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S. HRG. 109–191

EXPLORING THE PROMISE OF EMBRYONIC STEM CELL RESEARCH

HEARING

BEFORE THE

SPECIAL COMMITTEE ON AGING
UNITED STATES SENATE

ONE HUNDRED NINTH CONGRESS

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EXPLORING THE PROMISE OF EMBRYONIC STEM CELL RESEARCH

WEDNESDAY, JUNE 8, 2005

U.S. SENATE,
SPECIAL COMMITTEE ON AGING,
Washington, DC.

The committee met, pursuant to notice, at 2:05 p.m., in room SD-G50, Dirksen Senate Office Building, Hon. Gordon H. Smith (chairman of the committee) presiding.

Present: Senators Smith, Collins, Kohl, Wyden, Lincoln, Carper, and Clinton.

OPENING STATEMENT OF SENATOR GORDON H. SMITH, CHAIRMAN

The CHAIRMAN. Good afternoon, ladies and gentlemen. I would like to welcome you all to what is sure to be an interesting and highly informative hearing of the Senate Special Committee on Aging: exploring the promise of embryonic stem cell research.

Stem cell research is one of today's most exciting and rapidly advancing fields in modern medicine. It holds the key to potentially unlocking the secrets of diseases that have mystified scientists for years, namely, Alzheimer's, Parkinson's, diabetes, cardiovascular disease, and more. This becomes particularly important as our Nation's population ages and more and more of our seniors become afflicted with ailments that take a great toll on the families of loved ones, as well as on the individuals themselves.

Scientists are just beginning to scratch the surface of the knowledge and benefits that can be reaped through a thorough understanding of stem cells and their potential for creating breakthroughs in therapeutic disease treatment.

This hearing will examine some of the most important progress being made in the area of embryonic stem cell research, the need for new stem cell lines, and the reasons these additional lines should receive Federal support.

Among the elderly, diseases such as diabetes, Alzheimer's, Parkinson's, and cardiovascular ailments are among the most prevalent and the most costly to the Federal budget and family budgets. Together, the estimated annual direct and indirect costs of caring for patients with these diseases is $650 billion. Alzheimer's alone is a disease that afflicts one in ten Americans over the age of 65, and nearly half of all persons over the age of 85. As baby boomers begin to age, the prevalence of Alzheimer's is expected to grow by 350 percent, from 4 million Americans today to an estimated 14

million by 2050, which will make it one of the most costly diseases in our society.

The impact on Medicaid and Medicare and our private health care system will be enormous. However, if we can find a way to delay the onset of Alzheimer's by just five years, we could reduce the number of cases and spending on the disease by more than half, by more than 50 percent. In addition, diabetes, neurodegenerative, and cardiovascular diseases also happen to be areas for which stem cell therapy seems most promising. Although a limited number of human embryonic stem lines are eligible for use in federally funded research, many scientists are concerned about the usefulness of these lines. While some claim a total of 78 embryonic stem cell lines are listed, in reality only 22 lines are currently available to researchers. Furthermore, scientists have serious concerns about the quality, longevity, and availability of these existing lines because they were grown in culture dishes coated with mouse cells which have contaminated them. These mouse cells helped them to generate, but they also eventually create contamination.

At the time this method was created, the mouse cells were necessary. However, in a dramatic new achievement earlier this year, scientists were successfully able to maintain stem cell lines without using animal feeder cells. In order to allow researchers the opportunity to fully explore the possibilities and promise of stem cells, we must ensure they have expanded access to these new uncontaminated stem cell lines.

The Stem Cell Research Enhancement Act, S. 471, introduced by Senators Specter, Hatch, Harkin, Kennedy, Feinstein and myself, and H.R. 810, introduced by Congressman Castle and Congresswoman DeGette would allow research to receive Federal funding for the study of embryonic stem cells derived from excess embryos created for fertility treatments and willingly donated by patients. Last month, H.R. 810 passed the House on May 24, 2005, by a vote of 238–194, and it is now time for the Senate to act.

I am also currently working on legislation titled "The Stem Cell Research Investment Act," which would buildupon S. 471 to promote cutting-edge research to fight devastating chronic diseases and health conditions. Modeled after California's recently passed Proposition 71, the bill encourages States to issue up to $30 billion in zero-interest bonds to fund their own stem cell research initiatives and provides bondholders a Federal tax credit in light of interest payments. As with S. 471, such funding could only be used for embryonic stem cell research that uses embryos that are bound for destruction from fertility clinics donated by patients.

In the field of medicine, there is no such thing as a Republican science or a Democratic science. There is just science. New advances in technology have allowed us to understand the nature of the human body like never before, and with it the ability to prolong life and to cure disease. Responsible research grounded in the roots of scientific principles and conducted with the ultimate goal of saving life must be allowed to flourish. We owe a moral obligation to the sufferers of these debilitating diseases and their loved ones to provide our best and brightest scientists with the tools they need

to undertake their quest in a safe and ethical environment free from unnecessary Government barriers but with moral parameters.

I eagerly await the testimony of our experts who understand the financial, emotional, and physical costs of these diseases and who are among the leaders in cutting-edge research that is being done in this field. It is my hope that by the end of today's hearing we will have a greater understanding of embryonic stem cell research and a deeper appreciation of the incredible potential of this exciting branch of medicine.

It is my privilege now to turn to my colleague and friend, Senator Herb Kohl, the ranking member of this committee. Senator Kohl.

OPENING STATEMENT OF SENATOR HERBERT H. KOHL

Senator KOHL. We thank you, Mr. Chairman, for holding this very timely hearing today, and we welcome our witnesses who will be testifying today. Every family in America has experienced the tragedy of watching a loved one suffer through a deadly or debilitating illness. Diseases like Parkinson's and Alzheimer's take a terrible toll on families' lives and livelihoods. While we have made great strides in biomedical research in recent years, we still do not have all the keys to unlock the secrets of disease, and that is why the potential of embryonic stem cells is so exciting. Embryonic stem cells have the ability to develop into virtually any cell type in the human body. Scientists tell us that harnessing the power of these cells could one day lead to new treatments and maybe even cures for a number of diseases that afflict American families.

Important research is being done every day on stem cells, and I am proud that some of this research is being done at the University of Wisconsin in Madison, which was the first to isolate human embryonic stem cells. So we are pleased to have Dr. Su-Chun Zhang from the university's Waisman Center here today to testify on the ground-breaking work that he and his colleagues are doing.

We all understand, of course, that this research is not without controversy. We respect the concerns that some people have about the use of embryonic stem cells in research, and we agree that we must closely monitor this research to ensure that it is done ethically. However, scientists and disease advocates are warning us that the current limits on Federal funding for stem cell research are seriously inhibiting our potential to find new cures. Without expanded Federal support, we do risk slowing down the tremendous progress that could be made to alleviate human suffering. It would not be right for the Federal Government to turn it back on the discoveries that expanding stem cell research promises.

Now more than ever it is important to grasp this opportunity in an ethical manner by making sure that potentially life-saving research does keep moving forward. So we look forward to hearing from our expert panel today and hearing their recommendations. Again, we all thank you, Mr. Chairman, for holding this hearing.

The CHAIRMAN. Thank you, Senator Kohl.

We are joined by two of our colleagues, Senator Collins of Maine and my colleague from Oregon, Senator Wyden. So, Senator Collins, you are next, and then Senator Wyden.

OPENING STATEMENT OF SENATOR SUSAN M. COLLINS

Senator COLLINS. Thank you, Mr. Chairman.

I want to begin by commending you for holding a hearing on an issue of profound importance to this country. The title for this hearing that you have chosen is highly appropriate. Embryonic stem cell research does indeed hold tremendous promise to treat and possibly even cure a vast array of devastating diseases and conditions. It is a promise that must be explored. From Alzheimer's to Parkinson's and ALS, to cardiovascular disease, diabetes, and cancer, this research offers great hope to our seniors and their families.

For some seniors, these devastating diseases can turn their last years of life into a time of suffering and misery. The potential of this research to relieve this suffering and misery is far too great for us not to explore it. But this research will not just benefit our seniors. As the founder and the co-Chair of the Senate Diabetes Caucus, I am particularly excited about the promise that stem cell research holds for a cure for juvenile diabetes. This disease has had such a profound effect on more than 1 million American children and their families. It condemns far too many of them to a future of heart disease, stroke, kidney failure, blindness, and amputation.

We simply cannot ignore the potential that stem cell therapy holds for these young people, and indeed in two weeks' time, I will be chairing the biennial Children's Congress where children with diabetes come from every State in the Nation. It is so important because they put a human face on this debate.

Of course, we know that this research offers the possibility of recovery or at least better treatment to people of all ages who have suffered devastating spinal cord injuries. We are now engaged in a great national debate over whether this vital research can proceed at a vigorous pace given the administration's decision to make Federal funding available only for that research using embryonic stem cell lines that existed before August 2001. As the chairman indicated, many scientists contend that the existing lines are contaminated with mouse cells and that severely compromises their potential therapeutic values for use in humans. I look forward to hearing the testimony from our experts today on that crucial issue.

This debate is often portrayed as a choice between scientific advancement on the one hand and medical ethics on the other. I believe that that is a false choice. I believe that we can advance this vital research and at the same time maintain the highest ethical standards.

Last month, the House of Representatives took an important step in resolving this debate. The legislation passed in the House would expand the current restriction on Federal funding for embryonic stem cell research to the more than 400,000 cells unused in fertility clinics that would be made available by willing donors. I would point out that these are cells that otherwise would be discarded. Isn't it far better to put them to work to help advance this research?

The House legislation is the result of bipartisan cooperation and compromise. As the chairman indicated, there is similar legislation in the Senate which we have cosponsored along with our colleague Senator Specter. This legislation also makes available to research-

ers that vast number of unused cells, and it respects the ethical considerations that are such an important part of this research.

I believe that the ethical thing to do is to move forward with this research, and I want to again thank the chairman for his leadership in making this research possible.

The CHAIRMAN. Thank you, Senator Collins. I think the issue is often cast as a Republican or a Democrat issue. Actually, I think we have proven that this is a human issue.

Senator Wyden.

OPENING STATEMENT OF SENATOR RON WYDEN

Senator WYDEN. Well, thank you, Mr. Chairman. I would like to characterize it as an Oregon issue as well because you have done a tremendous job in terms of leading the committee. I also want to say how much I appreciate Chris Dudley being here. Chris Dudley is involved in just about every good cause in our home State, and we are just thrilled to have him and appreciate all he has done.

Mr. Chairman, I am going to have to be in the Intelligence Committee and I think a couple of other committees in the next 10 minutes, and I am not going to be able to stay. I just wanted to come by and make a point or two on behalf of the leadership that you are showing.

The principal thing that I wanted to touch on is that the opponents of embryonic stem cell research seem to be arguing now that the reason this research should not go forward is that there are not enough medical discoveries or cures using embryonic stem cells. I guess if you follow that kind of logic, some of the opponents of embryonic stem cells would have criticized the Wright brothers for not launching a moon flight when they took their very first flight.

The fact of the matter is you have got to let science advance. Scientific research takes time and money to develop treatment and cures, and the fact is that embryonic stem cell research has been hamstrung by limiting the cell lines and not giving Federal funds to non-approved stem cell lines.

So I think we also ought to note that there is progress being made in adult stem cells. It is important that that work continue. But in the Commerce Committee, we examined some of the limits on what adult stem cells could do, and it is clear that the country ought to go farther.

Now, I would be shocked, I guess, to say that politics is involved. We all remember that line from "Casablanca." But suffice it to say nobody can be naive. There is politics in this debate, and I would just hope that people would pause a little bit because in an age when there is not a lot of bipartisanship, when there are not enough legislators who are doing what Chairman Smith is doing, what Senator Collins is doing, this is a piece of legislation where there is true bipartisan support. I think that that is the case because this country wants science to be science. This country does not want science to be seen through a political prism. So what you have are Senators who want to pursue science in that kind of approach.

I commend you, Mr. Chairman, and I apologize to our friend, Chris Dudley, for not being able to stay throughout the afternoon.

But I know that the Oregon juggernaut, with the chairman and Chris Dudley, is going to prosper in terms of advancing this cause, and I thank you both for all you are doing, and my colleagues as well.

Thank you.

The CHAIRMAN. Thank you, Ron.

We have been joined by Senator Carper of Delaware, so we welcome your statement if you have one.

OPENING STATEMENT OF SENATOR THOMAS R. CARPER

Senator CARPER. I am not from Oregon. [Laughter.]

But I have been to Oregon, and I must say I liked it a lot. In fact, I thought about going to graduate school there when I got out of the Navy in California, and they told me they would never let me be a Senator, Governor, or any of that stuff. So I decided to find a smaller State. But we are glad that you are here and look forward to hearing from you.

I have a statement I would like to enter for the record, but let me just make a brief personal comment if I may.

My mom passed away a couple of months ago. She had Alzheimer's disease and congestive heart failure and arthritis and all kinds of maladies. She lived to be about 82, a full life, but the last 6 years she was in a wonderful facility in Ashland, KY, called Woodland Oaks, where they took great care of her. She lived close to my sister and closer to my mother's sister. But she had Alzheimer's disease, and it sucked away her vitality and a special part of her life in her later years. Her mom had had Alzheimer's disease. Her grandmother had Alzheimer's disease as well.

As we focus on the issues of stem cell research and try to apply the research as we develop it, I think of my mother. I think of my mother's father, who had Parkinson's disease. I was in born in West Virginia, and my grandfather was a butcher. He was kind of an amazing guy, and he lived to be about 85 years old. But he would drive to work at Patton's Market, which is one exit off the West Virginia Turnpike from Robert C. Byrd Drive. My grandfather would drive up to Harper Road, and his hands were shaking just like this. I always remember when I was a little boy going to visit him and wondering how will he ever go into the butcher shop and not cutoff a finger or a thumb. He would get in the butcher shop and he was like a rock for the rest of the day until it came time to go home. Then he would have what we would call palsy or the trembles.

I remember my grandfather who made the best of the hand that he was dealt and stayed with it for a long, long time. Not everybody, including a fellow I had a chance to spend some time with at lunch on Monday in Philadelphia, not everybody is as lucky as my grandfather to be able to go that long and that hard.

Everybody here, probably everybody on the panel, I guess everybody in the room, can talk about their own mom or dad or their aunt or uncle or their grandparent and how their lives, their quality of life has been diminished because of their battle with a disease that I think can be tamed, can be cured, or at least delayed through the kind of research that would be enhanced by the legislation that my Congressman, Mike Castle, has introduced and

championed in the House. I am pleased to cosponsor it here in the U.S. Senate.

So I would say, Mr. Chairman, thank you for pulling this all together and for letting a couple of guys who are not from Oregon say a few words and to say hello and good work. Thanks. Welcome, Chris.

The CHAIRMAN. Thank you, Senator Carper, and we will include your full statement in the record as well.

[The prepared statement of Senator Carper follows:]

PREPARED STATEMENT OF SENATOR THOMAS CARPER

Mr. Chairman, thank you for holding this hearing on the potential of stem cell research, a topic that is very important to many people in Wisconsin. I commend you and the Ranking Member for providing leadership on this issue.

I am pleased that the Aging Committee is providing a forum for some of the country's leading researchers to speak about the progress they are making with embryonic stem cell research, and the limitations that currently impede their ability to further advance this research.

I am especially proud that Dr. Su-Chun Zhang of the Waisman Center at the University of Wisconsin is here to describe firsthand his experience in using embryonic stem cells to further understand degenerative diseases and medical conditions such as Parkinson's, ALS, MS and spinal cord injuries. I thank Dr. Zhang for his contributions to these efforts, and for agreeing to take time from his valuable work to appear before this Committee.

Dr. James Thomson at the University of Wisconsin first broke ground in this amazing research, and with the help of talented researchers such as Dr. Zhang, Wisconsin continues to be a proud leader in this field.

Embryonic stem cell research holds the potential for better understanding, and possibly developing cures and treatments for, many fatal and debilitating diseases and medical conditions. That is why I have cosponsored S. 471, the Stem Cell Research Enhancement Act of 2005 introduced by Senators Specter and Harkin. This bill would help our nation's researchers unlock that potential by increasing the quantity and quality of stem cells lines available for research.

There is much work that needs to be done to further understand the role that embryonic stem cells can play in providing answers to some of the most troubling medical diseases and conditions that affect so many Americans. Limiting our ability to find these answers when researchers are only starting to make headway would be a huge step backwards for the many Americans who could benefit from this groundbreaking research.

Embryonic stem cell research could very well be the gateway to finding treatments or cures for diabetes, heart disease, ALS, spinal cord injuries and other medical conditions that millions of Americans currently suffer from. I will continue to support this incredibly important science which would expand our research horizons, and bring hope to so many people.

I was very pleased to see stem cell legislation pass the House, and I am proud to be part of the bipartisan effort to expand federal funding for embryonic stem cell research in the Senate. It is my hope that the Majority Leader will soon bring this bill to the Senate floor for a vote, and that the Senate will overwhelmingly pass this legislation.

The CHAIRMAN. It is not a requirement to be from Oregon to be heard today, but our first witness is from Oregon, and he will be joined by a second panel of very distinguished scientists and physicians who will hopefully illuminate us on the promise that embryonic stem cell research may offer.

To introduce Chris Dudley, I think many of our audience would recognize and remember him from his stellar career as an NBA center. He was with the Portland Trailblazers through some of their brightest days, and after this last season, they could use your help again, Chris. But as the owner of the Milwaukee Bucks is right here, he probably does not want to see you return.

Chris Dudley understands firsthand the enormous financial, physical, and emotional costs of a particular disease, that is, diabetes. He was diagnosed with that affliction at age 16, and yet he nevertheless went on to achieve remarkable success in athletics.

Chris' desire for every child to succeed regardless of their economic, education, or health liabilities inspired him to create the Dudley Foundation in 1994, which includes the Chris Dudley Basketball Camp for Kids with Diabetes in Vernonia, OR. His personal story will underscore the importance of exploring all scientific avenues, including human embryonic stem cell research, to prevent and find a cure for diabetes.

Chris, thank you for coming this long way to participate in this hearing.

STATEMENT OF CHRIS DUDLEY, DIABETES ADVOCATE, AND FORMER CENTER, PORTLAND TRAILBLAZERS, PORTLAND, OR

Mr. DUDLEY. Thank you. Good afternoon, Senator Smith and members of the committee. Thank you for the invitation to appear before your committee today to tell you about how living with juvenile diabetes has affected my life and the lives of so many children that I have met over the years through my foundation and at my basketball camp.

My name is Chris Dudley, and I played professional basketball for 16 years with Cleveland, New Jersey, the Portland Trailblazers, and the New York Knicks. I am the proud father of three wonderful children ages 6, 5, and 3 and husband to wife Christine. I also have juvenile diabetes.

I was diagnosed at the age of 16. I had the classic symptoms of excessive thirst and having to go to the bathroom constantly. My uncle also has diabetes, so my dad recognized the symptoms, luckily, and brought me home a test kit, and it showed that my blood sugar was extremely high. We immediately went to the hospital, and I was diagnosed with diabetes.

When I first heard the news, I was devastated. I did not really know enough about the disease, and I was terrified that I would no longer be able to play basketball. In fact, my dad tells the story that the first question I asked was would I still be able to play basketball.

I was fortunate that the doctors and nurses said that I would be able to continue to play if I was careful about monitoring my blood sugar, and this to me was a tremendous relief. I thought if I can keep playing, I can go forward.

This is not always the case. Many times kids with juvenile diabetes are not encouraged to keep playing sports because of fears of what can happen, especially from low blood sugars. I was also fortunate to have a supportive family that encouraged me to continue to play basketball and not let diabetes stop me from doing what I loved.

After my diagnosis, I really looked up to people like Bobby Clarke, Hall of Fame hockey player for the Philadelphia Flyers, who had diabetes and also of a triathlete—I was then living in San Diego—who had diabetes. I felt at that time it was a tremendous help for me to realize that if Bobby Clarke can play Hall of Fame

hockey and this other person can run a triathlete, then I can play JV basketball.

Ever since that time, I have been an outspoken advocate for encouraging kids with diabetes to pursue their passions—whether it be sports or other activities—provided that they take care of their diabetes. That being said, that provision is a hard one. Diabetes is such a hard disease because you have to stay on top of it every hour of every day, or you can face serious complications. It is a disease that is 24/7 and takes no breaks. Diabetes never stopped me from playing basketball, but by no means was it easy. There were many times when the disease did hinder my performance.

When I was playing in the NBA, I would have to test my blood sugar 14 times on a game day and take multiple insulin shots. When you are preparing to play in front of 20,000 people, you want your sugar—blood sugar level—to be as close to the ideal as possible. This is very difficult to do, and some days no matter how hard you try, it is never perfect. It took a lot of practice and monitoring, but I was able to play to the best of my abilities regardless of my diabetes. I was fortunate that my teammates were also supportive. It was through my teammates that I realized how widespread this disease really is. I was amazed at how many teammates would have a father, brother, sister, uncle, grandfather who had some connection to diabetes. I was fortunate that I always felt there was a great understanding and appreciation of what I had to go through every day just to be able to play basketball.

I also had my battles with diabetes. In college, I was in a car accident. After working out, my blood sugar dropped dangerously low and I ran into a parked car. That is one of the dangers of what can happen when you have diabetes, and with working out your blood sugars can drop dramatically. I have had diabetes for 24 years, and I have had that constant worry about the long-term risks and what the disease is doing to my body.

Now that I am retired from the NBA, my passion is my family—my three children—and advocating on behalf of research to get us to a cure for juvenile diabetes as soon as possible and enabling kids who already have diabetes to be able to pursue their dreams. I started the Dudley Foundation in 1994 and the Chris Dudley Basketball Camp for Kids with Diabetes in Vernonia, OR, in 1994. At the camp, I see firsthand what these kids—some of them very young—have to go through every day. Some struggle much more than others, not because they are being lazy about monitoring their blood sugars, but because it is just more difficult for some kids to keep their sugar levels in range, as hard as they try. At that age, you have hormones, you have stress, colds. Anything can throw your blood sugars out of whack.

When I talk to kids with diabetes and work with them at camp, I walk a fine line. I want to show them that the diabetes does not have to stop you from doing whatever it is that you want to do; but, on the other hand, I know that it's not easy for them and that they will never get a day off from this disease. It is not easy for their parents either. Parents of the kids who come to my camp tell me that it is the only week throughout the entire year that they can sleep through the night without having to get up to check on their

kids and check their blood sugar levels. To me that is just amazing as parent.

I worry every day that one of my kids will be diagnosed with juvenile diabetes. Even though I have been very blessed in my life and have been able to achieve some great things even with diabetes, this is not the life I want for my children. I am missing my 6-year-old son's kindergarten graduation to be here today, but I explained to him that being in Washington was my opportunity to help people understand why a cure for diabetes is so important. I want this cure for the children who come to my camp, my children and your children.

Last August, I received an award, a Freedom Corps Award, from President Bush for my camp and the foundation. I had the opportunity to travel with President Bush and Leery Bush in Portland that day. I told them what it was like to live with juvenile diabetes and the struggles the kids who come to my camp face every day. I also told them that even though I share and empathize with some of their same concerns, I believe that there is an ethical compromise that will allow the tremendous potential of stem cell research to flourish. Research is the only avenue to the cures and therapies for diabetes and many other diseases, and we should pursue this promising research aggressively within an appropriate ethical framework.

I want to be able to tell the children I see—and I see a lot of them—with diabetes and tell them with a straight face that in this great country we are doing everything possible to find a cure and that help is on the way.

Mr. Chairman, thank you again for this opportunity. It has been an honor to appear before you today.

The CHAIRMAN. Chris, just as an Oregonian, I just have to tell you how proud I am of what you do and your basketball camp. Obviously, these kids that you take into the camp—do they all have childhood diabetes?

Mr. DUDLEY. Yes, they do. That is a requirement of camp.

The CHAIRMAN. That is a requirement.

Mr. DUDLEY. Yes.

The CHAIRMAN. Would you just repeat once again the comment of the parents, that they are saying this is the one week in their life that they do not have to worry about their children's blood condition during the middle of the night.

Mr. DUDLEY. Absolutely. One of the greatest fears of parents is that during the night while their child is asleep, they will have a blood glucose reaction; their blood sugar will drop during the night. So the parents get up and check them during the middle of the night. Especially with the younger ones, parents tell us that this is the only week that they can sleep through the night because they leave these kids with us for the week. It is really two camps in one. It is a basketball camp with a basketball staff, and it is a diabetes camp with a diabetes staff, and doctors and nurses and counselors. We test them during the night, and we take care of everything. In fact, the parents have to check their children into the camp, and then they check them out, and they meet with our doctors.

To get back to your point, they feel comfortable leaving their kids with us during that week, and this is the only week of the year that some of them actually sleep through the night.

The CHAIRMAN. How many kids do you have?

Mr. DUDLEY. We have 75 kids, and that is the most we are able to have because of just handling the medical requirements, boys and girls, 10 through 17. We have figured out that this is the tenth year. Throughout the years, we have had a child I think from every State in the Union except——

Senator CARPER. Not Delaware?

Mr. DUDLEY. Mississippi. No, we have had Delaware. [Laughter.]

Mississippi is the one we have got to work on. I think it was Mississippi and one of the Dakotas. But we have had pretty much the whole United States covered.

The CHAIRMAN. Yours is the only camp in the country of this kind?

Mr. DUDLEY. It is the only camp in the country of its kind that is basketball and diabetes. One of the more—there are other diabetes camps, but they are more along the lines of regular camp, arts and crafts, and this is more of a sports camp we go after it. It is a real basketball camp, and the kids go hard, and they learn how to handle their diabetes while going hard.

The CHAIRMAN. Well, we are just thrilled that this hearing can help spread the message of the remarkable service that you provide, and I cannot thank you enough. I do want to ask you one question. I understand you describe yourself somewhat, as I do myself, a pro-life Republican, and sometimes you are asked to justify that. I obviously have my own reasons for being supportive of embryonic stem cell research. Could you share with the committee why you think it is not inconsistent to be pro-life and pro-embryonic stem cell research?

Mr. DUDLEY. Yes, I can, and that is obviously not an easy issue. When you say pro-life—I try not to get painted into either corner but—because I feel like there are exceptions. But for the most part, I am a Christian, and I believe in life, as I am sure we all do here today. It becomes a difficult question, and I have to wrestle with it when taking a stand for this. I think there are—I think the fact that these are embryonic cells that are going to be discarded anyway, that it is just a shame and a lost opportunity to not protect the life that is already with us today.

The CHAIRMAN. Thank you very much, Chris.

Senator Kohl.

Senator KOHL. Thank you very much, Mr. Chairman, and it is a particular delight, as I pointed out to you before, that we convene today, Chris, to have you here, to break bread and to make peace with you for all the torment that you inflicted on my team over your years in the NBA. [Laughter.]

As I said to Chris, the only redeeming feature of Chris' career with respect to us is that he could not make a free throw. [Laughter.]

Mr. DUDLEY. Thanks for bringing that up.

Senator KOHL. Otherwise, he was and is a great guy and a great player.

For those of us who do not know all that much about diabetes in terms of its daily treatment, you talked about during your career—is it still true today?—that you need to monitor your condition? Explain that one. Do you need to keep in touch with physicians?

Mr. DUDLEY. Yes.

Senator KOHL. How does that work for a person with diabetes?

Mr. DUDLEY. I take anywhere from four to six insulin shots a day, test my blood sugar six to ten times a day. I think I did a little—I got the calculator out last night and figured that over my lifetime I have taken over 35,000 shots of insulin. So it is a disease that does not go away.

Senator KOHL. It does not go away.

Mr. DUDLEY. It does not go away.

Senator KOHL. Is that true about virtually all people who have diabetes?

Mr. DUDLEY. Well, there are two types of diabetes. There is Type I, which is what I have, which is commonly called juvenile diabetes, where my pancreas does not produce any insulin. Type II, which is also called adult onset, although that has changed because now it is becoming more common with younger and younger—it is really becoming an epidemic in this country. Their pancreas produces some insulin, but either not enough or their body is resistant to it. A lot of times they can take pills, exercise, diet, and then it becomes shots. So there are two types. But my body does not produce insulin and will not produce insulin.

Senator KOHL. Currently is there any hope out there beyond embryonic stem cell research?

Mr. DUDLEY. Well, that is a tough question because people are working on transplants and they are working on different areas, and there is hope, but it is not a near-term hope. You know, I have had diabetes since 1981, and you always have to be careful about saying there is too much hope because a lot of people with diabetes have thought that we were that close to a cure many times, and that is a tough thing, especially at a younger age, to be told that is coming around the corner and then it does not come. So, yes, I think there is hope, but how close, I don't know. This seems to be the most promising, the greatest potential Senator Kohl. Out there right now.

Mr. DUDLEY. Out there right now.

Senator KOHL. So that it is fair to say for those with diabetes stem cell research is a huge, huge part of their hopeful future.

Mr. DUDLEY. Oh, absolutely. I think with diabetes and a number of diseases, there is such a tremendous potential with stem cell research that, yes, it is a huge part.

Senator KOHL. Thank you.

Mr. DUDLEY. Thank you.

Senator KOHL. Good to see you again, Chris.

Mr. DUDLEY. Good to see you, too.

The CHAIRMAN. Senator Collins.

Senator COLLINS. Thank you.

Mr. Dudley, when I heard your statement about diabetes being 24/7, I was reminded of the first time that I met a family with a son who is age 10 who had diabetes. He looked up at me, and he

said that his greatest wish was that he could just have one day off from his diabetes. He said, "If only I could take Christmas off or my birthday off." That just touched me so deeply. In fact, it led me to be the founder of the Diabetes Caucus in the Senate, to see this 10-year-old boy having to struggle with the treatment of his disease and never being able to take a day off. So I think your testimony just reminded me so much of that.

It is one thing to ask an adult to struggle with a disease 24/7, but to ask a little child to have to bear that just is so painful. It is one reason that I have been such an advocate of stem cell research.

The issue that I want to bring up with you is whether you could help us better understand—and I know subsequent witnesses will—why stem cell research holds particular promise in the treatment of juvenile diabetes. Is it the hope that there could be islet cells produced from stem cells that could then be transplanted? Or what is the theory that makes stem cell research particularly important in the treatment of diabetes?

Mr. DUDLEY. Yes, I would like to hit two points, and the first with your last point. I think it is becoming that islet cells are the Holy Grail, so to speak, but I better—because we have these three gentlemen here, I think they would be better served going into the detail on that. I don't want to be exposed by them later. [Laughter.]

On your first part about the children, I really—for me I want a cure personally, but that is not why I am here, because I have lived with it. I want it for the children, because I get so touched by dealing with the parents and the children at camp, it is like you said. I have had that same experience, and it really just does, you know, melt you down. I really believe we need to do everything possible to cure it for this children, because they want just one day, you know, to have a normal day.

You know, I realize—and it is so difficult for them to deal with it on a constant level. One of the benefits from the camp that I did not know about or did not know how great it would be until I started the camp was a lot of these kids come from cities or towns or schools where they are the only one with diabetes. They feel so alone out there. They are just battling this and going through it, and that is one of the greatest benefits of the camp, was for them to see that there are other kids who are wearing the same shoes with them, are in the same boat. I think that really helped them. But they are all just great, great kids, and I think maybe it is something about having to deal with adversity at such a young age. But they are tremendous kids, and there is nothing that—I mean, there is nothing that would be better than to cure this disease and let them live a normal life. I cannot imagine a greater thing. I really appreciate your help, what you have done through the years for the Diabetes Caucus.

Senator COLLINS. Thank you, and thank you for your testimony today.

Mr. DUDLEY. Thank you.

The CHAIRMAN. If we find that cure, Chris, I assume that the basketball camp will just be more broadly attended.

Mr. DUDLEY. Exactly. It will be a little bigger.

The CHAIRMAN. Senator Carper.

Senator CARPER. Thanks, Mr. Chairman.

Before I ask some serious questions, let me ask one that is not so serious. Who are you putting your money on in the NBA finals? [Laughter.]

Mr. DUDLEY. You know, my pick before it started was San Antonio, so I have got to go with them. I have got to keep going with them.

Senator CARPER. All right. We are going to see some good defense.

Mr. DUDLEY. We are going to see a lot of good defense.

Senator CARPER. That is for sure. None of them can make foul shots, though.

Mr. DUDLEY. No—well, a couple of them can.

Senator CARPER. A more serious question. Going back to your childhood, I understand you were diagnosed when you were 16 with juvenile diabetes.

Mr. DUDLEY. Yes.

Senator CARPER. Did you have some inkling beforehand? You did not just wake up someday and say, "Boy, I am thirsty and I have got to go to the bathroom." Kind of just tell us how it happened.

Mr. DUDLEY. I was working, playing basketball every day after school, and going to school. It was after the season was over. All of a sudden, I just had to start drinking and I was just so thirsty. I would come home and, I mean, literally drink half a gallon of whatever it was. Usually, especially in 1981, that "whatever it was" was Gatorade or Kool-Aid or whatever and it had sugar in it, so that made the situation worse. By drinking this, you just have to go to the bathroom every 5 minutes and race into the bathroom and not feeling well and just know something is definitely wrong. I was fortunate that—my dad's brother has diabetes. I was fortunate in the fact that my father——

Senator CARPER. He had juvenile diabetes?

Mr. DUDLEY. Yes, he had juvenile diabetes and had struggled with it. Fortunately, technology has gotten a little better, but he had really struggled with it. My father knew that those were some symptoms, and he went to the drug store and got a little home tester and saw that my blood sugar level was sky high and raced me to the hospital. I was fortunate that we caught it when we did then. There are cases where, because you are so thirsty, people pass out and go into a coma because they just keep drinking, and what they are drinking has sugar in it and is just making the situation worse and worse. The reason you are so thirsty is your body is trying to flush out that sugar, and so it is really just trying to do it but it cannot.

Senator CARPER. OK. I think you said that for kids who have juvenile diabetes, their pancreases do not create insulin. Your pancreas created no insulin or small amounts or diminishing amounts over time? How did it work?

Mr. DUDLEY. It is my understanding that it produces no insulin. It just for whatever reason stops producing insulin. Where people with Type II diabetes produce some insulin, are capable of producing insulin, Type I diabetics to my understanding, there is no insulin; or at least if there is, it is so negligible it does not make a difference.

Senator CARPER. For a person whose pancreas produces no insulin, how would, in theory, embryonic stem cell research applied actually make a difference?

Mr. DUDLEY. Well, again, I think this will get touched on later, but I believe it is going to be to transplant islet cells which help produce the insulin. So by having those transplants, they will be able to make my body have the capability of producing insulin.

Senator CARPER. You mentioned at some point in your testimony about flying out, I think you said, to Oregon with President and Mrs. Bush?

Mr. DUDLEY. I was in Portland, OR, and when the President traveled in different States, they recognize—it is part of the Freedom Corps Act, and I was recognized for the State of Oregon. So I met him and the First Lady in August of last year, and I brought out one of my campers and we went out and met the President. Then I was able to travel with him a little bit—not with him but with the party. I was able to talk to the First Lady a little bit about it. She had just gone through a tragic loss, I believe, at that time. I think it was her father. So we were able to talk about the issue, and she was very sympathetic and understanding. Obviously there are differences, but it is kind of my belief that this is not a partisan issue. It is a scientific issue, and there has got to be a way of figuring it. There has got to be a compromise in there somewhere that makes sense to help people.

Senator CARPER. Some of us think that the compromise crafted by my friend and colleague in the House, Mike Castle, comes pretty close to a fair compromise. I do not know how familiar you are with this, but——

Mr. DUDLEY. You know, I have looked at it, I mean the rough sketch of it, and I think it makes a lot of sense. I really do. I think my feeling is that if you are going to take a hard line against it, then you have got to get rid of in vitro in the first place, that it is all or nothing; that if you are doing in vitro, then it makes sense to use these excess embryos for scientific research. If you follow the argument that every embryo should be used, then we should not have in vitro in the first place. That is kind of where I fall on it.

Senator CARPER. One last question if I could, Mr. Chairman, and this may be a question that would be better directed to our next panel of witnesses. But let me just run it by you, and if you have any thoughts on it, fine. If not, we will just hold it in abeyance for now. But with regards to adult and cord blood stem cells, I am a layman at this sort of thing, as others are in the room, but any thoughts with respect to the kind of benefits that they may be able to provide and may not be able to provide in comparison to embryonic stem cells?

Mr. DUDLEY. Yes, sure. From what I understand, they hold potential but not nearly as much, that they are not as—I don't know the terms, but multifaceted. They are not as able to become as many things. There are a lot more issues with them, and it is not something that I think should be either/or. I think it should be both. I think you should look at both. I think you have to go with the most promising, which is what we are talking about today, but that does not mean drop the other ones, because adult stem cell I believe has been around for quite some time, and it has some pur-

poses but does not hold nearly the potential that what we are talking about today does.

Senator CARPER. Well, by watching you testify, I get to see some of the next panel of witnesses behind you, and it is interesting watching them nodding their heads. You might have gotten it right.

Thanks so much for being here, and thank you for the example you provide for all of us in what you are doing for a lot of kids.

Mr. DUDLEY. Thank you for having me.

The CHAIRMAN. We have been joined by another First Lady, the Senator from New York. Senator Clinton, if you have a statement and/or questions, we would be happy to receive them.

Senator Clinton. Mr. Chairman, I would just ask unanimous consent to submit my statement for the record.

The CHAIRMAN. Without objection.

[The prepared statement of Senator Clinton follows:]

PREPARED STATEMENT OF SENATOR HILLARY RODHAM CLINTON

I'd like to thank Senators Smith and Kohl for convening today's hearing on the promise of embryonic stem cell research. Like them, I am a cosponsor of S. 471, the *Stem Cell Research Enhancement Act of 2005*. The House companion bill, H.R. 810, passed that chamber last month, and I would urge Senate leadership to bring it to the floor as quickly as possible.

When the promise of embryonic stem cell research became apparent in the 1990s, the Clinton Administration, working through the National Bioethics Advisory Commission and the National Institutes of Health (NIH), examined the ethical and medical issues involved with such research.

In September 1999, the National Bioethics Advisory Commission released its report, "Ethical Issues in Human Stem Cells Research." In this report, it recommended that research using cells from embryos created, but not used for, infertility treatment, should be eligible to receive federal funding.

By August of 2000, the NIH had released guidelines for research using stem cells. These guidelines would have allowed funding for research from lines derived from embryos voluntarily donated, with no coercion or financial incentives, by couples who had determined, after informed consent, that such embryos would not be implanted or otherwise used in their fertility treatments—in short, embryos that would, if not used for research, be discarded.

And these recommendations are followed in S. 471, which provides for the funding of research conducted in an ethical manner according to these guidelines—that is, research on lines derived from embryos created for fertility treatments and voluntarily donated by parents.

But, because of this Administration's policy, which prohibits federal funding of research on any stem cell lines created after August 9, 2001, we are prohibited from funding research that would meet the high ethical standards developed by both the NIH and independent scientists.

Instead, federally funded scientists, some of whom are here today to testify about their work, are limited to using slightly over 20 stem cell lines, instead of the 78 lines originally thought to be available. And not all of these lines are suitable for research. Some of them may be contaminated with mouse feeder cells, which can increase the risk of creating strains of diseases which can more easily pass from mice to humans.

It's clear that the Administration's policy is far more restrictive than it appeared when first announced. And the limited number of cells available for federal funding means that we are not fully achieving the promise of these cells for research into many chronic, debilitating and fatal conditions.

And this delay is hurting millions of Americans—those living with Parkinson's, Alzheimer's and diabetes, as well as their friends, families and caregivers. We have the potential to develop treatments, even cures, for these diseases, but we can't move forward if we don't have new cells.

The Administration's stem cell policy is not just limiting our ability to discover new treatments for diseases. It is a case where ideology is impending science. Without access to federal funds for embryonic stem cell research, our scientists are fall-

ing behind, as researchers in South Korea and other countries make advances that our scientists simply cannot.

I look forward to hearing both the scientific and the patient perspective from our panelists today, and I hope that with their input, we will learn more about the promise of this research to create medical breakthroughs for many diseases.

Senator Clinton. Mr. Dudley, I want to thank you for your very thoughtful testimony and, more than that, for your example in your work. You have great intellectual and moral authority in how you are addressing this issue, and I agree with you that we have a tremendous opportunity here. Starting back in 1999, the National Bioethics Advisory Commission came up with the formulation that we are discussing now about using excess embryos that were created through in vitro fertilization so long as the donors with informed consent agreed that they could be used for scientific research as opposed to being destroyed. The NIH guidelines in 2000 really ratified the Bioethics Commission report.

I think that there is a growing consensus in the country that really does cross every kind of line one can imagine, particularly partisan lines, that this is a very promising area that there is an appropriate ethical framework for us to follow in engaging in this research. S. 471, which a number of us sponsor, which would put into law the advice of the NIH and the Bioethics Commission of 5 and 6 years ago, I think would be a tremendous step forward. I believe if we can be successful in making that case and passing legislation, as the House recently did, I think you will really be entitled to share much of the credit for that. We can then hope that we can get about doing the research and finding the cures for diabetes and other diseases.

So I just want to thank you for taking your personal story and your celebrity and telling it to the world and running the camp for the children. It means a great deal. We are very grateful to you.

Mr. DUDLEY. Well, thank you very much.

The CHAIRMAN. Chris, thank you so very much. God bless you and your work.

[The prepared statement of Mr. Dudley follows:]

THE PROMISE OF EMBRYONIC STEM CELLS

UNITED STATES SENATE SPECIAL COMMITTEE ON AGING

JUNE 8, 2005

TESTIMONY
OF
CHRIS DUDLEY

Good afternoon Senator Smith and members of the Committee. Thank you for the invitation to appear before your Committee today to tell you about how living with juvenile diabetes has impacted my life and the lives of so many children that I have met over the years through my Foundation and at my basketball camp.

My name is Chris Dudley and I played professional basketball for 16 years with Cleveland, New Jersey, Portland, and New York. I am the proud father of three beautiful children ages 6,5 and 3. I also have juvenile diabetes.

I was diagnosed at the age of 16. I had the classic symptoms of excessive thirst and having to go to the bathroom constantly. My uncle has diabetes, so my dad recognized the symptoms and bought a home test kit and it showed that my blood sugar level was very high. We immediately went to the hospital and I was diagnosed. When I first heard the news, I was devastated. I didn't really know enough about the disease and I tried to learn as much as I could. I was fortunate that the doctors and nurses said that I would be able to continue to play basketball if I was careful about monitoring my blood sugar, and this made me feel better. This is not always the case – many times kids with juvenile diabetes are not encouraged to keep playing sports because of fears of what can happen. I was also fortunate that my family was so supportive and encouraged me to continue to play basketball and not let my diabetes stop me from doing what I loved. After my diagnosis I really looked up to people like Bobby Clarke who had diabetes and was playing for the Philadelphia Flyers and I had heard of a triathalete who had diabetes. I thought, if these people can compete at such a high level then I can play Junior varsity basketball.

Ever since that time, I have been an outspoken advocate for encouraging kids with diabetes to pursue their passions – whether it be sports or other activities – provided they take care of their diabetes. That being said, diabetes is such a hard disease because you have to stay on top of it every hour of every day or you can face serious complications. Diabetes never stopped me from playing basketball, but there were many times where the disease did hinder my performance.

When I was playing in the NBA, I would have to test my blood sugar 14 times on game day and take multiple insulin shots. When you are preparing to play in front of 20,000 people you want your sugar to be as close to normal as possible. This is very difficult to

do, and some days, no matter how hard you try, it's never perfect. It took a lot of practice and monitoring, but I was able to learn how to play the best I could. I was fortunate in that my teammates were always supportive. It was amazing to me how many people have some kind of connection with diabetes, so there was a great understanding of what I had to go through every day just to be able to play basketball.

I also had my battles with diabetes. In college I was in a car accident. After working out, my blood sugar suddenly dropped too low and I ran into a parked car. There are so many variables in trying to manage your diabetes and it is a daily battle. I have had diabetes for 24 years, and I worry every day about the long-term risks and what the disease is doing to my body.

Now that I am retired from the NBA, my passion is my family – my 3 kids – advocating on behalf of research to get us to a cure for juvenile diabetes as soon as possible, and enabling kids with diabetes to pursue their dreams. I started the Dudley Foundation in 1994, and the Chris Dudley Basketball Camp for Kids with Diabetes in Veronia, Oregon. At the camp, I see first hand what these kids – some of them very young – have to go through every day. Some struggle much more than others, not because they are being lazy about monitoring their blood sugars, but because it is just more difficult for some kids to keep their sugar levels in range, as hard as they try. When I talk to kids with diabetes and work with them at the camp, I walk a fine line. I want to show them that diabetes doesn't have to stop you from doing whatever you want to do. But on the other hand, I know that it's not easy for them and that they will never get a day off from this disease. And it's not easy for their parents, either. Parents of the kids who come to my camp tell me that it's the only week throughout the whole year that they can sleep through the night without having to constantly get up to check on their kids.

I worry every day that one of my kids will be diagnosed with juvenile diabetes. And even though I have been very blessed in my life and have been able to achieve great things even with diabetes, this is not the life I want for my children. I am missing my 6 year old son's graduation to be here today, but I explained to him that being in Washington was my opportunity to help people understand why a cure for diabetes is so important.

Last August, I received an award from President Bush for my camp and I traveled with the President in Portland that day. I told him about what it's like to live with juvenile diabetes and the struggles the kids who come to my camp face every day. I also told him that as a pro-life Republican I supported embryonic stem cell research and believed that the federal policy should be expanded to allow our brightest scientists to pursue embryonic stem cell research with vigor. Research is the only avenue to cures and therapies for diabetes and many other diseases, and we should pursue this promising research aggressively within an appropriate ethical framework.

Mr. Chairman, thank you again for this opportunity – it has been an honor to appear before you today.

The CHAIRMAN. We will now call forward our second panel. We are very fortunate to have a very distinguished group. Lawrence S. Goldstein is a Ph.D. from the University of California-San Diego, School of Medicine; and Doug Doerfler is the president and CEO of MaxCyte in Gaithersburg, MD, testifying on behalf of the Biotechnology Industry Organization; and then there will be John Gearhart, a Ph.D. from Johns Hopkins University, Department of Medicine, Institute for Cell Engineering, Baltimore, MD; and Su-Chun Zhang, M.D., Assistant Professor of Anatomy and Neurology, Stem Cell Research Program at the University of Wisconsin.

We welcome you all.

We have by video Lawrence S. Goldstein. We will start with him and then go to you, Doctor. So, Dr. Goldstein, if you can hear me, we thank you for taking the time to participate in this hearing, and we will be pleased to receive your testimony now.

We are just working on the sound a little bit. Now we have got it. Go right ahead.

STATEMENT OF LARRY GOLDSTEIN, PH.D., UNIVERSITY OF CALIFORNIA-SAN DIEGO, SCHOOL OF MEDICINE, SAN DIEGO, CA; REPRESENTING THE AMERICAN SOCIETY FOR CELL BIOLOGY

Dr. GOLDSTEIN. You can hear me?

The CHAIRMAN. We can hear you.

Dr. GOLDSTEIN. Great. Mr. Chairman, members of the committee, I want to thank you for inviting me to testify today, and in particular I want to thank you for letting me testify by videoconference. I am a professor of cellular and molecular medicine at UC-San Diego. I am an investigator with the Howard Hughes Medical Institute. My research that is relevant to today's hearing is focused on understanding the molecular mechanisms that are used to move vital materials inside neurons, brain cells, and we study and are trying to learn how failures of those movements contribute to the development of diseases such as Alzheimer's disease, Huntington's disease, Parkinson's disease, and perhaps others, including mad cow disease.

Now, before I tell you about my science, I do want to just take a moment and thank you, Senator Smith, and your colleagues there for your longstanding support of the Federal investment in biomedical research and, in particular, for your leadership in developing Federal funding, we hope, for broader areas of human embryonic stem cell research. I respect your courage on this issue. I know it is not easy.

With respect to the science, I want to discuss how my research is trying to take advantage of the enormous scientific and medical opportunity provided by human embryonic stem cells. I do want to be cautious. I want to stress that scientific progress in the fight against these diseases, particularly Alzheimer's disease, is very difficult. This is a hard problem, and sometimes our advances are agonizingly slow, even when we have the best tools available to us. Importantly, it is very hard to guarantee the rate at which we can progress. Nonetheless, I and many of my colleagues think that human embryonic stem cells potentially hold the key to major ad-

vances in the search for new understanding of and new treatments for these terrible diseases.

Now, for many diseases, including, for example, juvenile diabetes, as Mr. Dudley just testified about, there is great enthusiasm for using human embryonic stem cells to replace cells that are lost in disease. For Alzheimer's disease, however, I think that there may be an even more powerful approach to the use of human embryonic stem cells to develop new understanding and new therapies. That is what I want to talk about today—another way of taking advantage of this enormous scientific opportunity.

Now, before doing that, I need to explain why it is so hard to learn what happens, what goes wrong in brain cells in brain diseases such as Alzheimer's disease. The bottom line in a sense and an important basic principle is that we can rarely, if ever, do the kinds of biochemical and cellular experiments on brain cells of human patients while they are still alive and while they are still in the earliest and, we hope, treatable stages of the disease. I think you can understand why a patient might not be willing to give their brains to experiments before they have died of the disorder. They still need their brain, after all.

So much of what we learn and can learn about the basic cell biology and biochemistry of brain cells that have this disease comes from studying brain cells from people who have died of the disease already and, hence, were in late stages. The problem is that we then end up studying the cells in the brain after most of the damage has already happened. If you think about it, this is in some ways like trying to detect and prevent, to learn to detect and prevent plane crashes by studying the pattern of wreckage on the ground after planes have already crashed. There is a great deal that is missing.

What we really need in a sense is the black box. We need the black box to reveal what went wrong in the earliest stages of the disease, the nature of the cellular changes and malfunctions, so that we can then learn to treat or prevent these diseases. So in our search for understanding of Alzheimer's disease, we are effectively looking for the black box of this disorder.

The question then is how to find that because, after all, we need to learn what those early changes are so that we might learn to fix them.

Now, a very important approach that we have used for the past decade in the fight against Alzheimer's disease is to take advantage of the existence of very rare forms of the disease that are caused by large genetic changes that give rise to what we call hereditary Alzheimer's disease. These large genetic changes are in many cases known. So what we can do is we can take these large genetic changes, and we can introduce them into laboratory animals such as mice. We can then study the brain cells from these mice and learn what cellular changes and what changes in the brain happen in these mice that have these large-scale genetic changes that cause Alzheimer's disease in people.

The problem is that while we have learned a great deal from this approach—and, indeed, there are many ideas that my lab and others have generated from this approach—I am sure you realize that people are not just big mice, and there are many important dif-

ferences in the physiology of our cells and our brains that lead us to need to test ideas that come from studying mice in human people, human patients, and particularly if we are going to develop treatments and drugs. Of course, the question then is how to do that, and that is where human embryonic stem cells provide what I think is going to be an incredibly important tool for doing this. This is what we are trying to do now in my laboratory.

What we are trying to do is to learn to take these human embryonic stem cells and invert them into the brain cells, the types of brain cells that die and fail to function properly in the earliest stages of Alzheimer's disease. Some of the properties of these cells make it possible for us to make the genetic changes, the large genetic changes in these cells that cause hereditary Alzheimer's disease in people. So what we can then do with these brain cells in a dish that have Alzheimer's disease because of the genetic changes is to study them at their earliest stages and test our ideas that come from studying mice. Ultimately, we think, as we learn which ideas are truly correct, which I hope we will do in the next few years, we can use these cells, we believe, to begin testing and developing new drugs that we can use to treat this terrible disorder, because as you know, we have very little in the way of drugs to treat Alzheimer's disease.

Now, there is a second problem in treating and understanding diseases such as Alzheimer's disease where human embryonic stem cells also have a major contribution to potentially make, and that comes from the observation that most Alzheimer's disease is what we call sporadic. It is not caused by the large genetic changes that are strictly hereditary. Instead, it appears to occur almost randomly. However, there is a great deal of evidence that suggests that each one of us has different genetic susceptibility or potential genetic resistance to the development of this disorder. We think that there are many small genetic changes that each of us harbors in different combinations that interact together or interact with the environment to cause us to either develop or not develop this disease.

The problem is that we do not have a way to study this major form of the disease in animals. It is a huge limitation. These embryonic stem cells, however, potentially give us a way to crack that problem because each different embryonic stem cell line has different combinations of these small genetic changes. We think that we can convert those cells, these different cell lines, to the brain cells that malfunction in this disease and study how those small genetic changes lead to the different cell behavior, cell function in the disease that causes those symptoms. This is where the availability of many different embryonic stem cell lines may turn out to be crucial in the fight against this disorder because we can begin to evaluate how our different genetic constitutions confer susceptibility or resistance to this disorder and potentially teach us how to predict which people will respond to different types of treatment or for whom a particular drug will not work and, thus, help us both in the development of clinical trials, the development of drugs, and not treating people with drugs that are not going to help them.

Now, obviously, these are very difficult goals. We have to work very hard to get there, and we are going to need far more than a

single scientist laboring in San Diego to make the kinds of breakthroughs that are going to be needed on this one disorder.

I want to close with just saying that the ideas that I have just described and the approaches that I have just described for Alzheimer's disease will, I believe, be very valuable in the fight against Parkinson's disease, Huntington's disease, Lou Gehrig's disease, and other neurodegenerative diseases where we do not even understand them well enough to give them a name. But I think in the future, if my colleagues and I have the opportunity to do this, we are prepared to devote our lives to solving these problems using these methods.

Thank you for listening to my testimony today, and I would be happy to answer questions when you have them.

[The prepared statement of Mr. Goldstein follows:]

Testimony of Larry Goldstein, Ph.D.
Representing the American Society for Cell Biology

Senate Special Committee on Aging

Regarding"Exploring the Promise of Embryonic Stem Cell Research"

June 8, 2005

Mr. Chairman, members of the Committee, thank you for inviting me here today and thank you for allowing me to join you by video-conference. My name is Larry Goldstein. I am a Professor of Cellular and Molecular Medicine at the University of California, San Diego, School of Medicine and an Investigator with the Howard Hughes Medical Institute. I serve as Secretary of the American Society for Cell Biology, a professional society of almost 12,000 basic biomedical researchers in the United States and in 50 other nations. I am also the Chair of the Public Policy Committee of the American Society for Cell Biology and Chair of the Government Affairs and Policy Committee of the International Society for Stem Cell Research. My research is focused on understanding the molecular mechanisms that are used to move vital materials inside neurons such as brain cells, and testing the role that failures of movement in brain cells play in the development of neurodegenerative diseases such as Alzheimer's Disease, Huntington's Disease, and others including mad cow disease.

I want to thank you for holding this hearing today, and for your long-standing support of the Federal investment in basic biomedical research at the NIH and the NSF. Every year this vital research brings new understanding and new treatments for the many diseases that afflict our friends and families. I also want to thank you for your leadership and courage in our shared goal of broadening Federal support of human embryonic stem cell research.

I am here today to discuss how my research, and that of other scientists, is trying to take advantage of the enormous scientific and medical opportunity provided by human embryonic stem cells. I want to be cautious and stress that scientific progress in the fight against diseases such as Alzheimer's is difficult and sometimes agonizingly slow-even when the best tools are available; importantly, guarantees are hard to come by. Nonetheless, I, and many of my colleagues think that human embryonic stem cells potentially hold the key to major advances in the search for new understanding of, and new treatments for, these terrible diseases. For many diseases there is great enthusiasm for replacing cells lost or damaged in disease with new cells derived from human embryonic stem cells. For Alzheimer's Disease, I think that there is an alternative approach that may be even more powerful.

To explain why I think there is so much promise with these cells, I want to make a few general points about how human brain diseases are generally studied using Alzheimer's Disease as a specific example of a serious disease where we have very little in the way of

therapy and where we do not understand why brain cells malfunction and die. An important basic principle is that we can rarely do the kinds of biochemical and cellular experiments on brain cells of human patients while they are still alive and in the earliest, and we hope treatable, stages of Alzheimer's Disease. Thus, much of what we learn about the basic cell biology and biochemistry of human brain cells that have Alzheimer's Disease comes from studying brain cells of people who have died of the disease, and hence were in late stages of the disease. Thus, we are often relegated to studying the biology of the brain and its component cells after the ravages of Alzheimer's Disease have destroyed most of the normal functions. In some ways, this is like trying to learn how to detect and prevent plane crashes by studying the pattern of wreckage on the ground after a plane has already crashed. While valuable, what we most need is the "black box" to reveal what went wrong in the earliest stages of failure-the nature of the cellular mistakes and malfunctions so that we can learn how to prevent them or to treat them. Thus, in our search for understanding and for new treatments, we are effectively searching for the "black box" of Alzheimer's Disease.

The question then is how to find the "black box" so that we can learn what cellular events and mistakes cause Alzheimer's Disease and how we might fix them. One very important approach has been to take advantage of rare forms of Alzheimer's Disease caused by known, and in some sense, large, genetic changes. These large genetic changes can be introduced into laboratory animals such as mice and the cellular changes that they cause in the mouse brain can be studied. A great deal has been learned from this approach and a number of important ideas about what causes brain cells to malfunction in Alzheimer's Disease have been proposed. But, people are not just big mice-there are many important cellular differences, particularly in the brain. As a result, ideas that come from studying mice must be tested in human cells and ultimately human patients. Testing ideas in humans, however, is hard for the reasons I have already described. This is where my lab is trying to use human embryonic stem cells to develop a new, and I believe, very important approach to solving this problem. By learning to induce human embryonic stem cells to become the types of brain cells that malfunction in Alzheimer's Disease, and by introducing into them large scale genetic changes that cause Alzheimer's disease in people, we are working to test the different ideas for what goes wrong at the earliest stages in brain cells afflicted with this disease. As we learn which ideas are likely to be correct, these very cells may be important test-beds to evaluate or develop new drugs for treatment.

Another problem that we face in understanding and treating Alzheimer's Disease comes from the fact that most people who develop this disease develop what we call sporadic disease. In sporadic Alzheimer's disease, we do not know of large genetic changes that cause the disorder, but, we suspect that several, or perhaps many small genetic changes combine together, and perhaps combine with environment to cause disease. In this regard, we suspect that each of us has a unique susceptibility profile, or potentially resistance, to Alzheimer's Disease. To my knowledge, we have no way to study this major form of the disease in laboratory animals or to evaluate some of the proposed small genetic changes that have been suggested by genetic studies in humans. This is where I think that human embryonic stem cells have a second, and potentially even more

important contribution to make. For it is here where the availability of many diverse human embryonic stem cell lines may help us. Each stem cell line has a specific and unique genetic constitution; each stem cell line has some or many of the small genetic changes that might control susceptibility or resistance to Alzheimer's Disease; and, each line in principle may be induced to form brain cells that fail in Alzheimer's Disease. These cells can then be evaluated at the cellular and biochemical level to test how the genetic variability found in humans may contribute to the disease state. Again, there may be great value in taking advantage of this variability to test the response of brain cells with different genetic variants to different drugs so that we might learn to predict which people will respond best to different types of treatments.

The ideas and methods I have just described are not limited specifically to Alzheimer's Disease but could be used profitably with Huntington's Disease, Parkinson's Disease, and perhaps Lou Gehrig's Disease. Only further research will tell us for sure, but many of my scientific colleagues and I are prepared to devote ourselves to these goals if given the chance. Thank you for taking the time to listen to my testimony today. I would be happy to respond to questions if you have any.

The CHAIRMAN. Dr. Goldstein, I think in the interest of the time we have on this video link, my colleagues and I will ask questions to you now before going to our next panel members.

Dr. Goldstein, obviously California has passed a bond initiative, and it is not illegal to do embryonic stem cell research in the United States. Has that already provided you additional lines? Is that working? Even if it is working, is there value in the Federal Government playing a role through NIH and other of our research institutions?

Dr. GOLDSTEIN. Yes, Senator, that is an excellent question. California is just getting ready and beginning to establish the mechanisms for issuing funding. But I think there are many important roles—in fact, a much more important role for the Federal Government to play relative to the State governments.

First of all, even the availability of State funding in California will not solve many of the problems. The Federal Government has been a longstanding funder of basic biomedical research since the Second World War. This is the genius of our system, that the Federal Government has funded research throughout our Nation, and most of our best scientists are funded by Federal funds—their equipment, their labs. They all have Federal funds in them. So what we are faced with in California is potentially building separate facilities, which we can do, but it is an enormous waste of resources that could go instead to the fight against this disease.

Second, as much as I love California and as much as I believe that the very best scientists in the world are here in California, I will concede that there are other excellent scientists in other States around the Nation.

The CHAIRMAN. Would they be in Oregon? I am just curious. [Laughter.]

Dr. GOLDSTEIN. There are some very excellent scientists in Oregon. Now, we may try to recruit them from Oregon if something is not done to enable them to work in this important field. The Federal Government has an enormous role to play in ensuring that our best scientists, regardless of where they are located, can participate in the fight against this disorder, because, again, we do not want just one scientist doing this. We want dozens, hundreds, taking advantage of these opportunities and making progress.

The CHAIRMAN. Relative to other countries who may be pursuing this—specifically in Europe and Japan I am aware of—what is the importance of the U.S. Government participating along with other nations? The same logic holds?

Dr. GOLDSTEIN. Well, I think it is partly the same answer, and it is partly that in the drive to develop uniform international ethical standards, both for the treatment of human subjects and for the development of therapies, I think that the United States has a very important leadership role to play, which it has played historically.

I will also note that there is an enormous economic interest here. The United States has a positive balance of trade in this area of its economy, and to be honest and blunt, if I look ahead 10 years from now, I would rather have my children selling therapies to the rest of the world as opposed to buying them from the rest of the

world. I would be loath to see the United States cede its historical lead in this field.

The CHAIRMAN. Doctor, I understand that over 100 new stem cell lines have been developed since August of 2001 and that some of these lines are disease specific. Can you tell me what it means to have a disease-specific stem cell line and what this means to a researcher like yourself?

Dr. GOLDSTEIN. Well, for the case of Huntington's disease, that is perhaps the easiest to understand because I gather that one of those lines or a few of those lines have the major genetic change that causes Huntington's disease, a terrible disorder where the brain malfunctions and people have movement and cognitive disturbances.

The ability to develop brain cells from those cells that have those genetic changes will let us understand what fails early in the disorder and could be an incredibly important tool in solving the problems of that disease.

Similarly, for Alzheimer's disease one approach is to introduce known genetic changes. But, of course, the ability to have stem cell lines that are patient or person specific allows us to compare how that person's disease has developed in their adult state with how we can study the biology of those brain cells that have the identical genetic constitution in the laboratory and really learn what are the nature of the cellular changes that cause the dysfunction, that cause the failure that leads to the inability of these people to remember, to think, to speak, any other terrible symptom with the disorder.

The CHAIRMAN. Are there specific experiments that scientists want to do but cannot do now because of the limitation on federally approved lines?

Dr. GOLDSTEIN. I think that nobody knows the answer to that, but I think in some ways it is a question of rate. The current system in my view—and I will be blunt about this—is incredibly clumsy. It turns scientists such as myself into lawyers and accountants to navigate the very complicated licensing, patenting, and, of course, separation issues if one wants to work with more than just the approved cell lines. Of course, I do want to work with more than the approved cells lines. We have established methods for doing that in my laboratory. I am in a very unusual and fortunate situation because of the Howard Hughes Medical Institute that I can do that. But most of my colleagues cannot, and so they are, as has often been said in this debate, working with one hand tied behind their back. Of course, you can make progress with one hand. I would rather have two.

The CHAIRMAN. Thank you, Doctor.

Senator Kohl.

Senator KOHL. Dr. Goldstein, in your opinion, what will happen to your research and that of others in our public institutions if embryonic stem cell research is forced to be conducted largely in the private sector or in other countries?

Dr. GOLDSTEIN. Well, I think that those are two somewhat different issues. The private sector I think is unlikely to tackle many of these problems in the way that we will in the academic sector. The kind of approach that I described in trying to understand and

develop new treatments for Alzheimer's disease is in many ways a longer-time-frame approach than the private sector with its quarterly reports and its inexorable bottom line can do. They will have the kinds of limitations that academic scientists such as myself will not have. So I think if you limit this to the private sector, it will eventually happen perhaps, but it will happen much slower. I think the issue really is one of time and delay.

Suppose, for example, that it takes 10 years to get to the point where we have some new drugs from this approach. That is a long time. On the other hand, if we delay 5 years before initiating that approach or we have in place restrictions that add 5 years to that time line, that is millions of people who will suffer and die before we have an opportunity to treat them. You add delay on at the end. That is the inexorable and terrible problem of the political situation that exists in this country now with this vital area of research.

Now, with respect to other countries, I have a great deal of respect for my colleagues in other countries. But I also have national pride. I believe in the enormous energy and creativity of the American scientific community, and I believe that things will happen much, much faster with the participation, the full participation of the scientists in this Nation.

Senator KOHL. States like California and my own State of Wisconsin are moving forward with their own initiatives to encourage and provide funding for stem cell research in the absence of a strong Federal policy. With no coordinated Federal oversight or strategy, are we at some risk for creating duplicative research efforts in the different States?

Dr. GOLDSTEIN. I think that is absolutely a danger, as well as a danger of not being able to freely exchange materials, of a patchwork of national regulation where what we can do in one place is different from what we can do in another place. I mean, it creates an enormously complicated playing field.

I think you have to remember that science is not the sort of ivory tower scientist laboring in isolation. We are a very interactive profession, and that interaction allows progress to proceed more rapidly because we are very open with communication of our ideas and our materials in most cases. When Government policies restrict that interaction and our ability to exchange materials and ideas, things go very much more slowly and incredibly inefficiently. It is a waste of valuable human and financial resources to proceed in that manner.

Senator KOHL. Thank you very much, Mr. Chairman.

The CHAIRMAN. Senator Clinton.

Senator Clinton. Thank you very much, and thank you, Dr. Goldstein.

I co-chair the Senate's Alzheimer's Task Force, and I want to thank you for the work that you are doing in this area. In your opening testimony, you discuss sporadic Alzheimer's disease which may be caused by a combination of genetic and environmental factors. Can you please discuss the ways that stem cell research may help us understand how genetic changes are effected by our environment? Will this type of research result in information that can contribute to our public health efforts to try to prevent diseases?

Dr. GOLDSTEIN. That is an excellent question, Senator Clinton, and I think you have really summarized the value of this approach. Using human genetic methods in large human populations, there are a great number of small genetic changes that have been identified in different genes where there is statistical evidence that those small changes may predispose someone to the development of this disease either on their own or perhaps in combination with environmental factors. But it is extremely difficult, if not impossible, to evaluate how those genetic changes affect the behavior of the human brain cells that fail in Alzheimer's disease. So the availability of brain cells that have those changes, which we believe we can get through the manipulation of these human embryonic stem cells, will let us study how those brain cells differ from people who did not develop the disease, whose genotypes or genetic constitution did not lead them to develop the disease. We can compare the behavior of those cells to cells that have the large-scale genetic changes and ask what are the physiological similarities and differences and, yes, the identity of those genes—for example, a gene involved in cholesterol metabolism or a gene involved in the response to foreign pathogens in the immune system. Studying how those things behave in culture gives us important clues about the environmental insults that may tip a cell over the edge into the disease stage because of its unique genetic constitution.

Senator Clinton. Dr. Goldstein, I appreciate your describing for us the problems that American scientists are having because of the lack of any Federal policy that really not only provides permission but a framework in which work can continue on embryonic stem cells.

There is a flip side to that which I find equally disturbing and it does not get much attention. Isn't it the case that right now, in the absence of any regulation other than the President's Executive Order confining research to a limited number of stem cell lines and prohibiting any Federal funding from being used for any further experimentation, there are no rules governing what the private sector does? Right now we have sort of an open door to private sector research. For all we know, if someone has enough money, they could be engaged in reproductive cloning as we speak. They could be engaged in all kinds of research that we might find ethically and morally abhorrent. But because we do not have a legal framework, we have no prohibitions against what goes on in the private sector.

Am I correct in that conclusion?

Dr. GOLDSTEIN. I think that you have made an excellent point, and with the exception of a few States, such as California, that have put in place strong legal frameworks for proceeding, strong legal prohibitions on the kinds of activities you describe, both in the public and private sector. Nationally, we lack that kind of uniform framework, and, indeed, there is a vacuum in many places where unethical or perhaps unreasonable things could proceed in the private sector.

You know, with recombinant DNA, gene splicing technology provides us with a good example of how the opposite can happen, which is that when the Federal Government plays an active, involved, and informed role, the public sector develops guidelines,

regulations, and practices that we practice in the public sector. The fact is that even in the absence of direct law and regulation, the private sector companies are staffed by people that come out of our laboratories. As scientists, we actually embrace regulation. It is not that we want to operate in a completely uninhibited way. We welcome reasonable regulation. We want to participate in the development of those regulations so that they are workable as well as ethically and financially reasonable. The private sector in my experience is happy to follow along and adopt those, either directly through the process of law and regulation, or indirectly, as happened in recombinant DNA, because our people who were trained in the public sector moved into the private sector.

So I hope that we can find a way through this impasse so that we can develop standards, perhaps regulation, that allow the public and private sectors to operate with an agreed-upon set of guidelines.

I will just add, of course, that even in the absence of Federal regulation, the National Academy of Sciences just released a wonderful set of recommended guidelines and regulation which I hope, even in the absence of Federal regulation—although I hope there will be some—our institutions are moving to adopt in many cases, and those guidelines I commend to the committee to have a look at because they are excellent.

Senator CLINTON. Thank you, Dr. Goldstein.

The CHAIRMAN. We have been joined by Senator Lincoln of Arkansas. Senator Lincoln?

Senator LINCOLN. Thank you, Mr. Chairman, and thank you, Dr. Goldstein.

Just briefly, I know that in some of the testimony from our other panelists here, there is some mention about the impact of embryonic stem cell research and its positive impact on the research using umbilical cord and bone marrow. At the University of Arkansas Medical Sciences now in Little Rock, we have become a leader in some of the blood stem cell transplants. I visited one of the research physicians and really was amazed at how much progress we have made in that area of adult stem cell transplants and the abilities that exist there.

Do you have any comments to elaborate on how embryonic stem cell research will further the development of this other type of research?

Dr. GOLDSTEIN. Yes. Although this is not my specific area of focus and expertise, there are two areas where I know there is enormous interest and promise for embryonic stem cell research to make an impact on cord blood and blood-forming stem cell transplants such as those you have described. Those kinds of transplants, as you know, are enormously valuable. If you have a disease that can be treated in this way, it is an enormously powerful way to do it.

However, there are many people for whom we cannot find genetic matches to give them a cord blood or bone marrow transplant. For those people, we will need perhaps embryonic stem cell-derived cells for transplant in the future unless the genetic match issues can be solved in some way.

The second thing is that one can learn a great deal, if you will, about the properties, the behavior of cellular materials, and the ability to use them in different ways, much as you might study the properties of metal if you were trying to learn how to fix automobile engines. That tells you a great deal about how to approach these problems. Human embryonic stem cells have a great deal to teach us about how cord blood stem cells and other stem cells can work in the therapeutic setting and perhaps how they can fail in the disease setting.

So scientists such as myself would never say that one should do one or the other. I am an enormous fan of adult stem cell research for the areas where it can make an enormous impact. I would not say that we should not do that. However, it is, again, the one-hand-tied-behind-your-back problem. We will do better with two hands, and those two hands can work together in order to solve some of these terrible problems.

Senator Lincoln. Thank you so much.

Thanks, Mr. Chairman.

The CHAIRMAN. Thanks, Senator Lincoln.

Dr. Goldstein, you have been a tremendous help to us and have added measurably to the debate that is going on on Capitol Hill, and we thank you so much for your time and your expertise and all the work that you are doing. All the best.

Dr. GOLDSTEIN. Thank you, Mr. Chairman.

The CHAIRMAN. We will turn now to our next witness, Doug Doerfler of the Biotechnology Industry Organization and president and CEO of MaxCyte in Gaithersburg, MD. The mike is yours.

STATEMENT OF DOUGLAS A. DOERFLER, PRESIDENT AND CHIEF EXECUTIVE OFFICER, MAXCYTE, GAITHERSBURG, MD; ON BEHALF OF THE BIOTECHNOLOGY INDUSTRY ORGANIZATION

Mr. DOERFLER. Thank you. Good afternoon, Mr. Chairman and members of the committee. My name is Doug Doerfler. I am president and CEO and founder of MaxCyte, a biotechnology company located in Gaithersburg, MD.

The CHAIRMAN. You may want to pull the mike a little closer to you.

Mr. DOERFLER. I am also here representing the Biotechnology Industry Organization, better known as BIO.

Thank you for the opportunity to present testimony today on the promise of embryonic stem cell research and in support of Senate bill 471, the Stem Cell Research Enhancement Act of 2005.

My company uses cell-loading and gene delivery technologies to develop cell-based therapies. Because many diseases and disabilities are caused by cellular malfunction, medical breakthroughs in the treatment of serious diseases and disabilities can be developed through these cell-based technologies. At MaxCyte, we are working to develop therapeutic products for treating pulmonary disease, oncology, infectious disease, autoimmune disease, diabetes, and other neuroscience diseases.

I want to make two points at the outset of my testimony. First, my company does not perform embryonic stem cell research. Sec-

ond, BIO supports all types of stem cell research, including research using cord blood and adult stem cells.

Mr. Chairman, I am here today to say that to help speed the development of all types of cell-based therapies, we need expanded Federal support of embryonic stem cell research. That is why BIO supports S. 471.

Scientists have found that existing cell lines are not genetically diverse, are difficult to grow, and may be contaminated with animal proteins. Your bill appropriately makes more cell lines eligible for Federal funding while creating a framework to ensure that research is performed ethically.

In particular, BIO strongly supports development of NIH guidelines. We believe this is an important step and is similar to the way the Asilomar Conference helped ease public anxiety and spur the development of recombinant DNA technology during the 1970's.

This committee has heard and will continue to hear about the potential benefits of embryonic stem cell research regarding cures and treatments for diseases and disabilities. Embryonic stem cells have the potential to be turned into any of the body's cell types, meaning they could possibly be developed into replacement cells and tissue for patients whose own cells are malfunctioning. This has not yet been shown to be true for adult stem cells.

It is that potential that has thus far generated the most enthusiasm amongst the scientific community because it shows promise toward developing breakthrough treatments for a variety of intractable diseases, including various cancers, diabetes, Parkinson's, Alzheimer's. We need to know these answers.

However, I would like to discuss other reasons to support this research.

It is important to emphasize that embryonic stem cell research will have a positive impact on all types of therapeutic research and will further the development of cell-based therapies.

Embryonic stem cell research will lead to greater scientific understanding of cell differentiation. This cell differentation is the process by which cells change from a stem cell to perhaps a nerve cell, a brain cell, or a blood cell to perform certain critical functions in our body.

In addition, if this bill is enacted, more genetically diverse cell lines will be available for funding. Scientists will then be able to learn more about how and when genetic anomalies cause cells to malfunction. This will help researchers understand the root causes of many diseases and, therefore, lead to the development of truly breakthrough therapies.

Expanded support of embryonic stem cell research could also go a long way toward reducing the time and expense needed for drug development. New chemical or biological compounds meant to treat diseases could be tested in specific human cells prior to their use in live human beings, accelerating development, reducing costs, and reducing adverse events in patients.

Mr. Chairman, I have heard opponents of your bill say that it is not necessary to expand Federal support for stem cell research because many States are moving forward with their own programs.

Nothing could be further from the truth. There is no substitute for increased commitment from the NIH. In addition to funds, the

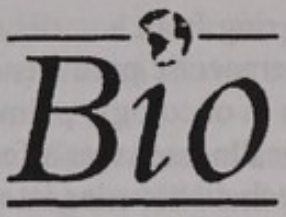
NIH provides the infrastructure and a uniform set of rules for the scientific community—especially as basic research is turned into therapies. Forcing companies to deal with a patchwork of State regulations and requirements will create huge inefficiencies and confusion that will hamper capital formation and inhibit critical collaborations and slow development of treatments for patients.

In conclusion, embryonic stem cell research holds the promise to dramatically improve scientists' ability to develop cures and treatments for disease. Whether these therapies are the direct result of this research or come about due to the advances in scientific knowledge that will come from this work, our Nation must increase its commitment.

BIO supports your legislation, Mr. Chairman, because it will expand Federal support for this important research.

Thank you for the opportunity to present this testimony today, and thank you for your courage in handling this very sensitive issue.

[The prepared statement of Mr. Doerfler follows:]



BIOTECHNOLOGY
INDUSTRY
ORGANIZATION

Testimony of Douglas A. Doerfler
President & CEO
MaxCyte, Inc.
On behalf of the
Biotechnology Industry Organization (BIO)

Special Committee on Aging
United States Senate
June 8, 2005
Regarding Stem Cell Research

Good afternoon. Mr. Chairman and members of the Committee, my name is Doug Doerfler. I am President and CEO of MaxCyte, a biotechnology company based in Gaithersburg, Maryland. I am here today representing the Biotechnology Industry Organization (BIO).

BIO represents more than 1,100 biotechnology companies, academic institutions, state biotechnology centers and related organizations in all 50 U.S. states and 33 other nations. BIO members are involved in the research and development of health care, agriculture, industrial and environmental biotechnology products.

Thank you for the opportunity to present testimony today on the promise of embryonic stem cell research and in support of S. 471, the Stem Cell Research Enhancement Act of 2005.

My company uses patented technology to develop cell-based therapies. Because many diseases and disabilities are caused by cellular malfunction, cell-based therapies have the potential to provide medical breakthroughs in

the treatment of patients suffering from a variety of diseases. At MaxCyte, we are working to develop therapeutic products – including stem cell products – that will have uses in oncology, pulmonary, infectious and autoimmune disease and other illnesses that affect millions of people. Our company will help turn research discoveries into therapies for patients.

I want to make two points at the outset of my testimony. First, my company does not perform embryonic stem cell research. And second, BIO supports all types of stem cell research – including research using cord blood and adult stem cells.

But Mr. Chairman, I am here today to say that to help speed the development of all types of cell-based therapies, we need expanded federal support of embryonic stem cell research.

That's why BIO supports S. 471. Research with existing cell lines has demonstrated the enormous potential of this work. However, scientists have found that these lines are not genetically diverse, may be difficult to grow, and may be contaminated by animal proteins.

That's where your legislation makes its mark. It appropriately expands support for this research by making more cell lines eligible for federal funding while creating a framework to ensure that this research is performed ethically.

The legislation will allow federal funding of research using embryonic stem cells provided the cells were obtained from *in vitro* fertilization procedures that are either in excess or otherwise do not qualify for transplantation. The bill also requires the informed consent of the donors and prohibits any financial inducement to donate. Moreover, the bill calls on the National Institutes of Health (NIH) to develop appropriate guidelines to govern this process. We believe this is an important step and is similar to the way the Asilomar Conference helped ease public anxiety and spur the development of recombinant DNA technology during the 1970s.

This Committee has heard – and will continue to hear – about the potential benefits of embryonic stem cell research regarding cures and treatments for diseases and disabilities. Embryonic stem cells have the capacity to be turned into any of the body's cell types, meaning they could possibly be

developed into replacement cells and tissue for patients whose own cells are malfunctioning. This has not yet been shown to be true for adult stem cells.

It is that potential that has thus far generated the most enthusiasm within the scientific community. According the NIH and the National Academies of Science, human embryonic stem cells have shown incredible promise toward developing breakthrough treatments for a variety of intractable diseases including various cancers, kidney disease, diabetes, multiple sclerosis, Parkinson's Disease and Alzheimer's Disease.

However, I would like to discuss other reasons to support this research.

The majority of companies currently involved in stem cell research use non-controversial sources of cells such as umbilical cord and bone marrow.

It's important to emphasize that embryonic stem cell research will have a positive impact on these areas of research and will further the development of cell-based therapies.

For example, embryonic stem cell research will lead to greater scientific understanding of cell differentiation – the process by which our cells become specialized to perform certain functions – and proliferation – the process where cells expand, or multiply for controlled use as a potential therapeutic.

In addition, if this bill is enacted, more genetically diverse cell lines will be available for funding. Scientists will then be able to learn more about how and when genetic anomalies cause cells to malfunction. This could help researchers understand the root causes of many diseases and therefore lead to the development of truly breakthrough therapies.

Expanded support of embryonic stem cell research could also go a long way toward reducing the time and expense needed for drug discovery. Using embryonic stem cells in drug testing could be significant because of the cells' ability to turn into all types of human cells. New chemical or biological compounds meant to treat diseases could be tested in specific human cells prior to their use in live human beings.

In addition to time saved, this process could reduce adverse events from drug candidates because tests on human cells might reveal harmful side effects before the drug is given to patients.

Finally, Mr. Chairman, I've heard opponents of your bill say that it is not necessary to expand federal support for stem cell research because many states are moving forward with their own programs.

Nothing could be farther from the truth. Put simply, while state support is enormously helpful, there is no substitute for an increased commitment from the NIH. First of all, the NIH can jump start an area of research with an influx of funds.

But just as importantly, the NIH provides the infrastructure and a uniform set of rules for the scientific community – especially as basic research is turned into therapies. The biotech industry has worked effectively for many years with the NIH and academia to develop products for patients. Forcing companies to deal with a patchwork of state regulations and requirements will create inefficiencies and confusion that could inhibit critical collaborations and slow development of treatments for patients.

In conclusion, embryonic stem cell research holds the promise to dramatically improve our nation's ability to develop cures and treatments for disease. Whether these therapies are the direct result of this research or come about due to the advances in scientific knowledge that will come from this work, it seems clear that our nation should increase its commitment.

BIO supports your legislation, Mr. Chairman, because it will expand federal support for this important research.

Thank you for the opportunity to testify today.

The CHAIRMAN. Well, thank you so much for your presence here today and your contribution to our committee.

Mr. Doerfler, this is not the first time new medical technology was considered controversial. There are a number of medical technologies considered routine today that were initially criticized and opposed. I am thinking specifically of recombinant DNA. Do you recall that that was criticized in earlier days as well?

Mr. DOERFLER. That is right. That recombinant DNA was the focus of a number of concerns, certainly in Boston where people were concerned about DNA being placed in drains of apartment buildings. The response was the scientific community stepped forward with the Asilomar Conference back in the early 1970's, and the best and the brightest lawyers, business people, scientists, and ethicists created a set of guidelines that were then used with NIH to create the framework by which recombinant DNA technology was able to not only start but flourish and has resulted in some incredible breakthroughs in the treatment of serious diseases.

The CHAIRMAN. As I understand your testimony, you do not do embryonic stem cell research.

Mr. DOERFLER. We do not.

The CHAIRMAN. But you do adult stem cell research. But you are here testifying on behalf of stem cell research to be expanded in other areas. Is that because you recognize that adult stem cells may work for some diseases and embryonic may work for other kinds of diseases?

Mr. DOERFLER. I am not sure we understand how broadly adult stem cells can be used, and one way of learning about that is being able to study embryonic stem cells, understanding how these cells differentiate into pancreatic cells or nerve cells, so we can directly take the learning from embryonic stem cells differentiation and apply them to cells like umbilical cord stem cells or bone marrow-derived stem cells. It is an important area that we need to know. We just do not have the background yet to understand how cells differentiate and what we need to do to proliferate these cells, expand these cells without them differentiating, which is a huge problem in the stem cell area.

The CHAIRMAN. Thank you.

Senator Kohl.

Senator KOHL. No questions.

The CHAIRMAN. Thank you very much, Doug. We appreciate your time and your contribution again.

Our next witness is John Gearhart, Ph.D., Johns Hopkins University, Department of Medicine, Institute for Cell Engineering. Thank you for being here, sir.

STATEMENT OF JOHN D. GEARHART, PH.D., JOHNS HOPKINS UNIVERSITY, DEPARTMENT OF MEDICINE, INSTITUTE FOR CELL ENGINEERING, BALTIMORE, MD

Mr. GEARHART. Thank you, Mr. Chairman, for the invitation. I think one of the advantages of testifying toward the end is that many of your points were already taken, but I think there is one area that I can be of service to the committee. My laboratory has been working on stem cells for the last 13 years. We were one of the two labs in the country to report the isolation of very unique stem cells from the earliest stages of human embryos.

Since that time, in 1998, we have developed a very large research program at Johns Hopkins centered in the Institute for Cell Engineering, and I think what I would like to present to you is what is going on in an institute such as ours on a daily basis with respect to human embryonic stem cell research, adult stem cell research, and umbilical cord blood research.

Our goal is not to promote one form of stem cell research over another. Our goal is to try to find cell-based therapies. We want to see what works, what does not work.

One of the difficulties in any stem cell research program at this point in time—and we will talk specifically about embryonic stem cell research—is that we have now cells in a laboratory dish that are capable of forming any of the over 200 different cell types that are present in your body. We do not want 200 different cell types present in that dish. We want cardiomyocytes, the heart muscle; we want dopaminergic neurons, those cells that are needed for Parkinson's disease; we want motor neurons.

How do we get them? Now, here is this cell that has these capabilities. How do we direct it to form what we want and in the numbers we need to do any kind of graft for a therapy?

So with these 200 different cell types, I think I have a graduate student working on each one, or a post-doc, and there are problems. We can to some degree generate all these different cell types, but it is a matter of efficiency. We rely upon basic science information that has been obtained over the years, principally through the NIH funding, on how in our bodies from the very earliest stages of embryogenesis a motor neuron becomes a motor neuron or a heart cell becomes a heart cell.

We try to take that information and utilize it in a dish to say, well, we know at this point in time it is seeing this type of a growth factor or any number of combinations of things, and we try to recapitulate the steps to get us to the endpoint. In some of these cases, we have succeeded in generating various kinds of cells with high efficiency. In our lab, we have been interested in cardiac tissue, in motor neurons. We are also interested in dopaminergic neurons for Parkinson's disease. We are also interested in blood cells.

But let me tell you what takes place now. We are able, for example, to grow large numbers of human heart muscle cells. We can do this now through embryonic stem cell technology. We are now in the process of grafting these cells into various animal models, whether it is congestive heart failure, heart attack, to see if these cells will function following transplantation. The same is true of dopaminergic neurons for Parkinson's disease or motor neurons, which you will about hear from Dr. Zhang.

This now is a whole other level of issues that we must confront and solve, and that is, how do we introduce these cells? Do they stay where you put them? Do they form tumors? Do they maintain their function over periods of years, which is what really we are targeting in humans? Do we have animal models that will model human disease, and how effective are they?

So we have our cells in rodents, we have our cells in fish, we have our cells in monkeys—all with the idea of how well will these cells function to cure or to ameliorate any of these disease processes. You say, well, this is great. It is great. This is a major step in the direction of moving toward human trials. But there is much work to do to get it to the human trials.

Where have the limitations come from? Well, quite frankly, it is from Federal funding into this area. We are very limited in what we can do with Federal funding in our laboratories. Dr. Goldstein made an important point that I hope you did not miss. If we are using the approved lines for NIH funding in our laboratories, they must be handled in a very, very different way logistically than lines that we use from Harvard or from Singapore in that it must be clear that everything that touches those cells, including the technicians that are using it, has a straight line and only a straight line to the Federal funding source, that there is no crossover into other areas of where that Federal money is going. That logistically is a nightmare. Most of us have to build separate laboratories to make sure that those walls are there. This is a major, major limitation.

The lines themselves that we use with the Federal sources, they certainly are proving to be valuable in many areas of research. I am not discounting that. But there are limitations. We find, for example, in some of these lines that we cannot isolate a specific cell type out of those existing lines, and we have to go to lines that were generated at Harvard or outside of this to find the cell type of interest that we are looking for. This is a major limitation.

Again, this whole issue of how much flexibility an investigator has among laboratories in the same institution, some working on the human approved lines with NIH—sorry, not human approved, but the approved lines for Federal funding, and down the hall or next door to us we have a lab working on another type of cell line. There is just a logistical nightmare with this as it now exists.

Still, I would commend you and commend the Members of the House for trying to expand the current policy. I think the generation of new lines is extremely important for our work. We will, I think, be more assured that these lines will have utility, that they will be safe, and provide us a broader base as far as the genetics are concerned within those cells.

So, again, I thank you for trying to expand that. I wish I could nudge you to even expand it in other directions, if we could, and perhaps we could talk about that at another time. But at this point in time, it is essential that this bill be passed, and it is essential, I think, as far as national policy is concerned, that we have a better policy covering more aspects of the work.

I will stop there. I would love to get into our issues with our investigators in other countries and how we are dealing with that

competitively, et cetera. But I again thank you for your support on behalf of S. 471.

[The prepared statement of Mr. Gearhart follows:]

THE PROMISE OF EMBRYONIC STEM CELLS

UNITED STATES SENATE SPECIAL COMMITTEE ON AGING

JUNE 8, 2005

TESTIMONY
OF

JOHN D. GEARHART
C. MICHAEL ARMSTRONG PROFESSOR
JOHNS HOPKINS MEDICINE

Senator Smith and Members of the Committee, thank you for inviting me to this important hearing.

An age-old dream of humankind has been to replace damaged, diseased, or worn out parts of the body with new, functional parts. This fanciful dream is coming closer to reality with the recent advances in stem cell research. For the past 13 years I have been deeply involved in stem cell research utilizing sources of human stem cells from embryonic and adult tissues. In 1998, my laboratory at Johns Hopkins published one of the two research papers that year that first demonstrated it was possible to isolate cells from human tissue that could form all the over 200 different cell types in the human body. These unique stem cells are only found in the earliest stages of human development.

The concept behind cell-based therapies is simply stated - if there is a tissue deficit (through disease or injury); correct it by providing the patient with normal, functioning cells. The power of this developing technology is derived from information inherent in our genes and the availability of stem cells, particularly those known as embryonic stem cells. Stem cells, for the immediate future, will be at the center of the developing technology for cell-based therapies. The problems associated with the development of therapies that are effective and safe are immensely difficult, but the potential benefits are extraordinarily great, for those who seek to understand biology or to help the disabled.

The promise of embryonic stem cell research

The promise and hope for the field of embryonic stem cell research was highlighted when stem cells were chosen as *Science* magazine's 1999 "Breakthrough of the Year." Although the first embryonic stem cells were not isolated until 1998, the field has advanced quickly as scientists have already made important progress investigating these cells. Embryonic stem cells are particularly valuable to medical research because they have the unique potential to develop into any cell type, meaning they could produce replacement cells for any tissue and have an impact on virtually every disease. Embryonic stem cells also represent the earliest stages of development, offering a unique

insight into human development and the biology of disease. In addition, embryonic stem cells are capable of dividing and renewing themselves for long periods and can be grown easily in culture, lending themselves to investigation and distribution. Researchers are now learning more about the properties of these cells, optimizing conditions for creating new lines, and have produced encouraging results in a number of experiments regarding their therapeutic potential.

In the area of diabetes research, for example, researchers have made exciting discoveries in recent years that give great hope to the millions of Americans suffering from the disease. In 2002, scientists at Stanford University used special chemicals to transform undifferentiated embryonic stem cells of mice into cell masses that resemble islets found in the mouse pancreas. When this tissue is transplanted into diabetic mice, it produces insulin in response to high glucose levels in the animals. Several studies have since suggested this can be done using human embryonic stem cells. In a 2004 study at Harvard University and the Howard Hughes Medical Institute (HHMI), researchers learned that adult stem cells in the pancreas do not contribute to new beta cell formation in mice. This finding strongly suggests that embryonic stem cells may be the only stem cells that will be useful to generate beta cells for the treatment of type 1 diabetes.

Advances with embryonic stem cells in the areas of heart disease, Parkinson's disease, juvenile diabetes, and motor neuron loss (Lou Gehring's disease)

Stem cell research is supported by overwhelming scientific opinion because the technology may enable us to develop new forms of therapies for some of the most debilitating diseases and crippling disabilities. Presently there are only proofs of principle behind this optimism, but these strongly suggest that if we are permitted to explore these opportunities, their benefits will be realized. Research and clinical efficacy are the only means of validating whether stem cell-mediated therapies will materialize. We are ethically and morally obligated to pursue them for the benefit of those who suffer. To me, a major ethical issue attending stem cell research is the slow pace at which the work is moving to diminish human suffering.

The scientific and medical challenges to attain our goal of providing cell-based therapies that are safe and effective are formidable. As we are learning, it will take the efforts of many scientists and clinicians in a variety of disciplines to bring this technology to clinic. The results of laboratory investigations, to date, however, are highly encouraging and consistent with this goal being attained.

In our laboratory at Hopkins, to which I would like to extend to you an invitation to visit and learn firsthand about ES research, we are pursuing studies on cardiomyocytes, motor neurons and glia, dopaminergic neurons, and insulin-producing cells among several others. We have been concentrating on deriving all these cell types from the stem cell and our success has been dependent upon the state of our basic science knowledge with each cell type. We have been most successful with motor neurons. For the motor neurons and cardiomyocytes we have grafted cells into animals models of heart attacks and motor neuron loss and the grafted cells are functioning. We have a long way to go to improve on the efficiency of generating the cell types of interest and on demonstrating that the

grafted cells will do no harm to hosts. In studies with dopaminergic cells and insulin cells, grafts have also shown some success and we are now working to increase the cell numbers and function.

We do not limit our studies to embryonically derived cells. In our experiments we compare and contrast embryonic, adult and umbilical cord sources of stem cells. This is the only way we will be able to determine which source will be best.

What we've learned since August 9, 2001: Limitations of the current federal policy
In the four years since the President announced his policy decision, the U.S. government's stem cell policy has fallen far short of its original goals, as less than one-third of the stem cell lines the Administration believed would be available for federally funded research are, in fact, available. In addition, a study at the University of Washington found that at least five of these available 22 lines will never be useful for the clinic, and the Chair of the NIH Stem Cell Task Force, Dr. James Battey, has stated that only 23 lines will ever be available for research.

Scientific research and progress since 2001 have revealed the limitations of the eligible lines, and shown us the extent to which these existing lines are not adequate to realize the full potential of embryonic stem cell research. The 22 lines now eligible for federally funded research are contaminated with animal cells, lack genetic diversity, are not disease-specific, and are not adequate for researchers to apply to a wide variety of diseases. Limiting researchers to work only with those lines with federal funding ignores scientific advancements and places unnecessary obstacles in the way of possible therapies and treatments.

Since 2001, we have learned that all the NIH-approved stem cell lines were isolated in contact with mouse "feeder" cells. The possibility of contamination in these lines compromises their quality, makes their therapeutic use in humans uncertain, and raises a high regulatory hurdle that discourages the biotech and pharmaceutical industries from developing treatments using those lines. Fortunately, the field has advanced to a point today where scientists have successfully replaced mouse feeders with either human cells used as feeders or with feeder-free conditions. For example, researchers at the University of California, San Diego and the University of Wisconsin, Madison recently published methods for growing human embryonic stem cells in the absence of mouse-derived "feeder" cells. Laboratories in California, the Czech Republic, Singapore, Israel, Sweden, and Finland have also isolated lines of embryonic stem cells that are not contaminated by mouse feeder cells. However, none of these lines are accessible to federally funded researchers in the United States.

The absence of disease-specific stem cell lines eligible for federal funding means that the current policy is limiting research on dozens of genetic diseases and potentially adding years to the discovery of treatments for millions of Americans. Since 2001, scientists have created "disease specific" stem cell lines that were derived from embryos identified as having a serious genetic disorder such as muscular dystrophy, fragile X syndrome, or Fanconi's anemia. None of the 22 stem cell lines approved for use by the NIH carry a

gene defect for these or other genetic diseases such as cystic fibrosis and Huntington's disease. Even diseases such as heart disease and cancer that are only partly genetic would greatly benefit from federally funded research on cells carrying disease-linked genes. Stem cells can be used to both gain a better understanding of normal cell development, and to derive insights about defects in that development, which cause the disease. This type of research could speed the development of treatments and cures for millions of Americans who are in a race against time.

A survey of laboratories around the world found that at least 128 human embryonic stem cell lines have been created since the current federal policy on embryonic stem cell research was enacted in 2001. Fifty-one of these lines are now available to researchers worldwide, but none of the lines can be used by federally funded researchers in the United States because the lines were developed after the 2001 policy enactment. Many of these new lines are "disease specific," and all of the lines were created in the absence of the mouse "feeder" cells that threaten therapeutic use in humans. In addition, scientists now know that the conditions in which cells are cultured plays an important role in maintaining cell stability, which is crucial for experiments, and that the older cells that are eligible for federal funding are more susceptible to abnormalities and chromosomal changes because the longer stem cell lines are grown, the more likely they are to lose their properties. Permitting the use of federal funds on stem cell lines created after August 9, 2001 would allow scientists to reap the benefits of the many advancements made since that date, and allowing this promising field to keep pace with science.

Why the current policy should be expanded

As we discuss the potential for embryonic stem cell research, it is important to remember the nature of science and the time it takes to investigate different paths, form and test hypothesis, pass rigorous clinical trials to advance to the desired result of therapeutic use in humans. Although embryonic stem cell research remains on the cutting edge of biological science today, the field is still in its infancy. It is in these early stages of research that federal funding is so essential. Since its founding, the United States government has taken the lead in funding the breakthrough medical research that has improved the health of the nation and saved lives. Today, the NIH is the engine of scientific research in this nation, investing more than $28 billion annually into medical research. The absence of strong federal support in this area has clearly hindered the field of embryonic stem cell research by discouraging researchers from entering the field and removing the collaborative and oversight mechanisms that are so vital to the advancement of medical research.

Expansion of the current federal policy on embryonic stem cell research is more than just a matter of deriving new stem cell lines. We will be assured that we are working with the best lines available from the standpoints of utility (no feeder layers, in defined media), safety (no contact with the products of other animals) and performance (we will be able to determine which lines out of many will produce the cell types that we need). We will be able to utilize cell lines with a wide range of genetic backgrounds, being able to investigate the role if these backgrounds in a variety of studies, from toxicology to tumor

formation. These new lines will also help in avoiding immune responses to grafted cells by eventually being able to match HLA types (genes involved in graft rejection) with patients – if clinical trials are started. All of these factors are important and significant improvements over existing lines that are eligible for federal federal funding.

Due to the uncertain political landscape of the field, few young investigators have been willing to begin careers in this area—despite NIH's efforts to attract investigators to the field. Young researchers are unwilling to begin their careers in a field that, no matter how promising, lacks guidance and support at the federal level. Perhaps the most vivid example of researchers' reluctance to join this promising field is seen at the level of funding and applications for stem cell research. Despite an Administration goal to fund $100 million annually in stem cell investigation, less than $25 million was allocated in Fiscal Year 2003. Researchers are clearly wary of entering a field that holds so much promise, yet remains mired in uncertain funding, regulatory, and political conditions.

In the absence of a strong, coherent federal policy, the field of stem cell research will progress slowly and inefficiently. The nation's top scientists, researchers, and 80 Nobel Laureates have urged the NIH to lead our nation in oversight of this research. State initiatives and legislation on stem cell research are not a substitute for strong, federal control. The recently released guidelines on embryonic stem cell research by the National Academy of Sciences (NAS) signal a strong step forward in the field, and should viewed as a model for oversight by the federal government.

CLOSING POINTS :

- progress is being made but there are formidable obstacles to provide effective and safe cell-based therapies. It will take time and it will take investment.
- an expanded federal policy would untie hands of researchers and hasten pace of progress
- we know more about embryonic stem cells than we did in 2001 and this knowledge has made it clear the current federal policy will not enable us to achieve the goals stated by President Bush in a reasonable time frame.

We have an incredibly diverse society, with many different cultures, beliefs, morals, religions and laws. In our pluralistic society, there must be ample room for differences concerning the moral and ethical interpretations of the earliest stages of human development, especially where acknowledging these alternative legitimate views can mean the difference between life and death for many of our citizens. If we can realize the potential of these incredible stem cells we will be witness to, and in benefit of, one of the greatest contributions that science has made to the improvement of our quality of life. The promises of embryonic stem cell research are too great, and the alternatives are too few. Research in this area must go forward under appropriate oversight and with funding that will enable US investigators to fulfill these promises rapidly. Therapies delayed are therapies denied.

The CHAIRMAN. Dr. Gearhart, I came from a luncheon of my Republican colleagues where there was—and in the interest of confidentiality, I am not going to mention anybody's names, but there were a lot of pro-life Senators in that room with different opinions on this issue. One of my colleagues, who will remain anonymous, made the point that there is a new type of embryonic stem cell research that does not destroy the embryo. I do not know what the name of it is or I would tell you that, but you probably know what I am talking about because it has been in the newspaper. The point he was making is that this argument will be moot in the very near future if that type of research is the one that goes forward.

Can you speak to that?

Mr. GEARHART. Oh, I would love to.

The CHAIRMAN. Is that person wrong on that?

Mr. GEARHART. Well, look, one of the most disheartening aspects of being in these stem cell battles—I have been there for 7 or 8 years—is the distortion of facts around any aspect of either other avenues that can work, the claims around the adult stem cells, for example, of doing everything that an embryonic stem cell can do. This is just very disheartening, and I would like to quote one of your former great Senators from the standpoint that, he was fond of saying you are welcome to your own opinion but not your own facts. This is the case:

We saw recently published in the Washington Post an article by Rick Weiss in which he summarized several new alternatives to the use of embryos or the destruction of embryos in embryonic stem cell research. None of this is published. Some of it is in the very earliest stages of research. If you read this carefully—and I would tell you I have read some of the manuscripts that have come out of this. I probably should not say that. This is not—the frequency of success in this is extremely low. There are many holes in the experimental designs and the outcomes of what people have even presented at meetings up to this point.

If we were to wait around for this to work, I mean, to say, yes, I would love just to take a single cell, one cell off an embryo and say that we can generate an embryonic stem cell line out of that, leaving the remainder of the embryo to be used for reproductive purposes, the frequency and success of this is so low you would have to sample from hundreds of embryos. No one is going to permit this in any kind of a protocol before an IRB, internal review board. It is not going to be that way.

So I would just say to you I hope it would work.

The CHAIRMAN. But it is not the silver bullet that it was presented as being?

Mr. GEARHART. It is not the silver bullet, and if we were to wait around any longer before we really established robust ES work in this country, we are really going to be behind.

The CHAIRMAN. That was my supposition to what was being said and argued very strenuously, but there was, you will be pleased to know, lots of Senators arguing in a different direction, in the direction you are advocating.

You are from one of the great medical schools in our Nation, and I don't know that Maryland has passed any bond initiative like

California's. What does the range of the CA-initiative mean to Johns Hopkins?

Mr. GEARHART. Well, we came, interestingly, within one vote of a filibuster in the Senate of Maryland of perhaps passing legislation. It was not a bond issue. It was $23 million for embryonic stem cell research.

I think you could argue the point in two ways. I wish that States did not have to get into this. I mean, money is scarce. States can do many things with it. But because of the national political scene, we have to get in it. I think we have to get in it not only to support our researchers, but I think where those States are going to be successful is they are going to serve as catalysts for bringing in biotechnology that is going to be there for decades.

This is what is happening in California. People are looking there. I mean biotech companies. The other thing which I am personally upset about is that they are coming to members in our research group and saying, How about a job in an environment with money? You do not have to worry about these things. For particularly young investigators, this is a major draw. I think we are going to see this not only in California, I believe New York is going to be in play, New Jersey is in play, other States are going to be in play. I think we are going to get partitioning out of not just stem cell research but, as I say, there is going to be a movement to where there is a progressive outlook on biomedical research, and it would be characterized by those States. So it does have an impact, I think, both in getting our work done and having the personnel that we would like.

One other point. For us to get this technology to the clinic, it is not just going to reside in the academic setting. We are going to have to be partnering with biotech companies, pharmaceutical companies, because it is going to be expensive to get it into the clinic. To go through trials and things like this, we are going to need the partnerships with biotechnology and pharmaceuticals, and if they are going to other places where this is more appropriate, we are not going to have it.

The CHAIRMAN. Frankly, the money and where people live that are doing the research is a very, very secondary consideration, but I think you have just told me that Johns Hopkins, Harvard, Oregon Health Sciences University, you know, they are going to be out of the business in a sense. They are certainly going to be in the back benches of this effort if the Federal Government does not participate financially and by creating ethical boundaries.

Mr. GEARHART. One of the arguments that is made is why isn't it just handled by the private side. I do not see where all the money would come from. I mean, we are now—yes, we are the recipients, as other institutions are, of millions of dollars for some of this research. But this cannot go on forever. Clearly, I think at the state-of-the-art of the work, at the very early stages, we need the Federal support which historically has come.

The CHAIRMAN. Senator Kohl.

Senator KOHL. Thank you, Mr. Chairman.

Your comments, your questions, and your responses have been quite informative and have really added to the depth of the dialog, and we appreciate very much your being here.

Mr. GEARHART. Thank you, Senator.

The CHAIRMAN. Thank you very much, Doctor. We appreciate very much your presence.

I think it would be appropriate for me to allow your Senator to introduce you.

Senator KOHL. Thank you, Mr. Chairman. Our next witness, Dr. Su-Chun Zhang, is a researcher at the University of Wisconsin-Madison at the renowned Waisman Center. He is known worldwide in the science community for recent scientific breakthroughs that successfully coaxed stem cells into becoming human motor neurons, the nerves responsible for movement throughout the entire body. Dr. Zhang's research illustrates the enormous potential of embryonic stem cell research in treating and curing diseases affecting the lives of Americans suffering from Parkinson's, ALS, and other disorders. So we welcome you here, and we are looking forward to your testimony.

STATEMENT OF SU-CHUN ZHANG, M.D., PH.D., ASSISTANT PROFESSOR OF ANATOMY AND NEUROLOGY, STEM CELL RESEARCH PROGRAM, WAISMAN MENTAL RETARDATION CENTER, UNIVERSITY OF WISCONSIN-MADISON, MADISON, WI

Dr. ZHANG. Thank you, Mr. Chairman, for inviting me to testify before you about the recent progress in the area of human embryonic stem cells. Since I am the last one, most of the points have been made. But I just would like to tell you how I got into embryonic stem cell research, how we feel about the current state of embryonic stem cell research in this country.

I used to work on brain stem cells, one kind of the so-called adult stem cells. The hope was to use these brain stem cells to produce specialized brain cells to repair neurological disorders. But it turned out that even though these brain stem cells can produce nerve cells, they actually have very limited capacity to produce very specialized nerve cells like dopamine neurons, motor neurons, or oligodendrocytes that are lost in multiple sclerosis patients and other patients.

That was one of the major reasons why I contacted Dr. Thomson, Jamie Thomson, who was the first person, along with Dr. Gearhart, to establish the first human embryonic stem cell lines in the world. We started, but because of the sensitivity of embryonic stem cell research, actually I waited for a year and a half in order to set up a separate small and very rudimentary laboratory outside of the institute in order to conduct this kind of research.

Like many laboratories in this country, my own laboratory was not able to use these cells until 2002, after the President's decision on the Federal funding on human ES cells. But if you look back just the past three years, the progress that has been made in the area of embryonic stem cell research is quite enormous.

It has already been shown that many cell types can be produced from human embryonic stem cells, including heart muscle cells, brain cells, or blood cells. In particular, at least in the literature, peer-reviewed literature, it has been shown that the dopamine neurons can be very efficiently produced from these embryonic stem cells, including from my own lab, and motor neurons which control our movement can also be produced from these cells. There is a re-

cent report showing that another type of cells which are making myelin sheath, and are lost in multiple sclerosis patients, can also be produced very efficiently.

So by just looking at this very short period of time in terms of the progress made in this area, it is quite unprecedented in the history of science, and it speaks itself of the great potential of embryonic stem cells.

Furthermore, actually recently it already began to show that these cells may work in repairing some of the damages in animal studies. I think what has been shown lately in animal studies, if you transplant some of these embryonic stem cell-derived nerve cells, they can help animals that have motor neuron disease or Parkinson's disease or even spinal cord injury. Of course, a lot more work needs to be done in order for this technology to be actually used in patients.

Now, if you look back at the past two or three years or beyond, there are also problems. The problem is that we have only a limited number of stem cell lines to work, and plus these cell lines were originally derived from growing on animal cells. Now more and more investigators want to use these cell lines. So the number of lines are simply not sufficient for the community to work, at least using Federal dollars.

Further, if you want to use some special stem cell lines, for example, some cell lines that have genetic defects which will allow us to understand what is going wrong and how to correct them, these are still not allowed to be used using Federal dollars. For example, my institute works on mental retardation and genetic disorders, and we are very keen to use these cell lines. But if I take these cell lines and I already have Federal money, it just is so difficult to separate these Federal dollars away from the private funding. So the reality is the current situation, current rule that you can work on these stem cell line using Federal dollars, actually not only slows down the research using stem cells, but also interferes, actually affects the effective use of private funding. If I am going to use private funds to work on the cell lines currently not in the (NIH) registry, I have to again go back to what I did several years ago to set up a separate lab outside of the area I am working on. So these problems really hamper the area of research.

That is why we really want to urge the Senators to consider changing the current rule. I think you already mentioned that you will.

Finally, before I came here, some of my graduate students grabbed me: "You have to deliver another point." That is, we Americans actually led the world by first establishing this human embryonic stem cell work, including the pioneers, Dr. Thomson and Dr. Gearhart here. Yet we should not be left out. I told them yesterday, I said the Senators are much smarter than us. They want Americans to lead the way, and will not let us down in leading the world in this area of promising research, which could potentially be saving life and improving health for all Americans.

[The prepared statement of Dr. Zhang follows:]

Exploring the Promise of Embryonic Stem Cell Research

Su-Chun Zhang, MD, PhD
Waisman Mental Retardation Center, University of Wisconsin-Madison

June 8, 2005

Mr. Chairman, Senator Smith, and Members of the Special Committee, I appreciate the opportunity to appear before you to testify about the recent progress in human embryonic stem cell research, particularly in the area relating to neurodegenerative diseases, and the future direction of stem cell research from the perspective of the scientific community.

Human embryonic stem (ES) cells were first established in 1998 by my colleague James Thomson at the Madison campus. His report attracted immediate attention in the scientific community and the entire world, because the human ES cells could become an almost limitless source for the many specific cell types in an adult body. I was studying the therapeutic potential of stem cells that were isolated from human brain tissues, one type of the so-called adult stem cells. These brain stem cells can produce nerve cells and supporting glial cells. However, the brain-derived stem cells have limited, if any, potential to produce such specialized neural cells as midbrain dopamine nerve cells that are degenerated in Parkinson's patients, spinal cord motor neurons that are lost in Lou Gehrig disease, and the myelin-producing oligodendrocytes that are attacked in multiple sclerosis patients. This remains true today after over a decade's effort in the scientific community. Hence, scientists like myself were seeking a better cell that could provide a continual and standard source of human cells for both scientific exploration and potential therapeutic application, and human ES cells were the answer.

I had the privilege to have my hands on the human ES cells at the end of 1999 through collaboration with Dr. Thomson. I was fascinated to see the plain-looking ES cells become beating heart muscles or beautiful process-bearing nerve cells in a Petri dish in a matter of weeks. Like many other investigators in this country, my laboratory, located at the NIH-sponsored Waisman Mental Retardation Center, was not able to use the human ES cells for research until the middle of 2002. In the past three years, progress and problems have begun to emerge in the field of human ES cell research.

Because of the limited number of available human ES cell lines, one question scientists asked was how stable and for how long the current ES cell lines could be maintained with current technology. Studies coordinated by a few laboratories around the world had examined some of the existing cell lines and found that human ES cells are relatively stable. However, genetic changes happen in cells cultured for a long-term and under some special culture conditions. This suggests that more cell lines will be needed.

Human stem cell lines established thus far grow on animal cells. A recent study confirms the presence of some animal contaminants in the current cell lines. Hence, new cell lines that are free of animal products will likely be needed in order to use stem cells in clinics. Through the efforts of Dr. Thomson and others, progresses have been made in this direction. Several groups have explored the possibility of using human cells, including the cells derived from human ES cells, to support the growth and derivation of human ES cells. Most recently, the WiCell Institute has developed a method to grow ES cells without the need of animal feeder cells. This technology significantly simplifies the growth of human ES cells, which will enable more investigators to get access to the stem cells. With further tuning of this technique, new human ES cells may be generated free of animal contaminants, which will clear the first roadblock to clinical application.

When the human ES cells take the path to more specialized cells such as the earliest brain cells (also known as neuro-epithelial cells), they form brain-like structures in a Petri dish at the right time when our brain begins to form during our development. This indicates that human ES cells offer an unprecedented and otherwise unavailable tool to unveil the secret of human development.

Similarly, human ES cells with genetic defects would allow us to investigate how each gene defect results in developmental disorders. Some of these human ES cells, such as those with adrenoleukodystrophy, Fragile X Syndrome, muscular dystrophy, Fanconi anemia, Huntington's disease, neurofiromatosis, and others, have been established through pre-implantation genetic diagnosis (PGD), a test used to avoid transferring diseased embryos into the uterus of a woman undergoing in vitro fertilization. This type of genetically abnormal human ES cells will be a precious asset to the scientific community to find ways to correct genetic defects.

The first and critical step in making human ES cells useful in patients is to teach the naïve stem cells to become a functionally specialized cell, e.g., dopamine neurons or pancreatic insulin-producing islet cells. In the past 2-3 years, reports have emerged that human ES cells can produce functional blood cells, cardiac muscle cells, brain cells, etc. Dopamine-secreting nerve cells and motor nerve cells can now be very efficiently produced from human ES cells. Comparing to the decade's failed effort in producing dopamine neurons from adult stem cells, the present development is very encouraging, reassuring us that the human ES cells are indeed much more plastic than other types of stem cells. Efficient production for many other specialized cell types and purification of human ES cell derivatives will likely be a major effort in the next few years.

Studies in diseased animal models have begun to show that these specialized cells produced from human ES cells may be useful in treating certain diseases. Transplantation of human ES cell derivatives into the spinal cord of rats suffering from motor neuron disease promotes the restoration of movement. Similarly, the myelin-producing oligodendrocytes, generated from human ES cells, restore the movement of spinal cord injured rats following transplantation

into the injured area. Cardiac muscle cells, produced from human ES cells, appear to repair infarcted swine heart tissue following transplantation into the infarct area. Dopamine nerve cells generated from nonhuman primate ES cells contribute to functional recovery of Parkinsonian monkeys. These developments in such a short period of time, though preliminary, are unprecedented in the history of stem cell research. It is testimony to the enormous potential of human ES cells in the future treatment of many degenerative diseases.

Immune rejection will be an issue if ES cells are to be used in patients. Scientists are exploring ways to overcome the potential rejection problem. One way is to create ES cells that have the DNA from the patient through somatic cell nuclear transfer (SCNT). A recent report from the Korean team suggests that SCNT is a potentially feasible approach. The patient-specific human ES cell lines are precious starting materials for scientists to determine if ES cells produced through SCNT have therapeutic value.

There are many questions to be answered and many more roadblocks to be removed before the human ES cell technology is applied to patients. Studies testing the function of human ES -derived specialized cells will likely be long-term. Pre-clinical investigations involving the use of nonhuman primates also require significant investment. These long-term studies involving translation of human ES cell technology to application will be significantly enhanced with the support at the federal level.

When I was asked last weekend to testify before you on stem cell research, I began to ask myself why I should work on the controversial embryonic stem cells. I suddenly realized that I already spent 20 years of my life growing nerve cells with the recent 10 years on brain stem cells. It is with the embryonic stem cells that many scientists and I are able to produce certain specialized cells such as dopamine neurons and motor nerve cells that we could not achieve for decades just a few years ago. These ES cell-derived special nerve cells may be useful in treating some devastating diseases such as Parkinson's disease, Lou Gehrig disease, Multiple sclerosis, and spinal cord injury, although significantly more research needs to be supported in that direction. Besides the transplant therapy, the stem cells can help us unravel the mystery of human development, offer a standardized tool for screening toxins or therapeutic agents, a tool for discovering the function of new genes, and a vehicle for delivering therapeutic gene products to targets, all of which can indirectly benefit our health and society. The progress in human embryonic stem cell research made in just the past few years, as outlined above, is itself the testimony of the enormous potential of human ES cells. It will be wise and responsible to support the research aiming at saving lives and improving the health of all Americans.

The CHAIRMAN. Dr. Zhang, to that point, are there other nations that are ahead of us now in this area?

Dr. ZHANG. In some areas, for example, the cloning of human-specific cell lines, because these techniques have been already there, here in Wisconsin or in Johns Hopkins.

The CHAIRMAN. You are referring to the therapeutic cloning.

Dr. ZHANG. Yes.

The CHAIRMAN. But how about in the embryonic stem cell?

Dr. ZHANG. Well, sir, as I mentioned, currently we only have limited numbers of cell lines available.

The CHAIRMAN. Can you name a country that is proceeding on embryonic stem cell research? Is Great Britain?

Dr. ZHANG. Great Britain, Japan, South Korea, Singapore, Australia, also including China, Israel, many, many other countries.

The CHAIRMAN. Dr. Gearhart, do you have a sense of where they are relative to where we are?

Mr. GEARHART. Yes there are certain ways in science that you measure where progress is being made. One of the best measures, Senator, is looking at where publications are coming from, just the number of publications and the quality, you could argue, also, of that. Initially, as was pointed out, this work started in the U.S., and we saw U.S. investigators publishing most of the papers. The last few years we have seen a tremendous upswing in papers coming from these other countries, just in sheer volume and in quality. Good work is being done by these investigators. So that is one measure that we take as to, you know, where this work is being done.

The second is looking at the investment, either through the government or through the private side, and we are seeing hundreds of millions of dollars dumped directly into embryonic stem cell research. This does not deal with the cloning issues at all in whether it is Singapore, whether it is China, Israel. We can go down the list, and this is another measures of just looking at where the investment is.

We also now look at where the investigators are going to do this work. Now, before California was in play, you know, students, post-docs, were leaving the country. Now they may go to California, which would be the same difference, perhaps. But the issue is it is in play now. But I am telling you that when our students were finishing and looking for jobs, looking for what we call post-doctoral fellowships, they are looking at other countries.

The CHAIRMAN. Dr. Zhang, you mentioned that the President's decision to allow embryonic stem cell research to proceed on these 78 approved lines has developed some real progress on the issue.

Dr. ZHANG. Yes.

The CHAIRMAN. But as I have read, the way in which they were allowed to be generated through mouse cells ultimately makes it impossible for them to take this page and just xerox it, you know, ad infinitum, and that they are just played out.

Dr. ZHANG. Yes, it will make it very difficult for you to translate what progress you made using the current lines to application to the clinical side.

The CHAIRMAN. So we are really at a dead end at this point in making progress.

Dr. ZHANG. Exactly.

The CHAIRMAN. Do all of you agree with that in terms of the lines that are available, they are played out at this point, in terms whether we can do more?

Dr. ZHANG. We can still use the existing lines to do basic research, but in order for the translation from basic to clinical side, we need cleaner lines that are derived using different technology. The reality is this technology is being developed. As mentioned earlier, Dr. Thomson's lab in Madison already established a method that you can grow these human embryonic stem cells without the animal cells as supporting feeder cells. In other words, you can potentially generate new stem cell lines that will be free of animal contaminants.

The CHAIRMAN. So what we have done to date has been of value, but the potential of these lines has been maximized. Is that what you would say, Dr. Gearhart?

Mr. GEARHART. I agree with that. From a pure utility standpoint in the laboratory, to work on lines that have to be grown on other types of cells makes it just very difficult to identify specific components that are critical for differentiating these into some specific types. So we would rather work on lines that had no feeder layers—these are now available—or have never seen an animal product. When you say animal product, you are generally talking about a mixture of stuff, we do not know what is in it, but they sure grow well. We would like to define what is in it, and that is now what is being done with some of these other lines that are available in what Jamie has recently done. This is an extremely valuable reagent that should be eligible for Federal funding if we want to progress.

The CHAIRMAN. Just for my own research, I understand that adult stem cells may actually really work well for some sorts of afflictions, but that it is more difficult to coax adult stem cells into specific cells. Is that accurate?

Dr. ZHANG. Yes.

The CHAIRMAN. Can you access specialized cells for advanced diagnosis and treatment of various diseases? Can you tell me what are adult stem cells most promising to help, and what are embryonic stem cells most likely to cure?

Dr. ZHANG. For some adult stem cells, like blood stem cells, they have been used in the clinic for years to treat anemia and leukemia and other disorders. But in terms of the nervous system, some adult stem cells can be used as vehicles to deliver some therapeutic agents into the brain because they can still generate nerve cells or some glial cells, the supporting cells of the brain. Therefore, I think they can still be used.

Also, maybe some time in the future when we figure out how to get embryonic stem cells to specialized nerve cells, we may actually learn these tricks and apply these tricks to adult stem cells and then teach these adult stem cells to become specialized. They may still have the potential. That is why in the scientific community we support both types of stem cells instead of just one or the other.

The CHAIRMAN. Cord blood as well.

Dr. ZHANG. Sure.

The CHAIRMAN. Senator Kohl.

Senator KOHL. Thank you. To put that in another way and elicit an opinion from you, you are saying, as I understand it, that embryonic stem cell research is essential, that adult stem cell research all by itself is really not a viable option.

Dr. ZHANG. I didn't say that. [Laughter.]

If I was understood in this way, then—no, I am saying that different types of stem cells have different kinds of uses.

Senator KOHL. Right.

Dr. ZHANG. I think Senator Smith already asked this question. I think the answer would be the same. Adult stem cells have their use in specific areas, and embryonic stem cells also potentially have their usage, although we are just beginning to understand how they might work. They actually will fuse to each other and to promote the understanding how stem cells, whether they are in embryo or in adult, work.

Senator KOHL. Yes. Is it fair to say it in another way that to maximize what you are doing, you need to have opportunity to do both?

Dr. ZHANG. Sure.

Mr. GEARHART. Correct.

Senator KOHL. That is categorical in your opinion.

Dr. ZHANG. Absolutely.

Senator KOHL. It is not subject to opinion anymore.

Mr. GEARHART. No.

Dr. ZHANG. No.

Senator KOHL. I thank you.

I thank you, Mr. Chairman.

The CHAIRMAN. Do you have any closing comments?

Senator KOHL. I am done.

The CHAIRMAN. OK. Gentlemen, this has been very, very helpful, and your contribution will be reflected ultimately when this debate goes to the Senate floor. So we thank you very much for your time. We respect your work, and we hope to help you advance it.

Chris Dudley, again, our many thanks for coming this long way.

With that, the committee is adjourned.

[Whereupon, at 4:13 p.m., the committee was adjourned.]

APPENDIX

STATEMENT SUBMITTED BY THE JOHNS HOPKINS UNIVERSITY

One of the greatest discoveries in Medicine is the potential to use a single undifferentiated cell to help address the severe pain and suffering that numerous diseases, such as heart disease, diabetes, and cancer inflict every day. However, The Johns Hopkins University recognizes that stem cell research raises significant ethical concerns and that public policy on stem cell research must carefully balance the ethical and medical considerations, yet enable researchers to fulfill the promise of stem cell research for providing medical therapies.

Johns Hopkins strongly supports the use of stem cells for legitimate research and therapeutic purposes. Stem cell research promises to have an enormous impact on human health and quality of life, and also on fundamental biomedical understanding. Stem cells can be obtained from embryonic, fetal, and adult tissues. It is essential that all these sources be investigated to determine which is most likely to fulfill the goals of basic research and lead to the development of new medical therapies.

John Hopkins supports the use of the somatic cell nuclear transfer technique (popularly known as "therapeutic cloning" or "research cloning") for the purpose of producing stem cell lines that are geneticaly idjentical to the person from whom the nucleus was obtained. These stem cell lines are critical to help researchers better understand the pathogenesis of disease and provide information useful in developing therapies for people with a wide variety of diseases and injuries. In addition, stem cell lines produced using somatic cell nuclear transfer could overcome the rejection of tissues following transplantation.

However, Johns Hopkins strongly opposes the use of stem cell technology and somatic cell nuclear transfer for the purposes of creating a cloned human being (popularly known as "reproductive cloning").

Stem cell research at John Hopkins is conducted under strict scientific and ethical guidelines that meet all federally mandated requirements. Johns Hopkins has long been a leader in the development of new therapies for patients, and stem cells represents a unique and promising approach in the development of new, critically needed treatments. Research at Johns Hopkins on stem cells is supported by the National Institutes of Health, patient-based organizations, partnerships with corporations, and private philanthropy.

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