exchanges. History shows that there will be two beneficiaries of this increased competition: Investors will receive improved service, innovative new services and dramatically lower costs; and companies will have access to more capital to help bring a new product or idea to market.

ISLAND AND CAPITAL FORMATION

Island appears in front of this Committee today with a unique view of venture capital. Island is both a recipient of valuable venture capital financing and a significant contributor toward making venture capital funding more available.

As a recipient of venture capital financing, we understand the importance of the infusion of capital to seed a business idea that promises to improve the lives of consumers. Americans have an instinctive appreciation for the importance of entrepreneurs in creating and pursuing new opportunities. It is not an overstatement to say that the future economic success of our country depends on it.

A little over two years ago, Island was a company of just three people with an idea in search of a market - literally. Island's goal was to make the securities markets fairer, cheaper, more transparent and thus more accessible to all investors. Making such a dramatic impact on the equity securities market, however, required the infusion of a considerable amount of capital. To continue to grow our core business, Island needs to continually make substantial investments in technology and staff. Finding the financing to make such investments was a challenge. Finally, approximately four months ago, Island received $30 million in venture capital financing that will allow us to carry out our plans to forever change how securities are traded. We are already seeing the benefits of competition in the equity markets as both Nasdaq and the NYSE have announced their intentions to make significant structural changes to enable them to compete with systems

such as Island. In the end, investors will benefit from the improved services and lower costs that inevitably result from competition.

As I stated earlier, we are not only a recipient of venture capital financing but, as an equity market, Island is making an important contribution toward making venture capital financing more available.

While we might be distracted by the endless stream of opinions, hype, and data, the most important function of the equity markets is raising capital for corporations. A venture capitalist's key motivation is the possibility of returns far in excess of the risk-free rate of return. Increasingly, the so-called "exit strategy" for a venture capitalist is an Initial Public Offering or IPO. IPOs are only possible, however, in an environment where there are investors willing to purchase the shares of the company going public. As the level of participation in the market has increased substantially, we have seen a growing appetite of investors for shares of IPOs. In turn, the success of IPOs encourages venture capital companies to make further investments which creates a "virtuous circle" of investment. We have all seen the investor frenzy over IPOs of companies in the Internet and communications sector. This seemingly insatiable demand for Internet related IPOs is providing Internet related companies with access to unprecedented amounts of capital far earlier in their life cycles. This access to capital will undoubtedly contribute to our country's continued leadership role in the Internet and in technology generally.

Another impact of the increased demand for IPOs is a reduction in the amount of venture capital needed prior to accessing the public market. Traditionally, a private company would go through a number of rounds of venture capital financing until it was deemed ready to go public. Now, companies are going public, in many cases, after just one round of venture financing often eliminating the so-called "Mezzanine" financing. In short, the equity markets themselves have become a source of venture capital. Average Americans can now participate in "public" venture capitalism.

Despite the relative ease of which many Internet companies have tapped the public equity markets, the biotechnology sector has, until recently, had a much more difficult time raising money through either the public markets or venture capital. The reasons put forward to explain this situation include: 1) a diversion of funds to the Internet sector; 2) venture capital firms focusing on large scale projects; and 3) a consolidation in the banking industry. We all recognize the tremendous amount of investment needed to fund the research and development necessary to bring a breakthrough technology to market. Bio-technology is a cash intensive business and its importance in improving all of our lives demands that we find ways to ensure its continued health and vitality.

While Island is not an expert on the biotechnology sector specifically, we do believe that one of the best ways to provide bio-technology companies access to capital (whether it be through venture capital or through an IPO), is to ensure that the trend towards cheaper, fairer, more transparent and more accessible capital markets continues.

Over the past few years, America has seen an explosion of interest in the equities markets. Seemingly every day, as investor participation in the market continues to increase, markets are setting records for volume. Average Americans are participating in the stock market at a level never seen before whether indirectly through their 401k company retirement plans and mutual funds or directly through newly opened on-line brokerage accounts. Driving this increased participation in the market is the democratization of information and plummeting transaction costs.

It was not long ago that we followed the stock market by checking our local paper each morning or by calling our broker to find out the latest news. Information about companies was difficult to find and often solely the province of large broker-dealers that wrote research reports shared only with clients. Commissions on transactions were routinely hundreds of dollars, thus putting investing out of reach for many. With the advent of the Internet and the revolution in communications, the landscape has been irrevocably altered. Whatever the social setting, it does not take long these days for the conversation to turn to the stock market.

No longer do you need to wait for the morning paper to follow your investments. Through the power of the Internet, investors have access to real-time stock quotes. Island, however, goes one step further in providing information to investors. In sharp contrast to traditional stock quotes that tell you that a share of Microsoft "just traded at 90", Island is the first marketplace to provide a free, real-time display of all orders on its Island BookViewer. Why is this so important? In addition to providing the investor with

the highest bid or lowest offer in a security, the Island BookViewer enables an investor to see all the orders to buy and sell on Island. This gives investors the ability to better gauge supply and demand and thus more accurately price their order.

Perhaps more importantly, the public display of the Island BookViewer reduces the informational and temporal advantages traditionally enjoyed by market makers and specialists, thus creating a level playing field for all investors. By eliminating these time and place disparities, Island helps lower the hidden costs of trading associated with higher spreads and inferior executions. In fact, according to the Securities and Exchange Commission, investors have saved hundreds of millions of dollars since the inception of ECNs just as a result of lower spreads.

In addition to reducing trading costs by leveling the playing field and eliminating hidden trading costs, Island also contributes to lower commissions. Specifically, for each share executed on Island, our revenue is $.00075 per share for each side of a transaction on Island. This is an exponentially lower price than was traditionally charged by some of our competitors. These lower execution costs are passed on to the investor in the form of lower commissions. Island believes that the plunging average on-line brokerage commission of approximately $15 has lowered barriers to entry and enabled widespread participation in the market.

Finally, electronic marketplaces such as Island have led to an increase in market participation by fostering confidence in the integrity of the market. Island strongly

believes that an electronic marketplace is inherently safer, fairer, and easier to surveil. For example, people in a trading crowd on the floor of an exchange may possess more information then the average investor sitting at home. Through surveillance and the implementation of restrictions on the activities of those in the trading crowds, regulators try to level the playing field. As recent events have shown, however, no amount of surveillance can completely prevent the misuse of information.

Electronic trading systems, such as Island, by eliminating informational disparities, reduce the chance of improprieties. Electronic trading systems permit the individual investor to electronically step into a virtual trading crowd and compete directly. This removes the potential for many abuses possible in a conventional trading crowd. At Island, we like to say that "you can't front-run real-time!" If all orders delivered to the virtual trading crowd are anonymously displayed in real-time to everyone in the trading crowd, no single person has an informational advantage.

Furthermore, because electronic trading systems automatically capture and store all information, the market surveillance mechanisms are uniquely equipped to perform the regulatory market surveillance functions. For example, at Island, we have the ability to take surveillance to the next level. Historically, surveillance has involved analyzing completed transactions for abuses like price manipulation. At Island and presumably other electronic trading systems, we are able monitor orders as well as completed transactions. Order information is imbedded in the data received from every user of the

Island system. Electronic markets can monitor a much larger and complete dataset for trading abuses such as price manipulation.

In sum, the increased transparency, lower barriers to entry, and enhanced integrity resulting from electronic trading systems such as Island have assisted in the creation of a nation of venture capitalists. As a result, the health of the IPO and secondary markets for any industry, including Bio-technology, are directly affected by developments in the secondary equity markets. Accordingly, in order to help Bio-Technology companies, and for that matter any company, raise money through venture capital or IPOs, Congress must remain vigilant in ensuring that competition between marketplaces continues to flourish. Through competition, our capital markets will be ever more accessible to all investors.

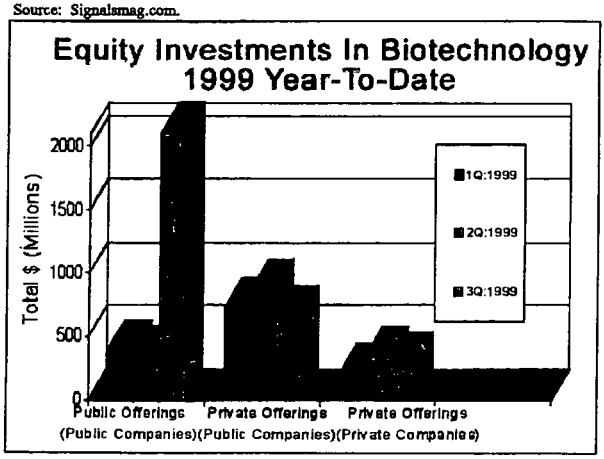
CONCLUSION

In conclusion, Mr. Chairman, I believe that we are witnessing one of the most dramatic and exciting times in the history of financial markets. We at Island are proud to have been at the forefront of this flourishing financial services industry. As a result, today's investors have better access and information than ever before, allowing them to reap the benefits of new services and lower costs. We hope that these improvements lead to an increase in the amount of financing available to Bio-technology companies and, indeed, all companies. I look forward to working with you and your colleagues to explore these exciting opportunities.

Information from Mr. Andresen to Senator Bennett

During the first three quarters of 1999, Biotechnology firms raised over $5 Billion through both Public and Private Offerings. Private biotechnology companies, to date, have raised a total of $843 million through private offerings and are likely to reach the $1 billion mark by the end of 1999. This total is just slightly lower than the amount raised in venture capital financing during the same period in 1998, which totaled $894 million. Public biotechnology companies have raised an additional $2 billion through private offerings. Finally, during the first three quarters of 1999, biotechnology companies have raised almost $3 billion. (Source: Signalsmag.com)

Source: Signalsmag.com.



For the first three quarters of 1999, the totals of each are as follows:

Public Companies dealing with Public Offerings totals $2,885,000,000.
Public Companies dealing with Private Offerings total 2,235,000,000.
Private Companies dealing with Private Offerings total $843,000,000.

PREPARED STATEMENT OF M. KATHY BEHRENS, CHAIRMAN OF THE NATIONAL VENTURE CAPITAL ASSOCIATION

BEFORE THE JOINT ECONOMIC COMMITTEE

BIOTECH SUMMIT

SEPTEMBER 29, 1999

Good afternoon. My name is Kathy Behrens and I am Managing Director of Robertson Stephens Investment Management in San Francisco. I am also Chairman of the National Venture Capital Association, which is comprised of more than 330 professional venture capital firms dedicated to stimulating the flow of equity capital to emerging growth and developing companies.

With a doctorate degree in microbiology from UC Davis, where I performed genetic research for six years, I am focused on investing in the life sciences area, and, in particular, in biotechnology companies. In fact, over the past 15 years I have sat on the Boards of many biotechnology companies including Mitotix, Tularik, Cell Genesys, Insite Vision, COR Therapeutics and Mercator Genetics.

These companies and others in the Robertson Stephens portfolio illustrate that biotechnology is giving new and renewed hope for people who suffer maladies across virtually the entire spectrum of diseases and afflictions.

In fact, without patient investment from venture capitalists, this industry literally would not exist. According to NVCA statistics, since 1990 venture capitalists have pumped over $5.8 billion into over 550 biotechnology companies. While companies in California, Massachusetts, Washington state, Pennsylvania and New Jersey have received the largest share of these dollars, biotechnology companies in 34 states have received venture financing. Venture capitalists invest on average over $10 million in each biotech company the industry funds.

There are currently 79 biotechnology therapeutics/vaccines approved for sale by the Food and Drug Administration. 350 biotech drugs, vaccines and therapies for conditions such as cancer, arthritis, genetic disorders, burns and blindness currently are in clinical trials. Most of these companies have been financed by venture capital.

However, there are serious and profound problems facing us right now. While the venture capital industry is investing more money in more companies than ever before, the amount of money going into biotechnology as a percentage of overall dollars disbursed is beginning to decline. While venture capitalists increasingly pursue Internet deals which have the ability to garner a significant financial return in a relatively short time-frame, the biotech arena, which faces heavy government regulation and requires longer-term and more patient investing, naturally suffers.

It is likely in 1999 that fewer new biotechnology companies will be funded by venture capitalists. Also, public biotechnology companies are having problems securing additional needed money to get through the FDA pre-market approval process. The market for biotech initial public offerings declined from 22 in 1997 to 6 in 1998, and 1999 does not look very optimistic either.

Does the federal government have a role in seeing that this trend does not continue? I believe it does. At this moment and in this very building a significant debates are taking place regarding health care reform, patent reform, tax policy and immigration policy. The results of these debates may directly affect the future of biotechnology companies and in turn impact the availability of the innovative products these companies are developing.

As a seasoned venture capitalist, I can attest to the enormous risks biotech companies face in an attempt to bring a product to market. Health care proposals which impose drug price controls or Medicare drug benefits which provide marginal reimbursement can create a perception or reality that our potential return is limited or at greater risk.

The biotechnology community has for years been asking for patent reform, only to get bills passed in the House and have them not acted upon in the Senate. The issue is simple- we need to make certain that the new 20 year GATT patent term does not end up shortening the terms of patents when the government causes delays in the issuance of a patent. Last month the House passed the American Inventors protection Act by

a vote of 376-43. NVCA hopes that the Senate will act quickly as well.

Advances in biotechnology will only occur with a highly educated workforce. Education reform is critical, but it will not occur overnight. In order to ensure the continued dynamism of the biotechnology industry, Congress needs to increase the number of H1-B visas available. New visas will be available shortly with the commencement of the new fiscal year, but this relief will only be temporary as the number of visas we need to keep our companies running is much larger than the number of visas available in the upcoming year.

The biotechnology industry currently is undergoing tremendous consolidation through mergers and acquisitions which on the whole is positive for the industry.

Pooling has been the most desired method of structuring acquisitions, but the Financial Accounting Standards Board has proposed eliminating pooling of interest accounting. By limiting our ability to use mergers and acquisitions as an exit strategy you limit our ability to invest in biotechnology.

I understand that the Senate Banking Committee is looking at holding hearings on this issue. I urge you to support this effort so that you can hear the reasons why changes in pooling accounting policy could very well limit the amount of capital invested in biotechnology.

I want to thank the Congress for enacting a capital gains rate reduction several years ago and urge you to move further in this regard. If a broad-based capital gains rate reduction cannot be passed this year, serious consideration should be given to enacting the Dunn/Matsui bill which would create a zero capital gains rate for investments in equities of entrepreneurial firms. This incentive is tailor-made for biotechnology and other emerging growth companies and would certainly help alleviate some of the funding problems biotech companies currently face.

Finally, I want to thank you for holding these hearings. Your effort to highlight the contributions that biotechnology has already created in our society as well as the problems we now face will help us move forward.

PREPARED STATEMENT OF JAMES K. GLASSMAN, RESIDENT FELLOW, AMERICAN ENTERPRISE INSTITUTE

Mr. Chairman, members of the committee:

Thank you for inviting me to give testimony at this very important Biotechnology Summit. I am honored to join you today, and especially to be on the same panel as Peter Lynch, who has been a hero of mine for a long time.

My name is James K. Glassman. I am a resident fellow at the American Enterprise Institute, a Washington think tank. For six years ending this past June, I was a regular columnist on financial, economic and political matters for the Washington Post. Next month, I begin a financial column for the Reader's Digest, the world's largest-circulation magazine. With the economist Kevin Hassett, I am co-author of a new book on the stock market, "Dow 36,000," which has just been published by Times Books.

You have heard today about the wonders that the biotechnology industry has already produced and those that it will certainly produce in the future. I want to talk to you today about one of the elements that is essential to the success—indeed, the survival—of the biotech industry. It is capital, which is merely a fancy name for money for investment.

The biotech industry needs capital to fund its research, and that capital is not easy to come by. The next panel of CEOs will probably be more specific about some of the problems of funding biotech—including a lamentable lack of understanding both at the political and venture-capital level of where innovation comes from and just how important it is.

I want to be much more general.

This is a nation of wonderful entrepreneurs, hard-working scientists, great managers, fabulous ideas. But they can't bring products to the marketplace without capital. Where does that capital come from?

Lately, much of it has come from abroad. Over the past 17 years, while the U.S. has undergone the greatest single round of prosperity in its history, capital inflows for portfolio investment purchases (stocks and bonds) have increased at an average annual rate of 19 percent. Net foreign inflows to this country: $4.8 trillion—a figure that amounts to 2.5 times the annual federal budget and about half of our annual GDP. A big, big number.

But will this strong flow of capital continue unabated? There are doubts.

Right now, the U.S. is attracting capital because of our sensible regulation, decent tax rates (which could become more decent, just as regulation could become more sensible), low inflation and entrepreneurial culture. But Europe and Asia are developing along similar lines, undergoing the same managerial revolution that we did in the early 1980s.

In other words, the rest of the world will have other places to invest as time goes on.

What about capital from within?

There, the picture is not nearly as bright as it could be. A major source of capital is personal savings—which the Bureau of Economic Analysis defines as the difference between after-tax income and expenditures. Unfortunately, the official household savings rate fell in May to its lowest level in recent history: minus -1.5 percent. The latest number is minus 1.4 percent. To give you an idea how low this is: In 1974, when Congress created the IRA to boost the incentive to save, the savings rate was 9.5 percent.

Why so low? One immediate reason is a little technical: after-tax income does not include the capital gains that Americans earn on their stock and bond holdings. But, frankly, spending those gains is not a very good idea. They should be reinvested anyway.

Also, Americans simply like to consume. It's their money. They should be able to do what they want with it. Right?

Well, yes and no. Americans are not saving enough—and, more importantly, not investing enough in biotechnology and other equities (that is, stocks) for two reasons that can be remedied.

The first is the subject of the book, titled "Dow 36,000," that I have co-authored with Kevin Hassett, a former senior economist with the Federal Reserve Board.

We argue that scholars, Wall street analysts and media pundits have scared millions of Americans away from the stock market with continual talk of bubbles and imminent crashes—talk we have heard all the way up from 1982, when the Dow was at 777, to the present, with the Dow over 10,000. These frightening judgements are based on a flawed model of stock valuation, a model that has been repudiated by the facts. In our book, we offer a new model, and we encourage Americans to

invest in the stock market in a prudent, long-term fashion with a sensibly diversified portfolio.

The other reason Americans aren't saving and investing enough is that public policy deters it.

And this is the main message I would like to leave you with today.

Americans are not investing because we have a tax code that encourages consumption over saving. We tax the earnings of corporations, then tax the dividends they pay, then tax the income from those dividends and the capital gains that result, and then, as if that isn't enough, we tax the estates of those with the prescience and the discipline to invest for the long term.

What kind of system is that? Yes, we have IRAs and 401(k) plans, but they are burdened with all sorts of restrictions.

And let's not forget Social Security. Including the employer's portion, 10 percent of the salaries of working Americans go into a system that produces returns of only one or two percent. If just as small portion could go into the stock market, Americans could develop real wealth, and get a taste for investing that would most certainly lead to a higher savings rate. In fact, I believe that even President Clinton's USA Accounts represent an impressive step in the right direction. Something needs to be done.

Indeed, as I see it, the most urgent economic issue on the policy agenda is wealth creation. Half of Americans have no wealth at all—no significant bank account, no stocks, no bonds, nothing to pass on to their kids, nothing for a decent retirement other than Social Security. That is a shame.

For most Americans, the best chance for a comfortable retirement--in many cases, the only chance—is through stock market investing.

The flip side to this deficiency is the lack of capital I discussed earlier. In the case of the biotechnology industry, capital saves lives. There is no substitute for it, and the need is urgent.

Thank you.

Biotechnology and the Public Interest

Daniel Callahan

Testimony Presented to the Joint Economic Committee of the U.S.Congress

September 29, 1999

My name is Daniel Callahan, and I am the Director of International Programs for The Hastings Center in Garrison, New York, and a Senior Fellow at the Harvard Medical School. I want to talk about biotechnology and the public interest. By that topic I mean to raise this question: how can biotechnology, full of promise for human welfare, be developed in a way that will potentially benefit everyone, not simply those of us who might individually be helped or might gain a financial benefit?

It is proper and important to ask this question and to pursue it with all the creativity and rigor that the scientific possibilities have elicited. A failure to do so might introduce both some biological hazards, always possible with new technological applications, and the social hazard that the potential human benefits will not fully be realized or be widely and fairly distributed. Those like myself, professionally focused on biomedical ethics, will surely have much to say about all this. But we will be commenting from the sidelines.

It is far more important that the scientists carrying out the research, and those who find or supply the money to support it, take the lead in fostering the importance of the public interest perspective. One reason to do so is simply that those two groups have been the most articulate and persuasive in touting the immense possibilities of biotechnology to contribute to a better life for human beings. They then have an obligation, it seems to me, to do all they can to make certain that happens. The other reason turns on obligation as well: the duty of the inventors and funders of biotechnology to take full social responsibility for the outcome, whether for good or ill.

From the perspective of ethics and the history of the biological sciences, two features of biotechnology are particularly important. One of them concerns the type of intervention into, and manipulation of, nature that is at stake. The other is the difficulty of gauging the long-term consequences of biotechnology.

Human beings have always worked to change nature, partly out of a need for self-protection - think here of the development of vaccines to prevent disease, or pesticides to save crops - and partly out of a desire to better their lot, of which the automobiles, computers, and airplanes offer obvious examples. Biotechnology, however, works to change nature from the inside, altering genes and changing the nature of organisms - and gene

therapy offers an example of such alteration. But as hard as it might have been to foresee the impact of automobiles on our lives - if anyone a century ago had tried to do - it is even harder to guess what the effects of modifying agricultural products or human traits will be. It is common enough to speculate about the possible benefits of such developments. It is no less necessary to speculate about the potential harms or, perhaps more likely, the potential mix of harms and benefits which is one of the vexing ways nature often responds to human intervention.

There have been two responses at the extreme to such speculations. One of them has been to see ethical worries and free-floating anxieties about the future simply as an obstacle to progress, a kind of needless hand-wringing that should be put aside in the name of a bold and visionary science. The other has been to see biotechnological manipulations of nature as a way either of illegitimately "playing God"; or, in its more secular version, as a dangerous tinkering with nature - that nature which has its own evolutionary good reasons for the way things are, including illness, disease, and bugs that eat up the crops. The first response takes the potential benefits seriously, the potential hazards not at all. The second response assumes a nature that never was and never will be without change and alteration, some of them from its own casual evolutionary forces and some from human interventions. "What's the big deal anyway?" is the way it is often put.

Neither of these responses is adequate or sufficiently balanced. Nature is surely open to human intervention, even if we are not sure how far that can go. We would not be here at all if it were not. If we do not know where the boundaries of danger lie with biotechnological interventions, we need not be excessively fearful as we push forward on the frontier of knowledge. At the same time it would be foolish to adopt a stance of careless recklessness, thinking we can get away with anything, or that if we do something harmful more technology can undo the harm.

In Europe what has been called the "precautionary principle" has gained a following: move slowly and carefully and thoughtfully, making as certain as possible in advance that biotechnological innovations will not have harmful consequences. Among the consequences most to be feared are three:

--that irreversible genetic or other biological changes will be introduced into nature, our own human nature or that of animals and plant life

--that damaging and difficult to control social changes will be set afoot, decisively altering, perhaps, parenthood and the family

--that changes hostile to our human dignity and important values will be insinuated into the cultural fabric of our lives and our societies, suggesting that we are no more

than complex machines, to be played with as we see fit

Yet a perspective that begins with fear or anxiety will not by itself offer sufficient guidance, nor is it likely to hold up in the face of insistent individual wants and demands for this or that piece of supposed progress - such as cloning a human being - or in the face of commercial allurements, which have been known on more occasions than one to turn our heads. I would like to offer instead two positive approaches to the ethical and social challenges of biotechnology. One of them is the need to work toward a sustainable medicine and a sustainable agriculture in the future. The other is to pursue broad population benefits for society, not simply the benefit of individuals.

By a "sustainable" medicine and agriculture I mean the aspiration that those two enormously important institutions, fundamentally necessary to our survivor and well being, will in the future remain affordable and equitable. Almost everywhere in the world medicine and the health care systems that provide it to people are caught in an increasingly difficult dilemma: the growing benefits of medicine are now clashing with the costs of that medicine.

The reasons for that dilemma are not hard to discern. There is the aging of populations, now well advanced in the developed nations, but now appearing as well in the developing nations. There is the cost of the technological innovations constantly being introduced into health care systems. It is generally estimated that somewhere between 30-40% of increased costs can be traced to those technologies. And then there is increased public demand. None of us want to get sick or to die, even though that is our biological fate, and we respond with enthusiasm to technologies that lengthen our life and improve its quality. To further complicate matters, the principal burdens upon aging societies are chronic illness and disability. For the most part, those conditions are not easy to cure, as the century-old war against cancer shows, but are instead mainly amenable to amelioration and marginal improvements. Those improvements are surely welcome but they amount to an expensive form of trench warfare, where millions of dollars are spent for every inch of health gained.

A sustainable medicine needs the help of a biotechnology that can effectively cope with chronic disease and do so in an affordable way. Pharmaceuticals that cost in the thousands of dollars a year for patient care may be affordable when the number of elderly is still manageable. But that will be insupportable when the number and proportion of elderly double and triple, as they are projected to do over the next twenty to forty years, with the greatest increase coming with those over 85. Our society already has trouble paying for the expensive AZT "cocktail" for those afflicted with HIV disease. Imagine the problem we will have if a comparably effective cocktail is developed for Alzheimer's Disease with its estimated four million sufferers.

No doubt a good market for expensive drugs can be found in affluent countries even if not everyone can pay for those drugs. It would be a shame, however, and a great failure of biotechnology if it could not do better than that. We are all, rich and poor, subject to the ills and failings of the body and the mind. An equitable provision of health care requires an affordable medicine. Without such equity there is a heavy economic and social burden of disease, with grave consequences for economic productivity and social well-being.

Equity is not a luxury. It is a necessity in modern societies. An affordable medicine is a necessity to sustain that equity. A biotechnology that can find cures for disease, that can find ways to reduce disability, that can better understand the genetic basis of disease and do something about it, will make a grand and historic contribution to an improved human life. If it can figure how to do this in a way that societies can comfortably pay for, the gain will positively be magnificent. And if it fails to do so, some of us will be gainers but most of us will be losers.

A comparable challenge can be found in the production of food. While world population growth has slowed in recent years, it is still a reality and will remain so for decades to come. There will be more and more mouths to feed. At the same time, the available land for viable agriculture steadily diminishes, forever captured by urban and suburban sprawl. Biotechnology has become a necessity in order to provide the wherewithal for a sustainable agriculture. In this case that means not simply an affordable agriculture so that everyone on the planet has enough to eat, but also an agriculture that can sustain itself in the face of more people and less land. That daunting requirement can only be met by a constantly improved, even ingenious agriculture, precisely of a kind that biotechnology can provide.

Yet again, if the cost of this agriculture renders it unaffordable to poor countries, or if it is not developed with their needs in mind, then there will be the loss of a critical opportunity - and the likely loss of many lives as well. While it is only fair and reasonable that those companies that have the nerve and the capital to pursue enhanced food and improved animal production reap a decent return on their investment, it will not do at all for them to have a stranglehold on the means of production.

You and I can live with, and enjoy, food produced on far distant and massive industrial farms, but then most of us are not subsistence farmers as so many poor people are. The latter need to be free as possible to produce what they need to live on. A sustainable agriculture, affordable and universally available, is the only kind of agriculture the world can morally afford.

The logic of sustainability is a population logic. It recognizes that there is a great difference between individual benefit and population benefit. Modern scientific medicine has been very good at providing individual benefit but far less skilled in providing population benefit. The fact that perhaps no more than 30%-40% of the great increase in life expectancy during the 20th century in the United States - some 35 years since 1900 - can be traced to medical gains tells the real story here. Most of the gain has come from public health improvements, from better diets and life styles, and from higher income and greater education. It is those gains that most contribute to a sustainable health care system. They did so in the past and they will do so in the future. The number of people who make it to age 90 by frequenting intensive care units is very small. Most of them have been able to avoid doctors, hospital and medicine almost altogether. That is what good population health means.

Many of the advertised benefits of biotechnology are individual benefits: possible cures for dread disease, possible new ways of providing needed organs (perhaps from animals), possibly more commercially efficient means of food production. But it will be the contribution of biotechnology to an overall improvement in human health - not this or that individual miracle that will be the most telling story. The question is not whether some of us might eat better in the future. It is whether all of us can eat better. Our private interest is that each of us will live long and healthy lives, and we will look to biotechnology to help us do this.

But the public interest lies in the benefits biotechnology can bring to whole societies and whole populations. Those benefits will not appear unless biotechnology can keep its contributions safe, which will require imagination, caution, and foresight. Nor will they appear if biotechnology can not keep its products economically affordable. That will require economic restraint, perhaps some sacrifice of profit, but most of all a scientific and venture capitalist imagination that is fully ambitious to promote everyone's good. Why not?

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Daniel Callahan is Director of International Programs at The Hastings Center and a Senior Fellow at the Harvard Medical School. Trained as a philosopher at Harvard, he is a co-founder of The Hastings Center, a research organization devoted to ethical problems of medicine, biology, and the environment, and located in Garrison, N.Y. An elected member of the Institute of Medicine, National Academy of Sciences, he is the author of many articles and books, the most recent of which is *False Hopes: Overcoming the Obstacles to an Affordable, Sustainable Medicine* (Simon & Schuster/Rutgers University Press). [e-mail: callahan@thehastingscenter.org]

PREPARED STATEMENT OF DR. JOHN K. NIPARKO, PROFESSOR OF OTOLARYNGOLOGY; DIRECTOR OF OTOLOGY, NEUROTOLOGY AND SKULL BASE SURGERY, THE JOHNS HOPKINS HOSPITAL

You and I share an obligation to the children of our nation. It is that common obligation that has brought me here today. Part of that obligation is to provide the tools that children with disabilities need to engage in the world around them and experience life to its fullest. We now have a growing opportunity to offer these tools to children born without hearing.

With me today is Julie Steinberg and her daughter, Rachel. Rachel, was born with an invisible handicap. As a consequence of a simple twist of genetic fate she was born without hearing. In the past, that handicap would have threatened her ability to hear speech and environmental sounds. She would have missed out on the sensations that make up so much of a child's everyday experience--her mother's, father's and brother's voice, a knock at her bedroom door, birds singing, and music--the rhythms and melody of life that contribute to a child's cognitive development.

Rachel's handicap would have also threatened her acquisition of one of the most human of all behaviors, that of spoken language. Language is best characterized as a "window on our thoughts." It is our language that enables us to report facts, to express our opinions and emotions, and to exchange information with the hearing world around us.

With a severe congenital hearing impairment, aggressive strategies are needed to enable a child to develop functional language. These strategies now employ a revolutionary development in communication technologies--the cochlear implant. The cochlear implant is a remarkable blend of digital circuitry and information processing. The evolution of the cochlear implant can be considered monumental for several reasons. Cochlear implants represent one of many innovative technologies that enable the rapid transfer of processed information. A unique feature of implant technology, however, is that it represents an alliance of strategies for processing information that use both manufactured and natural, neural circuits. The cochlear implant is best characterized as a device that provides access to the sound environment, enabling the hearing pathway to respond to environmental and speech sounds.

The principle underlying prosthetic stimulation of the auditory periphery in profound deafness is this: although specialized receptors of

the inner ear may be absent or fail to trigger electrical signals, large reserves of nerve fibers exist in the auditory nerve of profoundly deaf individuals. Furthermore, these nerve fibers retain the ability to respond to prosthetic stimulation. Through learning in the early, formative years or by virtue of auditory memory, the auditory centers of the brain are capable of processing information from the implant to enable speech comprehension.

Advances in cochlear implantation stem from emerging technologies that provide the means to process sound into meaningful codes, and present such codes efficiently over an implanted system of stimulating contacts placed within the inner ear. Most systems consist of similar component parts: microphone, speech processing unit, batteries to drive the system, and an implanted receiver and electronics package connected to an electrode array. The design of the electrode array must incorporate biocompatibility, mechanical stability, and practical fabrication, and permit atraumatic insertion.

Haring loss is the most prevalent chronic human disorder, with rates varying from 83/100,000 population among all ages to 360/100,000 for those age 75 and over. Severe-to-profound hearing impairment is of particular concern given the degree of disability it entails. A recent survey of individuals with severe-to-profound deafness showed the following characteristics compared to other disabled individuals: lowest median education achievement level; lowest median annual family income; lowest rate of participating in the labor force; lowest rate of persons in professional/technical job positions; highest rate of persons reporting that they were limited in performing their daily activity; and the poorest (self-rated) general health. These findings are undoubtedly rooted in the separation and isolation felt by many hearing impaired individuals--separation from the environment, from educational and recreational activities that involve hearing environmental and voiced sounds.

The advent of the cochlear implant has also called attention to the support that is needed for a meaningful and timely exchange between the innovators of digital technologies and those of us in the University centers of research and clinical care. Just as access to key technologies is critical to the care I provide, information regarding the consequences of deafness is key to those who, for example, design a chip to encode the critical elements of speech sounds. This exchange carries critical implications for the selection of candidates for an implant, the time of implantation and the optimal strategy to be used in restoring hearing. I

congratulate this committee on its decision to explore programs that would expand these interactions.

We have learned in our combined experience with over 200 children that a focused program of rehabilitation in combination with the innovative technologies of the past decade can provide the gift of language to profoundly deaf children. I know that Rachel and her family can express this much better than I can. I'd like to introduce Julie Steinberg and her daughter Rachel.

TESTIMONY

of

Ed Fritzky
Chairman and Chief Executive Officer
Immunex Corporation
Seattle, Washington

Submitted
To
Joint Economic Committee
Congress of the United States

Biotechnology Summit

"Putting a Human Face on Biotechnology"

September 29, 1999

Introduction:

Good afternoon, Chairman Mack, Vice-Chairman Saxton, and Members of the Committee. My name is Ed Fritzky. I am the Chairman and Chief Executive Officer of Immunex Corporation. I thank you for the opportunity to participate in this biotechnology summit. I am honored by your invitation to testify and your interest in Immunex's contributions to medical treatment.

Immunex was founded in 1981 in Seattle, Washington and now employs 1,000 people who are helping discover new ways of treating disease, using advanced biotechnology as tools. Our research concentrates on mastering the biology of the human immune system and has provided dramatic health benefits for patients. As a result of our work, for example, older patients suffering from leukemia can live longer with a drug called Leukine®, and rheumatoid disease patients who have failed standard treatments can move again, thanks to ENBREL® (etanercept).

Our research focus has produced a pipeline of potential new products that target some of the most serious medical challenges known to man – cancer and rheumatoid disease, but also multiple sclerosis, heart disease, asthma, and osteoporosis. Currently, Immunex Corporation markets seven therapeutics to health care professionals in the U.S.

Our most recently approved product, Enbrel®, is used for the treatment of moderate to severe rheumatoid arthritis (RA). In November 1998, after ten years and $200 million invested in its development to date, Immunex brought Enbrel® to market, the first of a revolutionary new class of drugs to treat the devastating effects of rheumatoid disease. Enbrel® dramatically illuminates **the human face of biotechnology,** by

- allowing people to return to work and school;
- giving disabled people the ability to leave their wheelchairs behind and walk again;
- allowing mothers to play with their children;
- relieving people of the constant pain that once defined their lives; and
- enabling people to reclaim their lives.

The Tragedy of Rheumatoid Disease:

To understand just how important the discovery of Enbrel® has been for those with RA, it is essential to understand the heavy toll of this disease.

- RA is a chronic and potentially disabling disease that afflicts two million Americans at their peak of productivity. RA is a progressive, debilitating, potentially fatal disease. RA attacks the joints causing constant pain and swelling that can result in crippling and disfigurement.

- RA is typically diagnosed when a patient is in her thirties, forties or fifties, and it affects three times as many women as men.

- Unlike osteoarthritis which results from wear and tear on the joints, RA is a disease in which the body's immune system mistakenly attacks the joints and surrounding soft tissues.
- RA makes simple tasks such as getting out of bed, walking, shaving, and buttoning a shirt difficult or impossible, and can end a person's ability to work, take care of a family and enjoy life.

The human, clinical, and social impacts of RA have been extensively documented in medical and economic literature. The financial consequences are enormous. It is estimated that:

- RA burdens the U.S. health care system with more than 9 million physician visits and 250,000 hospital admissions annually;[1]
- RA and its related conditions lead to $17.6 billion a year in lost productivity due to lost wages and days of work;[2]
- Finally, the indirect economic costs of RA total approximately $5 billion a year.[3]

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[1] Arthritis & Rheumatism, "Guidelines for RA Management", ACR Clinical Guidelines Committee, May 1996.
[2] ibid.

The Enbrel® Story: Reducing the pain, disability, and economic losses attributable to Rheumatoid Disease.

In the late 1980s, Immunex researchers discovered a molecule that is a key player in inflammation. They developed a novel way to control inflammation, and its deleterious effects, by using the body's own elegant solution.

Enbrel® interrupts the inflammation process in a highly targeted manner, which is remarkably effective. Almost two-thirds of those who take Enbrel® experience significant improvement in pain, mobility and swelling within three months, and this includes those for whom no other therapy provided relief. As one measurement of relief, patients on Enbrel® no longer have to endure three hours of "morning stiffness", the time it takes to get moving again after sleep or rest. Enbrel® reduces it to just 30 minutes.

This novel therapeutic approach has changed the way rheumatoid disease is – and potentially other serious disorders will be -- treated in the future.

Let me tell you about just two of the pioneers of this new treatment – people on Enbrel® - who have shared their stories with us:

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[3] Journal of Rheumatology, "The Costs of RA: Absolute, Incremental and Marginal Estimates", Yellin, 1996.

Kaitlyn: Kaitlyn's mother Arlene has told us, "People don't realize how much more work it takes to care for a child who can't dress herself, brush her teeth, brush her hair, or get her own breakfast."

Kaitlyn, was severely stricken with Juvenile Rheumatoid Arthritis (JRA) at four and a half months old. Later this year she will celebrate her ninth birthday. Arlene describes Kaitlyn as a happy, well-adjusted child who smiles a lot and has friends at school who like to help her. Although she has used a wheelchair for more than two years, she has been mainstreamed at school and has an aide who accompanies her to all her classes and assists her in using the bathroom.

Kaitlyn's symptoms began with an erratic high fever and a traveling rash that is characteristic of Juvenile Rheumatoid Arthritis (JRA). However, it took hundreds of tests, including painful bone marrow biopsies, to pinpoint the diagnosis. Kaitlyn's symptoms are systemic. With the exception of a couple of fingers and toes, all of Kaitlyn's joints are involved, including her jaw and neck. Doctors routinely describe Kaitlyn's case of JRA as the worst they have seen.

Kaitlyn's hands are deformed, her range of motion limited. Some of her growth plates have seized, causing Kaitlyn to be very short and slow-

growing for her age. Kaitlyn is a foot and a half shorter than her sister who is sixteen months younger. Between the age of two and six, Kaitlyn was able to take about ten or 20 steps at a time, but the pain and swelling in her ankles and knees prevented further walking. When her hips could no longer bear her weight, Kaitlyn was unable to walk. At just about every visit to the doctor, Kaitlyn rated her pain at a level "10".

Until recently, Kaitlyn was not able to roll over by herself at night, so Arlene or her husband, Darrell, would have to get up five or six times a night to readjust Kaitlyn and try to make her more comfortable. Arlene would sometimes have to vary Kaitlyn's diet, because it was difficult for her to chew properly.

When Kaitlyn was diagnosed with JRA, Arlene quit her job to care for Kaitlyn full-time, which cut the family income in half. Even though she had worked for the federal government for more than 23 years, Arlene needed two more years of service in order to draw retirement benefits.

Before Enbrel®, Kaitlyn was on heavy doses of prednisone and naprosyn. At various times, Kaitlyn was also given methotrexate, cyclosporine, and cytoxan, all of which were of limited help and caused severe side effects. Kaitlyn's mom was very concerned that the prednisone would bring on early puberty and stop Kaitlyn's already slow growth.

Kaitlyn started Enbrel® in May of 1999. After the first shot, Kaitlyn – and her parents – slept better. After the second shot, she could spread her fingers for the first time in a very long time. Kaitlyn's prednisone dosage has been reduced to 1 mg every other day, which is the lowest dosage she has ever been able to tolerate. She is also off the naprosyn, which was causing blood in her urine. Kaitlyn's hemoglobin and platelet counts and sedimentation rates have improved significantly.

Now, Kaitlyn can stand up and take four or five steps on her own. Arlene marvels that Kaitlyn can now sit on her father's shoulders. Kaitlyn is able to write better at school, and she can brush her teeth and sometimes brush her hair. She has grown an inch and a half and gained several pounds, and her lower jaw has stopped growing. Arlene has ordered a walker for Kaitlyn and hopes to get her into physical therapy so she can learn to walk again.

Arlene tries to minimize discussion about pain, because she does not want Kaitlyn to focus on her pain, but she has noticed that Kaitlyn does not complain as often about pain since she has been taking Enbrel®. Arlene can tell, by the way that Kaitlyn carries herself, that she is in much less discomfort.

In addition to getting more sleep, Arlene says she feels more peace of mind now that Kaitlyn is not in constant pain. "No parent wants to see her child in pain, especially when it's every day of her life." She is also pleased that she has been able to return to work as a secretary at the Naval War College.

Arlene is thankful that her insurer (Blue Cross/Blue Shield) is covering the costs of Enbrel®; otherwise, she would not be able to afford it. Without the Enbrel®, Arlene is certain that Kaitlyn would still be waking up in pain, not growing as well, and not learning as well at school because of her discomfort. "My husband and I have told 25 to 30 people with arthritis to look into Enbrel®. We'll tell anyone we can, because it has really helped us so much."

Joan London: "There is not an aspect of my life this disease has not touched."

Joan London was an active healthy, professional bowler and mother of two young boys when the stiffness and pain she was experiencing was diagnosed as RA. After having her third child, her RA flared up and Joan required wrist surgery to restore mobility.

After this surgery, Joan returned to work as a part-time teacher and started a tutoring service with many fellow teachers working for her. At this point, Joan's RA was episodic, sometimes painful and sometimes not. Joan continued her career, going to graduate school and then becoming a correspondent for the Houston Chronicle. In 1983, she became the media relations director and Assistant Director of Internal Communications for the Texas Children's Hospital. During this time, Joan's disease progressed to the point where she was unable to stand on her feet because of pain. She reports, "I had to hypnotize myself in order to walk down the hall of the hospital."

When both Joan's hands needed surgery in 1996, she was no longer able to work. Joan is now a Medicare beneficiary under the disability eligibility provisions of the program. Joan tried to pursue a Ph.D. in journalism, with the objective of returning to work to teach journalism. However, a horrible flare-up of her disease required her to undergo extensive surgery to fuse four vertebrae in her neck. This surgery will leave her with 30 percent mobility in her neck. She estimates the surgery costs to be $70,000. She requires an aide seven days a week and physical and occupational therapy twice per week.

To treat her disease, Joan had been on gold therapy (she was allergic to it) and was on methotrexate for ten years. Joan has a good friend, Judith

Levinson who has regained significant mobility and no longer lives with constant pain. However, because Joan relies on Medicare for her insurance coverage, she is not eligible to receive Enbrel®, if it were to be prescribed for her.

Joan says, "I've tried to remain an active individual all my life and earn a living, but this disease has interfered at every turn."

The Medicare Challenge:

Joan's story illustrates the most difficult challenge we face: the adverse effects of discriminatory coverage policy in the federal Medicare program.

The federal role in biotechnology research and development can be characterized as a three legged stool: federal funding, through NIH for example, invests in basic science research; the Food and Drug Administration (FDA) determines whether a drug or biologic may come to market with the goal of improving medical care while ensuring safety for consumers; and federal health insurance programs, like Medicare and Medicaid, reimburse for health care services. Increasing federal investment in basic research and continuing to streamline and improve the drug approval process are critical to ensuring innovation in high technology.

For the disabled and elderly, the third leg of the stool – coverage under the federal Medicare program – poses the greatest challenge, limiting access to self-injected biotechnology therapies, like Enbrel®, while providing access to intravenous and physician administered therapies. Access to biotechnology therapies is particularly vulnerable to the design and operation of the federal Medicare program. Medicare is the single largest payer for health care services in the U.S. Medicare is the primary source of coverage for seniors in this country. For many it is the only source.

All new health technologies and therapies that serve seniors must meet Medicare program and policy requirements to be covered and reimbursed. Unfortunately, Medicare coverage policies are rooted in 1960's medicine, long before biotechnology enabled us to create these breakthrough therapies. At that time, biotechnology was not an industry, and coverage updates adopted since 1965 simply have not kept pace with developments in the field. The result is a program that discourages innovation and denies seniors access to breakthrough therapies. Medicare's policy regarding self-injected therapies is an example.

Enbrel® was designed to be easily used by patients. It is a twice weekly injection that can be self-injected or given by a care-partner at home. Because biologics are solely human proteins and cannot be ingested, they must be administered systemically (through an injection or intravenously).

Even without a drug benefit, Medicare covers many outpatient administered drugs and influences biotechnology development dramatically. Currently, Medicare will cover outpatient drugs that are administered incident to a physician's service in an office, hospital, or clinic setting. Medicare will NOT cover self-injected drugs or biologics, because they do not require a physician to administer them.

This coverage anomaly means that intravenous therapies that must be administered in a hospital or infusion center are covered while self-injected therapies that are administered by the patient at home and treat the very same disease are not. This is true even where the self-injected version would be less costly to the Medicare program. Such a policy encourages development of the less patient-friendly, physician administered drugs, to the detriment of the patient.

Thus, biotechnology companies have an incentive to develop the more expensive, clinic or physician office based therapy. Companies that want to provide the best therapy in the best form for the treatment of a patient can be penalized by Medicare if this treatment is designed to be self-injected.

Enbrel® is a perfect example. It is a self-injected biologic. Thus it is not covered by Medicare. Supplemental insurance does not fill in the Medicare coverage bias against breakthrough biotechnology therapies, because the benefits may include substantial co-payments and are typically capped at levels well below the cost of expensive breakthrough biotechnology therapies. For many disabled individuals and seniors,

paying out of pocket is not feasible, so they are denied access even when no other drug or combination of drugs has successfully controlled the disease. Yet, approximately 55 percent of RA patients are Medicare beneficiaries.

Every day we hear from patients. We hear from those like Kaitlyn's mother whose worst worry is no longer their debilitating disease. And we hear from people like Joan London who know that a therapy is out there that can eliminate their pain and give them their lives back, yet they do not have access to it because of Medicare coverage policy.

We have Rheumatoid Disease patients making choices between what is covered and what is not, rather than choosing what would be best to treat their case and would allow them to return to work or eliminate the need for daily assistance.

This problematic Medicare drug coverage policy has three economic effects:

1) RA patients remain less productive than they might be if they had access to the full range of therapies. They are denied the therapy that might move them from disabled to able-to-work status.

2) Private market biotechnology research and development is unintentionally steered toward products that require physician or hospital administration. This ultimately encourages development of a less efficient and more costly health care system.

3) Health care that relies on hospital or clinic-based delivery is made more difficult for those living in rural or medically undeserved areas.

The Solution:

Whether as part of a Medicare reform proposal or as other legislation, this anomaly in Medicare coverage policy that denies access to self-injected therapies, like Enbrel® must be corrected.

I am encouraged by the tireless efforts of Senators Gorton and Murray to urge the Senate Finance Committee to address this issue in its Medicare reform legislation. Representatives Dunn and Inslee along with the entire Washington House delegation introduced a bill (H.R. 2892, Access to Innovation for Medicare Patients Act) that would correct the problem. In addition, Representative Cardin's Medicare prescription drug bill addresses this issue for a set of chronic diseases, among which is RA. Other proposals, including some sponsored by Members of this Committee, also address this issue by providing stop-loss coverage that would protect those with rheumatoid disease and other patients from running out of insurance coverage when health care expenses reach a certain level.

We should not wait for Medicare reform to address this issue. This is not a major change in the program. It is a small change that can help many elderly and disabled Americans enormously. It is a simple solution to a glaring inequity. I urge you to cover self-injected biologicals that are used as alternatives to already covered "incident to" drugs.

To ensure that government policy lets the private market work and bring innovations to health care, federal policy must require the Medicare program to cover self-injected biologics that may be prescribed in place of covered therapies.

The human face of biotechnology is all around us. It's on the faces of patients who beam in gratitude for the exciting new treatments, it's the financial community that underwrites the search for new cures, and it's the industry's employees from coast to coast who have the vision to create the future, and change the face of medicine forever.

Thank you.

4725 NORTH PAULINA STREET
CHICAGO, IL 60640

Sept. 1, 1998

Dear Ones at Lamamuney,

This letter has been a long time coming, and is very much overdue. I don't know that you need to hear this, but I feel the need to tell you how much you mean to me.

When I awaken during the night, and it's dark and quiet, I think of you. When I roll over easily and find another comfortable position I feel so grateful to you, and I thank God for you, and ask Him to bless all of you, each and every one. As I go back to sleep, I have a smile on my face, because I'm thinking of you.

You have changed my life. I used to just make it 'til after dinner, when I would lie down on the couch in the family room, with my feet on my husband's lap, where he would massage them to the point of my unconsciousness. He would watch TV alone, 'til bedtime, where I would then lie in the same position all night, because it was too

painful to toss and turn. Then in the morning I would have to "break myself out of a mold" to start another day.

Now, we've gotten rid of that couch. We're taking a computer course in the evening. We've come back to life!

Is it any wonder that I love you so?

To you scientists, I thank God that you were born, and I praise Him for every day that you live.

Sincerely,
Elizabeth Petersen

Raymond A. Kent School of Social Work

University of Louisville
Louisville, Kentucky 40292
(502) 852-6402
Fax: (502) 852-0422

UNIVERSITY of LOUISVILLE

June 15, 1999

Dr. Joseph Berman, Chief Medical Officer
Anthem
4361 Irwin Simpson Road
Mason, OH 45040-9498

Re: *Enbrel*

Dear Dr. Berman:

Thank you for covering *Enbrel*. I know that it is expensive and sometimes we do not expect HMOs to do the right thing when it's expensive. You have, however, and I need you to know that it is appreciated, and how important it is to me.

After starting on *Enbrel*, in my first checkup with my Rheumatologist, Dr. Gary Crump, he asked about my Arthritis and I replied "What Arthritis?" *Enbrel* makes that much difference in my life.

I had made several adjustments in my home and office environments so that I could keep doing the work I love so much: teaching research to Social Work doctoral students and directing our doctoral program. I had faucets and other equipment changed at home, had levers put on all of the doors that I use in my office building at the University of Louisville, opted for a palm pilot so I wouldn't have to carry so many computer disks and a heavy appointment book, and used the speaker phone in my office when I could. I also bought duplicates of many of the reference books I use daily both at home and work because I couldn't carry them. Decisions about what to wear depended on their fasteners and who was around to assist me, and selection of cookware for dinner depended on what I could lift.

I am accustomed to moving around very quickly and resent, I'm afraid, anything that slows me down. I loathed having to ask others to carry my briefcase to meetings (there are implications to asking students and even colleagues) and even scheduled some meetings to accommodate my limitations at the expense of others—not the way I want to be. I, like most people, did not want to give up my treasured independence, but before *Enbrel* I was losing it quickly.

Now, I carry the briefcase in either hand, open any door with any handle with either hand, turn any faucet with either hand, go anywhere for anything I need to do, hold the phone to my ear so that the caller has more privacy, wear what I want, and even use the right skillet for fixing dinner.

My needs, however, are not as critical as those of many I know, e.g. single parents caring for children and working on their Master's or Doctoral Degrees in Social Work, sandwich generation adults on whom both their parents and their children depend, and of course the many professionals whose work is critical to the quality of life in our communities. We need, for example, teachers, doctors, nurses, fire fighters, legislators and police officers to be functioning at full capacity when we need them. As you can see, when functional capacities are diminished for one, the quality of life and work are diminished for all.

Given the number of citizens benefiting from *Enbrel*, the availability of this product is surely making a profound improvement in the quality of life for whole communities, one individual at a time. But very quietly. I only know what a difference it makes for me – I am on the job, doing my job. People can count on me. I am not always aware, however, of those around me whose skills and services would not be present if they could not function at full capacity. Given that about 17% of the population has Rheumatoid Arthritis, the impact of *Enbrel* on the Louisville community alone could be dramatic—quietly dramatic.

Thanks to *Enbrel* and your coverage, fewer of us are now diminished in our capacities to work and lead active lives, and are contributing to society instead of drawing from limited resources. Again, In terms of productivity as well as in human rights and service issues: if one is diminished, all are diminished. With *Enbrel*, not so many of us are operating with diminished capacities.

Sincerely,

Ruth Huber

Ruth Huber, Ph.D.
Director, Doctoral program

cc: Dr. Gary Crump
7-A Suburban Medical Plaza, Louisville, KY 40207
Dr. Kathy Thomas
6400 Dutchman's Parkway, Suite 340, Louisville, KY 40205
Dr. Sheri Kalbfleisch, President Jefferson County medical Society
101 West Chestnut St., Louisville, KY 40202
George Nichols, III, Kentucky Insurance Commissioner
P.O. Box 517, Frankfort, KY 40602
Dr. Tom James, Chief Medical Officer
Anthem, 9901 Linn Station Road, Louisville, KY

Jim Wayne, MSW, KY Legislator, Appropriations and Revenue Committee
1280 Royal Avenue, Louisville, KY 40204
Tom Burch, Chair, KY Legislator, Health & Welfare Committee,
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Edward V. Fritzky, Chairman and CEO
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Vice Prresident for Pharmaceutical Development
Kathy Spencer, Vice President of Human Resources
Len Stevens, Sr. Vice President of Marketing
Douglas E. Williams, Sr.
Vice President and Director of Discovery Research

United States Congress
Joint Economic Committee
Summit
"Putting a Human Face on Biotechnology"

Written testimony of
Carl W. Rausch, Chairman and CEO
Biopure Corporation
September 29, 1999

Mr. Chairman and members of the Committee:

On behalf of Biopure Corporation, I want to thank you for organizing this summit and allowing me to speak here today. The title of my speech is "Oxygen Therapeutics as Alternatives to Red Blood Cells."

Biopure is a biopharmaceutical company with 175 employees located in Massachusetts, Pennsylvania and New Hampshire. Compared to some of the other participants in this conference, Biopure can best be described as a grain of sand on the beach of corporate America. However, our technology could potentially change the practice of transfusion medicine and, in doing so, affect the lives of millions of people in the United States and around the world.

Biopure's technology includes patented methods for purifying and modifying molecules, which have allowed us to develop and manufacture a new class of biopharmaceuticals called "oxygen therapeutics." These products are often called "blood substitutes" because they replace lost fluid in the bloodstream and deliver oxygen to the body's tissues and organs. However, oxygen therapeutics are pharmaceutical-quality products that overcome many of the medical, economic and logistical issues associated with blood transfusions. Because of this, they could have an enormous impact on patient outcomes and on the overall quality and cost of medical care.

Each year in the United States, more than 12 million units of blood are donated and as many as 4 million patients receive blood transfusions.[1] The General Accounting Office (GAO) has calculated that as many as 50 percent of recipients of a red blood cell transfusion would have risked immediate death had they not received this course of treatment.[2]

In the past several years, the government and the blood industry have taken major steps to improve the availability, safety and efficacy of blood transfusions. As a result, the blood supply is safer today than it has ever been. But because of the inherent nature of blood and the blood supply system, the concerns and problems associated with the administration of blood cannot be completely eliminated.

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[1] Comprehensive Report on Blood Collection and Transfusion in the United States in 1997, National Blood Data Resource Center, 1999.
[2] Blood Safety: Enhancing Safeguards Would Strengthen the Nation's Blood Supply (GAO/T-HEHS-97-143) June 5, 1997.

Each year there are regional shortages in the blood supply, which may soon become a national problem. In April, the Health and Human Services Advisory Committee on Blood Safety and Availability heard testimony projecting a shortage of up to 300,000 units of red blood cells in the United States next year.[3] The new FDA donor deferral criteria could increase this shortage to almost 550,000 units,[4] which would be an enormous stress for patients, blood collection agencies and the healthcare system in general. Biopure's oxygen therapeutic addresses availability issues because it can be stored at room temperature for two years and is compatible with all blood types.

Blood is also a product with inherent, unavoidable risks. It is licensed by the FDA, but it does not have the same standards for purity or effectiveness as a pharmaceutical product, it is classified as a biohazard,[5] and it is unique among healthcare products in being shielded from most product liability laws. In contrast, pharmaceutical manufacturers like Biopure are responsible for producing products that adhere to FDA requirements for safety, identity and strength and that meet the quality and purity characteristics they are represented to possess.

Donated blood will always carry the risk of emerging pathogens for which no tests initially exist. For example, today nearly 4 million Americans are infected with hepatitis C virus,[6] many unknowingly, and infection-related illnesses represent a significant economic burden on the healthcare system. (This is despite the screening tests instituted in the early 1990's). Biopure's oxygen therapeutics address the issue of known and unknown pathogens because they are purified through a validated and patented process to remove potential infectious agents.

A 1997 GAO report found that 8 out of every 10,000 units of blood pose a potentially serious risk to the recipient, including bacterial or viral infections, allergic reactions or reactions due to blood incompatibility.[7] A separate study recently estimated that the administration of red blood cells was associated with a 35 percent greater risk of serious bacterial infection and a 52 percent greater risk of pneumonia in the patient group studied.[8] This study calculated that serious bacterial infection increased hospitalization costs by approximately $14,000 per patient. This additional cost does not show up in the unit price of blood, but it is an example of how hidden blood-related expenses drive up the overall cost of healthcare.

In addition to availability and safety, there are also efficacy issues associated with the use of blood. For example, the ability of donated red blood cells to immediately deliver oxygen depends on how long they have been stored. The FDA's requirement for preserving red blood cells is that 75 percent of the red cells survive after 24 hours of storage, which means that 25 percent can be non-functional. In fact, red blood cells cannot be used after 35 to 42 days of storage. Biopure's oxygen therapeutics do not lose their oxygen-carrying capacity during storage, and deliver oxygen immediately upon administration.

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[3] Transcript, DHHS Advisory Committee on Blood Safety and Availability, Meeting of 29 and 30 April 1999.
[4] Guidance for Industry: Revised Precautionary Measures to Reduce the Possible Risk of Transmission of Creutzfeld-Jakob Disease (CJD) and New Variant Creutzfeld-Jakob Disease (nvCJD) by Blood and Blood Products, Food and Drug Administration, August 1999.
[5] Physicians Handbook on Blood Transfusion Therapy, American Association of Blood Banks, 1999.
[6] Hepatitis C: An Emerging Threat to Public Health, U.S. Department of Health and Human Services Fact Sheet. June 17, 1999.
[7] Blood Supply: Transfusion-Associated Risks (GAO/PEMD-97-2, Feb. 25, 1997)
[8] Carson J, Altman D, et. al. Risk of bacterial infection associated with allogeneic blood transfusion among patients undergoing repair fracture, *Transfusion*, 1999: 39: 694-700.

The impact of oxygen therapeutics upon transfusion medicine has already been proven in the field of veterinary medicine. Biopure has a product called Oxyglobin, which in 1998 became the first FDA-approved oxygen therapeutic for veterinary use. Since then the product has provided a safe and effective treatment for anemia in dogs, and has helped save the lives of hundreds of animals.

Biopure has a similar oxygen therapeutic for human use, which is called Hemopure. This product is being evaluated in a pivotal Phase III clinical trial to evaluate its ability to eliminate red blood cell use in surgery. In trials to date, Hemopure has compared favorably with red blood cells, and Biopure recently received fast-track review status for the product in South Africa. Hemopure is also being developed to treat conditions where blood is not normally transfused, such as heart attack and stroke, and to oxygenate hypoxic (or oxygen-deprived) tumors to increase their response to chemotherapy or radiation.

Biopure's oxygen therapeutics consist of purified bovine hemoglobin that has been chemically modified for increased stability in the body. These products have a two-year shelf life, are stored at room temperature, are compatible with all blood types, and are purified to remove infectious agents. They transport oxygen immediately upon administration and more readily release oxygen to tissues than blood.

Because Hemopure is easily stored and used, it has the potential to improve treatment in critical care conditions and in emergency situations in the field where immediate oxygen support is vital to prevent tissue damage or even death. In addition, it could facilitate military preparedness and medical care in remote geographic areas in the United States and around the world. For example, it is our understanding that during Desert Storm it cost our military almost $1 million dollars a day to maintain a state of blood readiness, and most of the blood on hand went unused.

It is our belief, then, that Hemopure will support the world's blood requirements by addressing blood availability issues, costs, infectious agents and the complexity of our extensive blood-banking infrastructure.

The United States has historically been the leader in the biotechnology industry. However, this leadership position will only be maintained if economic policy fosters new technologies so products like oxygen therapeutics can fulfill their potential. Since our inception in 1984, Biopure has raised $300 million to fund development of our technology and products, and like all companies we ultimately must show a return to our investors. To achieve a return and to make our innovative product available to the healthcare system, we need a true understanding of the overall costs of patient management in relation to the collection, storage, distribution and use of blood.

The government, with its payment systems for Medicare, cannot be a bystander. Reimbursement policies for blood and blood products have not significantly changed for more than twenty years. Yet, during that time, eleven new tests and multiple new procedures have been mandated to improve the safety of blood. With oxygen therapeutics now in advanced-stage clinical trials, it is time for reimbursement guidelines to consider the total costs of the blood infrastructure on our healthcare system.

In addition, the government and particularly the U.S. Patent Office should consider revising patent protection coverage so that it compensates for the lengthy FDA review process. The full economic value of patent protection is often greatly reduced because much of the patent's life span elapses prior to commercialization of a product.

At Biopure, we hope to significantly improve medical care for millions of people worldwide. With your help, we can make this a reality and ensure this technology fulfills its potential.

In closing, I would like to "put a face" on our technology by describing a recent compassionate use case. In this case, a 21-year-old woman was suffering from severe progressive immune-mediated hemolytic anemia. Essentially, her immune system was destroying her own red blood cells, which severely compromised the delivery of oxygen to her tissues and organs. At one point her red blood cell volume dropped below 4 percent, a level, which cannot sustain life and is far below the norm of 40 percent. However, this patient was administered Hemopure over the course of eight days and was subsequently stabilized until the hemolytic process could be controlled. Although she was at death's doorstep, she eventually recovered and was discharged from the hospital. This is just one compelling example of the impact our product can have when blood is not available or cannot be used.

Thank you again for inviting me to speak today. Mr. Chairman, I will be happy to respond to any questions that you or members of the Committee may have.

PREPARED STATEMENT OF RONALD W. DOLLENS, PRESIDENT AND CEO, GUIDANT CORPORATON

Putting a Human Face on High Technology
A Hearing of
Joint Economic Committee
September 29, 1999

INTRODUCTION

Mr. Chairman, Members of the Joint Economic Committee:

It is an honor to be here today to discuss ways in which technology is driving the U.S. economy and how medical technologies are enhancing the lives of millions of Americans. While this Committee addresses many issues of importance including taxes, trade, employment, and government spending, I believe that, increasingly, technology—related subjects will dominate debates about our economy. This is a positive development, and one that should make Americans confident about the future. During the past decade we have seen how technology has become not only the leading source of innovation and invention, but also the foundation for economic growth and wealth creation. American entrepreneurs, inventors and businesses dominate the field. Whether it is in the semiconductor industry, telecommunications hardware, or my own field, life-saving medical devices, the U.S. is blazing a path in technological development that our foreign competitors can only hope to imitate. Our success in bringing new technology to the market and raising standards of quality has been a remarkable economic achievement. As this testimony will argue, preserving and promoting the environment that has allowed U.S. technology to flourish must remain one of our highest public policy priorities. To do that, I believe, requires a deeper understanding of the sources of innovation and economic underpinnings of product invention.

THE ROLE OF TECHNOLOGY IN DEVELOPING MEDICAL DEVICES

My interest in the dynamic role of technology is both personal and professional. I am President and Chief Executive Officer of Guidant

Corporation a world leader in the design, development and manufacture of technology-driven products that help patients with cardiovascular illnesses lead active and productive lives. Headquartered in Indianapolis, with manufacturing facilities in Minnesota, California, Texas, Puerto Rico and Ireland, Guidant is a $2 billion company with more than 6,000 employees. Our products integrate the technologies of the computer industry as well as the most significant advances in material sciences. Patients around the world benefit from these innovations. Toward that end, over the last five years, we have invested $2.4 billion in technology.

Guidant products are used by physicians and other providers around the globe. We launched in 1985 the world's first implantable defibrillator, the device that can deliver an electric shock to stop extremely rapid and lethal heartbeats and return the heart to normal rhythm. We have also developed an array of medical instruments to treat coronary artery disease, the underlying cause of heart attacks. Our products have allowed physicians to perform such procedures as angioplasty and utilize coronary stents, the tiny mesh tubes that are implanted into an artery, providing the necessary scaffolding to hold that artery open and ensure blood flow to the heart.

But the market and technological success of our products is only part of the story. From my perspective, the most important thing I can report about my company is that Guidant and its people invent and manufacture innovative technologies that save and improve the lives of people around the world. In the past five years, six million patients' lives have been saved or improved by our technologies. We in the medical device industry, and the patients treated with our products, know how vital and satisfying the benefits of technology can be.

THE NEED FOR CAPITAL

It is important to understand that the medical technology industry faces special challenges, especially if compared with the world of information technology. While both industries rely on innovation to create better, more efficient and cost-effective products, there are important distinctions. First, the medical technology field is made up of hundreds of entrepreneurial, relatively small companies. In this respect, we are also different from most of the health care industry, where large payers or global pharmaceutical firms have long been the norm. This entrepreneurial character has helped shape the highly competitive and fluid business of medical technology innovation. New product flow has become the lifeblood of the industry, which has demanded a constant

stream of creativity. The pressure for new ideas and superior therapies is immense. For example, more than sixty percent of my company's revenue comes from products that are less than a year-old.

Such a demanding market needs a steady supply of capital to fund state of the art experimentation and research. But it is often assumed, erroneously, that medical technology, like the other technologies emerging from Silicon Valley and similar corridors of innovation, enjoys a flood of eager venture capitalists and investors who want to fund our business. Unfortunately, that is not always the case. The work of medical technology—with its long development time, intensive capital needs, and complex regulatory structure—makes our industry a less certain one for investors who otherwise have great faith in the wealth-producing capacity of technology. As an example, in 1996, there were 65 initial public offering in the medical technology industry; to date in 1999, there have been none. Uncertainties surrounding the healthcare industry in the country certainly have contributed to this situation.

PUBLIC POLICY ISSUES

I urge this committee to take note of the set of public policy issues that hang over all medical technology companies eager to develop new products to meet the demands of patients and physicians. I will briefly identify just three, recognizing that each of these merits a much longer, more comprehensive discussion.

First, there are the myriads of policies that permit a research-driven industry to thrive. These extend from the R&D tax credit, to the availability of highly skilled technical professionals. When Congress deals with these issues, it rarely thinks of them in terms of their impact on life-saving medical devices. These issues that help make the U.S. the pre-eminent research center of the world, are both critical and highly sensitive, and impact the healthcare provided to Americans.

Second, there is the much-discussed issue of how new products are reviewed, approved, and brought to market. The FDA has made significant strides in affording a more timely review of medical technologies and the legislation passed by the last Congress helped to provide the agency with direction in this area. But here again, I would stress the nexus between the federal government and the technology world. Many medical technologies are complex and the FDA must carefully review all products for safety and effectiveness. However, regulations that unnecessarily prevent or delay safe products from reaching patients in a timely manner must not be allowed.

The third public policy issue that is never far from the mind of a medical technology company executive is the question of reimbursement. A debate in underway about the parameters of Medicare coverage, and it is my hope that a new more market oriented and patient responsive system will emerge. I remind this committee that the debate must address the value and benefit of innovative technologies to America's healthcare system and in particular its patients. Medicare processes and policies can impose major hurdles—and become a barrier—to the introduction of vital technologies to the beneficiary population. The unpredictability and lack of transparency in several aspects of the Medicare program have become a disincentive to inventors and investors. Why should companies invest in the development of novel therapies if the process for determining value is unclear and not dictated by the beneficiary of the innovation? As this uncertainty increases, investment will flee.

This forum is not the place to debate the nuances of Medicare reimbursement policy or HCFA's management of the program. Indeed, HCFA deserves credit for making efforts to improve the process. But I mention this issue to illustrate how closely tied government policy can be to the success or failure of the most innovative sectors of our economy.

The problem I have seen when medical technology interacts with government policy makers is, at bottom, a problem of misunderstanding the true value of invention and the environment in which the most sophisticated level of innovation can take place. Too often, government policy ends up trying to control technology, direct its course, predict its future, or limit its influence. Those of us who have worked in the industry, seen the process of invention up close, know how fragile the flows of both financial and intellectual capital can become.

THE UNPREDICTABILITY OF INNOVATION

Like technology in general, our industry is experiencing changes at a dizzying pace. Every new innovation gives way to further refinements, more research, and still newer discoveries. Our researchers find that every search for a new medical solution yields dozens of unanticipated new discoveries that had never been envisioned in the initial research plan. Similarly, we find that the very nature of invention has a fascinating effect in the medical field. New products that have made major advances in medical care suddenly find they have uses never anticipated by the inventors. We have seen the technology incorporated in pacemakers for slow heartbeats expand to treat lethal arrythmias of the heart and the technology is currently being investigated for the treatment of congestive heart failure, the leading cause of death and hospitalization for patients over 65. We have seen catheter based approaches for heart

disease develop from diagnostic instruments to angioplasty then stents and now incorporating radiation to treat patients We are seeing these therapies expand from the heart to the brain for use in the prevention and treatment of stroke patients.

People who want to understand the development of technology need to recognize that these unanticipated consequences simply follow the natural course of technology. As the pace of discovery increases, the many-sided nature of products and innovations will come to light. Every new technological breakthrough opens new doors, creates new perspectives, and raises new questions for researchers. In the medical technology arena, that could mean saving more lives.

What we are witnessing is what Robert Fogel, a Nobel Prize winner from the University of Chicago, called "technophysio evolution," a process he believes began some 300 years ago. As mortality rates dropped, population increased, and technological breakthroughs vastly increased our food supply, igniting a revolution in "manufacturing, transportation, trade, communications, energy production, leisure-time services and medical services." What Fogel observed is that the time frame for each successive innovation was shrinking. The process of discovery itself was shortening the period we needed to wait until the next breakthrough. Thus, it took nearly five hundred years from Leonardo's drawings of a winged flying machine until the Wright Brothers successful experiment at Kitty Hawk, but just another 66 years until man could fly to the moon and land on it.

We see the same compressed timetable for innovation in the medical device field. In this century alone, medication and technology have radically transformed a profession that only a hundred years ago was largely one of experimentation. The notion of curing disease or treating illness, as opposed to merely giving comfort to the sick, is really a relatively recent phenomenon. Today the medical technology we rely on has undergone dramatic changes even in our lifetime.

Let me offer one caution: in an atmosphere of so much innovation, there is a risk of becoming complacent and assuming that innovation is something that simply occurs. But we know that is not the case. After all, what can explain the fact that the U.S. is so far ahead of every other Western country when it comes to technological breakthroughs? To anyone who has worked or spent time in Europe or Asia, the answer is readily apparent.

America has learned the secret of innovation: it remains largely a process of trial and error. The fact that our government does very little

(compared with other industrialized nations) to try to direct our research and development has allowed the private sector to excel in ways once unimaginable. It is probably safe to predict that in the years ahead, we will develop methods for certain surgeries that are quicker, less expensive, and more helpful to the patient than what we have today. We will also, in all likelihood, make significant advances in the treatment of cancer. Those are safe bets. But how those discoveries will come about is less certain. Thankfully, we do not have a Minister of Medical Technology or a Cabinet office devoted to national discoveries trying to manage that effort. No, instead we have companies, scientists, research labs, and new ventures each willing to take the risks of the trial and error method to discover something new.

Why is the spirit of trial and error so prevalent here in the United States. The simplest and most unsophisticated answer is that we have a faith in the power of the market. I know that at Guidant and in the labs of all our competitors, scientists and bio-engineers are driven by an assumption that the work they do is in demand. They assume, correctly, that a successful innovation is one that quickly finds a public willing to embrace it. We see that demand over and over again in survey data. According to a recent survey conducted by the Health Industry Manufacturers Association, 89 percent of respondents say that when they are sick, they want access to the latest medical technology; 83 percent oppose a plan that would limit the development of technology, even if it meant reducing costs; 94 percent agree that insurers should pay for a product if it is approved by the FDA.

In short, the public intuitively understands the value of new medical innovation—even if they don't yet know what it will bring. This is the real value of investing in innovation. It is an investment in the possibility of opening new frontiers. The competition to open those frontiers and meet that market demand is what makes our nation's culture of innovation and invention so dynamic.

PRESERVING A CULTURE OF INNOVATION

Keeping that culture alive should be the mandate of all policy makers who understand the connection between technology and economics. Let me conclude by mentioning just a few of the necessary preconditions to preserve and expand the culture of innovation that drives our economy:

Market-based Incentives. The private sector in the U.S. spends billions of dollars on information technology. The result is that better, less

expensive products are continuously finding their way into the market place and the competition among providers is intense. But for some reason we hesitate to apply the same rules to technology when it comes to medicine and health care. I am certain that the more our health care system is forced to operate like a market system, the more investment will come to innovative products that improve health at a lower cost. One reason that Europe and Asia trail the U.S. in medical innovation despite education systems that appear considerably better than ours is that consumers are replaced by third parties, especially government, in the process. There is no traction for innovation.

Promote Saving and Investment. Everyone on this Committee is aware of the power of the tax code to promote greater savings and investment. Ensuring that is part of our tax structure is an essential part of fostering research and discovery. It is no coincidence that the Internet sector, which is largely unregulated (and, at least as far as transactions are concerned, untaxed), has boomed while biotechnology and other advanced health sectors have had a tougher time raising capital.

Protect intellectual property. No one wants to develop a world-changing idea if it can be easily stolen. Protecting ideas is difficult in an Internet age, but worldwide patent protection is essential to technological progress. Countries that have developed strict intellectual property laws have seen how research-driven companies become more willing to invest there.

Remove Public Policy Uncertainties. The political process typically demands predictable results—an approach that fails to acknowledge the importance of the process of discovery in producing innovations. Removing the power of government to impede the process of innovation and allowing market forces, however unpredictable, to have a larger hand in determining what innovations come to market, would be a reassuring step for potential investors.

Educate the Public. We don't do nearly enough to tell the public more about the value and potential of innovation. This is a project for both the public and private sector. But if we want the market to work in favor of promoting new discoveries, than the public must have a better grasp the nature and scope of projects that are at hand. Rather than being told merely the cost of new technologies, the public should be able to see the potential savings that come from lives saved or made more productive, or the ability to dispense with older, more costly technology.

CONCLUSION

These suggestions are not intended as a policy agenda. Rather, they are guideposts for how we should begin thinking about the challenges faced in keeping the U.S. as the world leader in technological development. No one doubts that technology can be the most powerful and positive force in our lives as we enter the next century. The question we must consider is whether promoting this technology and removing obstacles to the introduction of new products, is best governed by government planners or by the discipline of the market. I believe that the marketplace has already shown how successful it can be in fostering the most sophisticated industry this country has every seen. We should study this success carefully and do everything to expand it in the years to come.

Thank you.

THE OTHER HUMAN FACE OF BIOTECHNOLOGY

Anticipating Biotechnology's Success in Developing Effective New Medications

Testimony of

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Written Version of Testimony before the Joint Economic Committee,
United States Congress

Biotech Summit: Putting a Human Face on Biotechnology

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Hart Senate Office Building

10:00 A.M.,
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Disclaimer: As always, I write and speak only for myself, not on behalf of Boston University or any of its components.

Acknowledgment: This testimony rests heavily on analyses conducted with my colleague, Deborah Socolar.

Mr. Chairman and members of the Committee—

Good afternoon.

My name is Alan Sager and I am a professor at the Boston University School of Public Health. I am honored to be here.

You have heard great things today. Many wonderful new medications may be marketed soon.

But biotech has a second human face—the face of people who cannot afford today's medications and will not be able to afford tomorrow's. I would like to address the problems of:

- making all needed medications affordable for all Americans, while
- building a durable financial foundation under drug research and delivery in the U.S.

I am absolutely convinced we can do both of these. But not by continuing business as usual.

What is the nature of the problem?

Many Americans can't afford needed prescription drugs because they lack insurance, suffer low incomes, and can't afford high American prices.

Today, 70 million Americans of all ages have no insurance for prescription drugs. Additional millions have skimpy coverage. Yet American prescription drug spending per person this year will be the world's highest. And total prescription drug spending will be close to $120 billion this year, or about ten percent of overall U.S. health spending.

Worse, people without insurance typically pay the world's highest prices for prescription drugs. That's because average American prices are highest in the world, and uninsured Americans pay prices above the average.

So it is not surprising that 17 percent of all Americans—and 42 percent of uninsured Americans—reported not filling prescriptions for financial reasons,[1]

And these are the economy's fat years, to paraphrase what Joseph told Pharaoh.

The drug cost problem will probably worsen. Drug spending in the U.S. has been rising about three times as fast as overall health care spending.

Today, some 300 new biotech medicines are reported to be in the pipeline, along with some 1,000 new drugs in the overall pharmaceutical pipeline.[2]

If too few of these medications work, we will have a lot of disappointed investors.

But what if a great number of them do work?

Then, many more patients will have to choose between their money and their lives. And still other patients will not even have this choice, because they will lack the money.

Will medical miracles be affordable for all or merely profitable for some?

If we fail to make vital drugs available to all who need them, how great will be the public fear and anger? Reasonable action today will prevent over-reaction tomorrow.

Together, we face three choices:

- Many of us could suffer and die for lack of needed medications, but that is intolerable.
- We could spend more public or private money—or both—to buy needed drugs, but that is both unaffordable and unnecessary.
- We could secure more drugs from manufacturers for the amount we already spend.

What are the causes of the problem of unaffordable medications?

To make sense these problems and to devise solutions to protect the biotechnology industry specifically, we must examine the prescription drug industry generally.

1. High U.S. drug prices make drug insurance unaffordable for many.

2. U.S. prices are high mainly because, alone in the world, our government does not protect us from the world's drug makers. This year, Americans paid at least $16.2 billion extra for drugs. This is an invisible subsidy to other rich nations—the world's least-well-targeted foreign aid.

3. The drug makers paralyze government action by claiming

- that today's prices and profits are legitimate products of a free market;
- that high U.S. prices and profits are needed to finance vital research; and
- that even moderate restraint on prices or profits will collapse the drug makers' fragile financial house of cards.

These three claims are false. The drug makers' prices and profits can't be sustained at current or hoped-for levels. During the 1990s, the nation's big drug makers' returns on equity were two and one-quarter times the average for all U.S. industries. It is unrealistic to expect that American patients can or will continue to pay prices high enough to sustain these profits.

Returns this high are not justified by legitimate market forces. Sadly, few signs of a living free market can be detected in the drug industry—outside the retail pharmacy

sector. (The evidence for this position is detailed in the July 1999 report to the U.S. House of Representatives Prescription Drug Task Force that I co-authored with my colleague, Deborah Socolar.) Without either functioning free markets or effective government action, we have only one thing—anarchy. And anarchy allows the strong to earn unwarranted profits.

That is why PhRMA, the drug makers' trade association, spreads a fog of fear—PhRMA's Fog of Fear—to try to paralyze public action and to preserve anarchy.

But the drug makers themselves sometimes pay a price for this anarchy. Some individuals connected with the biotech and prescription drug industries have worried aloud about the instability of biotech stock prices in 1993-1994 and again in recent months. They have condemned legislative efforts to contain prices or improve coverage, claiming that these efforts would impede the flow of capital to the industry. But their position amounts to condemning a symptom. As long as many Americans cannot afford needed medications, we will see repeated attempts to lower prices and improve coverage. The industry cannot wish away this simple reality. Without just and equitable access to medications, there will be no peaceful enjoyment of high drug and biotech stock prices. The challenge is to win both.

4. The United States government rejects PhRMA's claims emphatically by taking a 40 percent (or so) price discount for medications for the V.A. and military, and by taking a 15 percent (or so) price cut for the Medicaid program. This is what foreign governments have long done for all their citizens.

But unlike governments elsewhere, our government has protected itself alone. In so doing, it leaves the drug makers free to raise prices on the rest of us in order to reach their revenue targets.

Government's other main role has been in taking the risks to finance much of the basic research foundation on which the biotech industry rests, as many individuals have emphasized.

Sustained high drug prices and profits—in combination with growing numbers of patients suffering for lack of needed medications—could lead an angry future Congress to legislate harsh price and profit controls. These controls could indeed undermine needed research. Moderate action and compromise today will protect both Americans and our vital drug research community tomorrow.

What solutions are possible—to win affordable medications for all Americans?

Some drug makers' magical solution is to promise that new drugs will reduce costs of hospital and doctor care. That's easy to promise but hard to deliver, on average. Some short-run savings may be possible in some instances. While preventing or treating one disease is a blessing, doing so will inevitably expose patients to other diseases. This means that any dollar savings are one-time only.

Prudence demands that we plan against the contingency that drug breakthroughs will fuel higher spending.

It is widely recognized that efforts to expand Medicare or other coverage of vulnerable people will be very costly unless they are coupled with price or other spending restraints.

U.S. drug prices and spending per person are already the highest in the world. We already spend enough to buy all the drugs that all Americans require. Therefore, the first challenge is to protect all people without spending more money. The second challenge is to do so in ways that provide fair and adequate financing for new and effective drugs.

Several approaches could be used to meet these challenges. Here are a few:

I. ***Internationally***, negotiate a drug price peace treaty. All wealthy nations would agree to pay the same fair prices for prescription drugs, and to subsidize sick people in poor nations. Our government would have to take the lead. This is probably worth doing no matter what domestic approaches are taken.

II. ***Domestically***, I see only two alternatives. Either:

A. We could engage in years or decades of increasingly mean-spirited and fragmented fights over drug prices, profits, and coverage. Anger and threats would be the highlights. So would corporate stock price instability.

OR

B. We could sit down to negotiate a comprehensive package deal. By focusing on the two real bottom line issues—affordable medications for all plus fair returns on invested equity, this approach could short-circuit the angry trench warfare fights about the details. The package could include these eight elements:

1. Payors and drug makers negotiate fair returns on drug makers' equity. This would be the rate adequate to finance needed research and retain needed capital. Adequate overall profits would be combined with generous rewards to those who develop valuable medications.

2. In exchange, drug makers produce and distribute enough medications to fill all prescriptions written by physicians for Americans. Drug makers would find it inexpensive, on average, to provide the increased volumes (higher than today's production levels) required to protect all Americans. That is because drug makers face high fixed costs for research and setting up manufacturing plants, but extraordinarily low marginal or incremental costs to make additional amounts of most medications.

3. To make the deal real, drug prices would be set to achieve negotiated profit and total revenue targets.

4. To make medications more affordable, drug makers would be encouraged to cut wasteful marketing and advertising costs.

5. Physicians need better evidence on each drug's benefits and costs. Studies to obtain this information should be financed, compiled, and disseminated by objective parties, not by industry.

6. To encourage better use of medications, patients deserve improved information about proper drug use.

7. To protect patients, pharmacists need to be assured of payments adequate to cover the time of both patient counseling and accurate dispensing.

8. It may also be desirable to target scarce public and private research dollars down paths that are more likely to develop medications that are both effective and affordable for all.

Evidence supporting the findings and conclusions presented in this testimony is found in Alan Sager and Deborah Socolar, *Affordable Medications for Americans: Problems, Causes, and Solutions*, presented to the Prescription Drug Task Force, United States House of Representatives, 27 July 1999. It is available from www.house.gov/berry/prescriptiondrugs/. Refer to "studies of interest."

(A summary of that report is incorporated into this testimony; it appears on the following pages.)

Thank you for the opportunity to present these views. I will be happy to respond to your questions, either today or subsequently.

NOTES

[1] Karen Donelan, Robert J. Blendon, Cathy Schoen, Karen Davis, and Katherine Binns, "The Cost of Health Care System Change: Public Discontent in Five Nations," *Health Affairs*, Vol. 18, No. 3 (May – June 1999), pp. 206-216, exhibit 6.

[2] Neil Munro, "Technology: Frontier Ethics," *National Journal*, 4 June 99

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Affordable Medications for Americans

(AMA)

Problem, Causes, and Solutions

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For presentation to the Prescription Drug Task Force,
United States House of Representatives

Tuesday 27 July 1999
2:00 P.M.
Capitol Room EF100

Disclaimer: As always, we write and speak only for ourselves, not on behalf of Boston University or any of its components.

THIS REPORT IS DEDICATED TO THE MEMORY OF SENATOR ESTES KEFAUVER OF TENNESSEE.

AFFORDABLE MEDICATIONS FOR AMERICANS (AMA)

Problem, Causes, and Solutions

Alan Sager, Ph.D. and Deborah Socolar, M.P.H.

SUMMARY

INTRODUCTION

By all reasonable international standards, current spending on prescription drugs in the United States is enough to buy all the medications that Americans need. But today's high spending does not suffice, owing to high prices and inadequate coverage. Our nation and its people therefore face three choices:

- more people suffering and dying for lack of needed drugs—but that is unacceptable;
- spending more to give more people better drug coverage—but that is unaffordable;
- securing more drugs from manufacturers for dollars we already spend.

This report offers strategies for securing access to vital drugs without higher cost.

First, we need **relief**, gaining lower prices and greater volumes of medications so that today's dollars buy the drugs we now need. This will win time to design and test **reforms** in drug development, pricing, prescribing, and use, to make all effective drugs durably affordable for the future.

PROBLEM: MANY AMERICANS CANNOT AFFORD NEEDED MEDICATIONS

Lack of coverage

- **Roughly 70 million Americans of all ages—about 1 in 4—have no prescription drug coverage**, the Access and Affordability Monitoring Project (AAMP) estimates. This number rises as drug prices rise. Under-insurance for medications is also rising.

High spending

- U.S. retail prescription drug spending is predicted to rise to $103 billion in 1999 and $143 billion by 2002. Total prescription drug costs will be about $120 billion this year.
- **Retail prescription drugs will consume 8.4% of U.S. health spending in 1999,** up from 7.2% in 1997. Total prescription drug costs will be 9.8% of health spending.
- Per person U.S. prescription drug spending is now about $377 retail and $425 total. **At prevailing rates of increase, prescription drug spending per person in the U.S. will be the world's highest either this year or very soon.**
- Prescription drug costs are rising about three times as fast as overall health costs.

High prices

- For decades, the world's prescription drug makers have charged Americans more for the same pills (often from the same factory) than they charge in any other country.
- Yet we buy 1/3 of the world's prescription drugs, which should let us win low prices.

- In the early 1990s, drug makers charged Americans 32% more than Canadians for the same drugs. That gap suggests **Americans would save roughly $16.2 billion yearly if we paid the wholesale prices paid by Canadians.** By at least that sum, we subsidize other wealthy nations who do not pay their fair share of drug costs.
- Recently, U.S. prices rose as Canada's prices fell. Other wealthy nations have long paid even less. So the **international cost-shift** onto Americans is probably growing.
- **Even the $16.2 billion minimum estimate of the U.S. drug subsidy to wealthy nations is more than double the $7.6 billion in bilateral foreign aid appropriated by Congress in 1999 to aid vulnerable or starving nations.**
- The drug industry contradicts itself, saying that price controls abroad both work and don't work, denouncing controls as ineffective but also as constraining their revenue.
- **And U.S. drug prices are rising again—2.4 times as fast as the overall Consumer Price Index** (April 1998-99), and at an annual rate of 6.1% in early 1999.
- Because discounts for some Americans now mean a **domestic cost-shift** to others, people who lack coverage—many poor, sick citizens—pay the highest prices of all.

CAUSES: WHY ARE SO MANY PEOPLE UNABLE TO AFFORD NEEDED MEDICATIONS?

Government inaction

- U.S. prescription drug prices are high mainly because our government fails to protect us against drug companies as other governments protect their citizens.
- Drug makers' acceptance of price and profit caps in other nations is surely greater because they can freely raise prices for Americans, the world's shock absorber.

Research?

- Since all drug manufacturers charge more in the U.S. and appear to earn a disproportionate share of their profits here, how can American drug makers claim that high profits enable them, uniquely, to conduct more breakthrough research?
- **Pharmaceutical research in the U.S. was 0.97% of health spending in 1990-94, but averaged 1.53% for the U.K., Japan, France, Italy, Germany, and Canada.**
- Globalization makes it hard to judge industry attribution of research to single nations.
- Better drugs may cut short-run health costs, but long-run savings are far from sure. And we must not revere potential future cures while we deny people existing cures.

Profits

- In 1998, pharmaceuticals were **the most profitable industry** in return on equity, on revenue, and on assets. Remarkably, return on equity reached 39.4 percent in 1998.
- Indeed, drug makers had strikingly high profits for 7 decades, from the 1930s to '90s.
- Drug making was the most durably profitable U.S. industry over the past 3 decades. **Its median return on equity was 1.5 times the all-industry average in the 1970s and '80s, improving to 2.3 times the average in the 1990s.**

- The high drug industry profits year after year raise the question: **Where is the risk?**
- **Drug companies must be asked to specify and negotiate the profit level they need to attract and retain sufficient capital** to operate successfully in the public interest by developing and producing innovative, effective, safe and affordable drugs.
- Large drug companies rely on others to do much of the riskiest, early-stage research. Public funding for bio-medical research appears far greater in the U.S. than abroad.
- **Profits exceed research costs at the top 10 U.S. drug firms**, Public Citizen found. Merck and Pfizer used an average of only 11% of revenue for R&D in 1997, with 29% of revenue for marketing and administration and 19% for profit, the AAMP finds.

Lack of competition

- Patents grant monopolies to drug makers to spur innovation. But that gives drug makers much power over prices. **Other nations offset this pricing power with government action** to make drugs affordable and achieve free-market price levels.
- Laws that bar parallel imports and limit access to generics also reduce competition.

Manufacturers' pricing strategies

- Diverse evidence makes clear that drug prices are not set in direct relation to R&D costs. So it is wrong to claim that high prices are required to finance drug research.

Income inequality

- The **domestic cost shift** means drug makers and retailers extract higher prices from Americans who pay out-of-pocket. Yet these Americans disproportionately suffer low incomes. **It is hard to imagine a less just arrangement.**
- The problem is magnified since U.S. incomes are the industrial world's least equal.

Underlying reasons for the failure to make needed prescription drugs affordable

- These include governments' small role in paying for drugs; payors' focus on cost-*shifting*; drug makers' political power and campaign contributions; and focus on steps that cannot yield adequate savings (such as using generics and squeezing retailers).
- Drug makers and the Pharmaceutical Research and Manufacturers of America (PhRMA) say that if Americans do not pay high prices to "bear the world's research burden," many new drugs will not be developed. But:
  - **Lower U.S. prices need not mean lower revenue and profit for drug makers if they cut costs, boost volume, or raise prices in other wealthy nations.** (Charging poor nations more gains nothing, as they generally cannot pay more.)
  - Drug makers all face the same pricing policies worldwide. A more plausible engine of U.S. innovation is public funding for biomedical research through NIH.
  - We need not choose between extraordinary profits and high research spending on one hand and no profits and no research on the other. PhRMA ignores **reasonable middle grounds**, as the drug makers' first duty is to stockholders.
- The world's drug makers use market rhetoric but harvest huge profits from the U.S. because there is no free market for prescription drugs, either here or internationally.

- **With neither a functioning free market or effective government intervention, anarchy ensues.** Those with power—drug makers with monopoly or oligopoly power—can raise prices and profits above free market levels. This better explains high drug industry profits than do claims of risky investment or innovative research.

WHAT PRIVATE, FEDERAL, AND STATE SOLUTIONS ARE POSSIBLE?

Recommended solutions

- **U.S. policy on prescription drug financing should aim to:**
  - **assure that all Americans can afford needed prescription drugs,**
  - **do so without increasing public or private spending, and**
  - **maintain adequate profits so that the industry can develop new drugs.**

- Plans to **discount** prices for groups of patients would make drugs more affordable, while other plans would use **rebates** to secure more drugs without spending more.

Inventory and assessment of possible solutions

- **This report briefly analyzes 19 public and private methods of winning lower drug prices and 7 public and private methods of winning better drug coverage.**

- **Massachusetts, Vermont, and other states are considering pooling statewide buying power to negotiate discounts with drug makers.** The plan would make drugs more affordable to all, end the domestic cost-shift, and reduce the international cost-shift. Higher sales would offset some revenue loss for drug makers.

- AAMP uses estimates that 10% **lower drug prices would raise demand** 3.3%, thus offsetting some revenue loss for drug makers. Merrill Lynch projects more rise in demand, suggesting that a 40% discount for Medicare patients (as in Rep. Allen's bill, H.R. 664) would yield from a 3% drop to a small rise in drug maker revenue.

- Under the just-noted Massachusetts and Vermont bills, the states would negotiate with drug makers to win rebates as well, to help expand drug coverage. Most of the rebated sums would return to the drug makers to buy more pills. Marginal costs of making more pills are very low (estimated at 5% of the retail price). So **such rebates would cost manufacturers surprisingly little.**

- Such discounts and rebates combined would mean an **estimated gain to patients at least twice as great as the proposals' cost to drug makers.**

- Alternatively, the federal or state governments could negotiate with drug makers for **in-kind donations**. Companies could offer credit equal to perhaps 25% of their sales in a jurisdiction; costs of drugs for patients in need would be debited against the letter of credit. Dispensing costs could be paid by patients, public funds, or drug makers.

- **Providing 25% more medications in the U.S. would cost the world's drug companies only about $1.25 billion.** If the 12 largest U.S. drug makers had borne this entire burden in 1998, their combined profits would have fallen by $1.25 billion—the cost of making the added drugs—leaving them securely in 1st place among U.S. industries. **Americans would have gained additional medications with a retail price of $25 billion—without higher taxes, premiums, or out-of-pocket costs.**

- The cost of making more drugs need not erode industry profits at all. **Drug makers can cut waste**, especially in advertising, public relations, lobbying, and executive pay. (In 1997, 10 drug company CEOs alone received $229 million.) And drug makers, with U.S. government help, should act to end today's international cost shift.

- **Simple government action can cut drug prices, making needed drugs available for all Americans without great public cost and at little cost to drug makers.**

Elements of durable reform

- Steps are also needed to assure better use of prescription drugs and make them durably affordable. Durable reform requires:
  (1) **negotiating an international treaty that cuts U.S. drug prices** and gets other wealthy nations to start paying their fair share of drug research costs and profits;
  (2) assuring fair and adequate returns on invested equity;
  (3) better evidence on each existing drug's benefits and costs, so physicians can prescribe more reasonably;
  (4) better education for physicians, financed and disseminated by objective parties;
  (5) better patient education about proper drug use; and
  (6) targeting research to develop medications that are affordable and effective.

- **Fair levels of profit should be negotiated between payors and manufacturers**—levels adequate to retain and attract the capital to finance necessary research.

- The broad outlines of fair international drug pricing are clear:
  - **Wealthy nations all should pay the same prices for drugs, to finance most of drug makers' legitimate research, manufacturing, and other costs.**
  - Moderate-income nations should pay prices that cover the incremental costs of the medications they use, plus a small contribution toward fixed costs.
  - **Poor nations should obtain needed drugs at no more than symbolic prices.**

- Now is the time to begin changing course. Throwing more money into business as usual will make it harder to cure drug companies of their addiction to high prices.

CONCLUSIONS

- Prices must fall so that Americans pay only our fair share of the cost of profits and drug research. **In exchange for the large sums we spend on medications, all drug makers must agree to provide needed volumes of drugs to all Americans.**

- Winning affordable and effective medications for all Americans requires both federal and state efforts. State-level action could be effective in part because **states have surprising purchasing power: California's health spending is greater than France's, for example, and health spending in Texas exceeds Canada's.**

- **The future trajectory of prescription drug policy and financing should be planned cooperatively among all stakeholders.**

- Since U.S. prescription drug spending per person will soon be the world's highest, **winning affordable drugs for all should be the easiest job facing our nation.**