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UNITED STATES DISTRICT COURT
FOR THE DISTRICT OF NEW JERSEY

JACOB GUNVALSON, CHERI AND JOHN GUNVALSON, AS GUARDIANS FOR JACOB GUNVALSON, AND CHERI AND JOHN GUNVALSON, INDIVIDUALLY, Plaintiffs, v. PTC THERAPEUTICS, INC., Defendant.	Civil Action No. 08-3559 (WJM) (MF) **DECLARATION OF MARC E. WOLIN** ***Document Filed Electronically***

MARC E. WOLIN, of full age, declares as follows:

1. I am a partner of the law firm of Saiber LLC. Along with co-counsel at Blackwell Burke P.A., we represent Plaintiffs Jacob, Cheri and John Gunvalson in the above-captioned matter.

2. Attached hereto as Exhibit A is a true and correct copy of an excerpt of the Congressional Record – Senate S4115-S4116, dated May 1, 2001 – containing the Statement of

Senator Paul Wellstone regarding the Muscular Dystrophy Community Assistance, Research and Education Act of 2001.

3. Attached hereto as Exhibit B is a true and correct copy of an excerpt of the Congressional Record – House H5959, dated September 24, 2001 – containing the Statement of Representative Colin Peterson regarding H.R. 717, the Muscular Dystrophy Community Assistance, Research and Education Act.

4. Attached hereto as Exhibit C is a true and correct copy of an excerpt of the Congressional Record – Senate S11329-30, dated September 10, 2003 – containing the Statements of Senators Tom Harkin and Alan Specter concerning an amendment to rename the NIH Muscular Dystrophy Research Center program in honor of the late Senator Paul Wellstone, which was then enacted.

5. Attached hereto as Exhibit D is a true and correct copy of a Statement by Robert J. Temple, M.D., Associate Director for the Center for Drug Evaluation and Research, Food and Drug Administration, before the House of Representatives Committee on Government Reform on June 20, 2001. Dr. Temple's statement sets forth FDA policy on compassionate use exceptions for treatment with investigational new drugs.

6. Attached hereto as Exhibit E is a April 4, 2006 press release from PTC announcing Interim Phase 2 Results of one of the PTC124 trials for patients with Cystic Fibrosis. This was printed from PTC's Internet webs site. In the release, PTC states:

> PTC124 was generally well tolerated among the 15 patients included in the interim analysis. All adverse events that were potentially drug-related were mild in severity; there were no safety concerns identified in patients' physical examinations, vital sign measurements, or electrocardiograms; and no meaningful changes in laboratory safety parameters were observed. There were no dosing interruptions or trial discontinuations due to toxicity.

7. Attached hereto as Exhibit F is an October 5, 2007 press release from PTC announcing additional Phase 2 Results from a PTC124 trial for pediatric patients with Cystic Fibrosis. This was printed from PTC's Internet webs site. In the release, PTC states:

> "We are very pleased to see the evidence of drug activity reported at last year's North American Cystic Fibrosis Conference reproduced by additional investigators in a pediatric population," said Langdon Miller, M.D., Chief Medical Officer of PTC. "We are also encouraged by the findings of the Israeli three-month study. We believe these confirmatory results, coupled with supportive safety data in more than 50 patients participating in the Phase 2 trial program, can lead to the initiation of longer-term trials to evaluate the clinical benefit of PTC124 in patients with CF."

8. Attached hereto as Exhibit G is an October 11, 2007 press release from PTC announcing Pharmacokinetic and Safety Results from a Phase 2 study of PTC124 for patients with DMD. This was printed from PTC's Internet webs site. In the release, PTC states:

> The results, which include data from the third and final cohort of the study, show that treatment with PTC124 appeared well tolerated at all three dose levels . . . The analysis presented today showed that PTC124 appeared well tolerated among the 38 boys included in the study. Adverse events were infrequent, mild to moderate in severity, and did not result in therapy interruptions of discontinuations. There were no concerns based on physical examinations, vital sign measurements, electrocardiograms or laboratory parameters . . . "Based on the findings from this study, we believe that the safety profile of PTC124 supports continued testing in longer-term studies[," said Brenda Wong, M.D., Associate Professor of Pediatrics and Neurology, Cincinnati Children's Hospital Medical Center]
>
> "These results add to the growing body of safety data for PTC124, which has now been evaluated in more than 150 subjects, including patients with both DMD and cystic fibrosis. The safety profile has consistently shown that PTC124 appears well tolerated," said Langdon Miller, M.D., Chief Medical Officer of PTC.

9. Attached hereto as Exhibit H is an October 18, 2007 press release from PTC announcing additional results from a Phase 2 study of PTC124 for patients with DMD. This was printed from PTC's Internet webs site. In the release, PTC states:

> "Coupled with the emerging safety profile of PTC124, these data provide the impetus for moving forward rapidly to initiate longer-term studies for boys with DMD," said Langdon Miller, M.D., Chief Medical Officer of PTC.

10. Attached hereto as Exhibit I is an April 23, 2008 press release from PTC announcing the initiation of registration for the Phase 2b study of PTC124 for patients with DMD or BMD. This was printed from PTC's Internet webs site. In the release, PTC states:

> "We believe that the safety profile of PTC124 and activity we have seen in the Phase 2a studies clearly support the initiation of this longer-term, registration-directed efficacy and safety study. We are very pleased to be part of this groundbreaking trial[," said Brenda Wong, M.D.]

11. Attached hereto as Exhibit J is a June 12, 2008 press release from PTC announcing data from additional clinical studies of PTC124 for patients with Cystic Fibrosis. This was printed from PTC's Internet webs site. In the release, PTC states:

> The Phase 2a extension study in Israel assessed 3 months of oral PTC124 therapy at two different dosage levels . . . PTC124 was generally well tolerated, resulting in excellent mean compliance with the treatment regimen.

The release also confirmed that PTC124 was "generally well tolerated" in pediatric populations in a different Phase 2a study in France and Belgium.

I certify that the foregoing statements made by me are true. I am aware that if any of the foregoing statements made by me are willfully false, I am subject to punishment.

/s/ Marc E. Wolin
MARC E. WOLIN

Dated: August 15, 2008

EXHIBIT A

Currently, it can be very challenging for most Americans to find the information they need about their government. For example, if someone was looking for information on an issue pertaining to international trade, he or she would have to look at the web sites of the Department of Commerce, United States Trade Representative, International Trade Commission, possibly the Department of State or Agriculture, and a myriad of House and Senate Committees to find the information they seek. This process will undoubtedly frustrate the average American, and reinforce feelings of a remote, confusing government. Today, less than one percent of current interactions between government and citizens are online. There is clearly need for improvement.

This legislation will help create a coordinated government electronic policy. By establishing a Federal Chief Information Officer to operate within the Office of Management and Budget, the federal government will use staff and resources more effectively to promote e-government and address the nation's other pressing information policy issues. In addition, the bill establishes an Interagency Information Technology Fund to break down existing bureaucratic barriers, and set up a "one-stop shopping" portal that will make it easier for the public to access information. Finally, the bill will task the Office of Personnel Management to respond to the shortage of skilled Information Technology professionals in the federal government.

This bill is not simple, and I realize that some issues it raises must still be resolved. I believe that the Administration and relevant Congressional oversight committees must be involved in this process. I know that my colleague, the Chairman of the Government Affairs Committee, Senator THOMPSON, will examine this issue, and I would like to work with him to resolve any issues that he, or any other Member, may have with this legislation.

In conclusion, I urge my colleagues to support this legislation. It is important that we seriously examine how to use the Internet and other electronic commerce processes to make the federal government more open to public scrutiny.

By Mrs. FEINSTEIN (for herself, Ms. SNOWE, Mr. SCHUMER, Ms. COLLINS, and Mr. REED):

S. 804. A bill to amend title 49, United States Code, to require phased increases in the fuel efficiency standards applicable to light trucks; to require fuel economy standards for automobiles up to 10,000 pounds gross vehicle weight; to raise the fuel economy of the Federal fleet of vehicles, and for other purposes; to the Committee on Commerce, Science, and Transportation.

Mrs. FEINSTEIN. Mr. President, I am very pleased today to be joined by Senator OLYMPIA SNOWE to introduce this important legislation to gradually phase-in the fuel efficiency standards for SUVs and light duty trucks by 2007.

I would also like to thank the other cosponsors: Senators CHARLES SCHUMER, SUSAN COLLINS and JACK REED.

Put simply, this is the single most effective action we can take to limit our reliance on foreign oil, save consumers at the pump, and reduce global warming.

Today, the U.S. has 4 percent of the world's population, yet we use 25 percent of the planet's energy.

So as the world's largest energy consumer, I believe it is our responsibility to make every effort to be the world's leader in conservation.

Specifically, the results of this bill would be substantial. It would: Save America one million barrels of oil a day; reduce oil imports by 10 percent; and prevent 240 million tons of carbon dioxide emissions from entering the atmosphere—this is the single biggest cause of Global Warming.

Today, the fuel economy standard for passenger vehicles is 27.5 miles per gallon, while the standard for SUVs and light duty trucks is 20.7 miles per gallon due to a loophole in the 1975 law.

The result: SUVs and light trucks now comprise nearly half of new car sales, bringing the average fuel economy of all the nation's new vehicles to its lowest point since 1980.

The Feinstein-Snowe legislation would: Phase in fuel economy standards for SUVs and all other light duty trucks on the following schedule: By 2002, SUVs and light duty vehicles must average 22.5 miles per gallon; by 2005, SUVs and light duty vehicles must average 25 miles per gallon; and by 2007, SUVs and light duty vehicles must average 27.5 miles per gallon; require that vehicles up to a weight of 10,000 pounds must qualify for fuel efficiency standards by 2007. The current limit is 8,500 pounds; increase the fuel economy of new vehicles comprising the federal government fleet by 6 miles per gallon by 2005.

Last year, former Senators Slade Gorton, Richard Bryan and I fought an uphill battle to try and find a way to increase these fuel economy standards.

But, we were stymied by the auto industry and their supporters in Congress.

Ultimately, at the end of the session, we reached an agreement that directed the National Academy of Sciences to study whether, in fact, we could raise fuel efficiency with sacrificing safety or competitiveness.

Recently, the automakers have said that they will not actively oppose increases in fuel efficiency standards.

The Big Three manufacturers have promised a voluntary increase in efficiency for SUVs by 25 percent by 2005.

This is an important step forward, but we need to do more. I believe this bill is the best way to do that.

By Mr. WELLSTONE (for himself, Mr. COCHRAN, Ms. COLLINS, Mr. BENNETT, Mr. BREAUX, Mr. BUNNING, Mrs. CLINTON, Mr. CORZINE, Mr. DASCHLE, Mr. DAYTON, Mr. DORGAN, Mr. HUTCHINSON, Mr. JOHNSON, Mr. KERRY, Mr. KOHL, Ms. MIKULSKI, Mr. SARBANES, Mr. SCHUMER, Ms. SNOWE, Ms. STABENOW, and Mr. VOINOVICH):

S. 805. A bill to amend the Public Health Service Act to provide for research with respect to various forms of muscular dystrophy, including Duchenne, Becker, limb girdle, congenital, facioscapulohumeral, myotonic, oculopharyngeal, distal, and emery-dreifuss muscular dystrophies; to the Committee on Health, Education, Labor, and Pensions.

Mr. WELLSTONE. Mr. President, this is the Muscular Dystrophy Community Assistance, Research And Education Act of 2001. It really is the MD CARE Act. I thank Senators COCHRAN and COLLINS, especially, for their assistance. There are 20 colleagues who support this legislation. It is about equally divided between Democrats and Republicans, thank God, because of what this piece of legislation is about.

To look at the record of research on these debilitating and deadly diseases is to realize that despite our country's enormous resources, sometimes people are left behind. Today, despite all the advances in medical science, victims of muscular dystrophy—which afflicts tens of thousands of individuals every year in America—have no cure and no effective treatments available to them.

I became engaged with the muscular dystrophy community when I was approached by several families in my home state of Minnesota with children suffering from Duchenne's muscular dystrophy (DMD). DMD is the most prevalent form of muscular dystrophy affecting children and it is the most deadly.

Children with DMD are most often not diagnosed before the age of two or three years. Because it is sex-linked, the disease only strikes boys but in reality, it strikes the entire family.

DMD children don't begin to walk until late, and then in an unusual manner. They frequently fall and have difficulty getting up. Climbing stairs is a major ordeal.

By age 9 these children start to rely on a wheelchair and by their teen years reliance becomes total.

Most tragically, the disease is characterized by a continued rapidly progressive muscle weakness that almost always results in death by 20 years of age.

I have three children, ages 36, 31, and 28. I cannot imagine this.

Children afflicted with Duchenne Muscular Dystrophy have no ability to produce the protein dystrophin, the protein that binds the muscle cells together. It is an exceptionally cruel disease that slowly robs boys of their independence and ultimately immobilizes them, leading invariably to an early loss of life.

Sadly, the federal response to this disease has been inadequate. This year, in an NIH budget of more than $18 billion, research into Duchenne and Becker Muscular Dystrophies totals just $9.2 million. Only $17 million was devoted last year to all of the muscular dystrophies combined. If you want to understand why there is nothing available to treat DMD children, you need look no further than the weak federal response to this disease. The gene that is flawed in this disease is readily identifiable, and has been so for 14 years. Astonishingly, however, the pace of research on DMD actually slowed down after the gene was discovered.

One DMD child back in Minnesota that I have become especially fond of is Jacob Gunvalsen. Jacob is an adorable 10-year-old. He loves to play with his siblings out on his parents' farm, draw pictures for his family's refrigerator and play video games. Jacob and his mother Cheri Gunvalsen have made quite an impression on several members of Congress, and Jacob's picture adorns the desks of numerous health care legislative staff throughout Washington. This is because like so many other parents facing the day-to-day experience of living with a child suffering from this debilitating disease, Cheri is focused on leaving no stone unturned in her quest to help improve her son's chance of survival. One day, Jacob drew a picture of himself, and in a cloud above his figure he wrote the words, "What I want most in the world is a cure for Duchenne Muscular Dystrophy". I say to my colleagues, that's what I want, too. Today, we are getting one step closer to making Jacob's wish come true.

David Mesick, also of Minnesota, is the Chairman of the Parent Project Muscular Dystrophy, a national voluntary health organization committed to promoting medical research efforts specific to Duchenne and Becker muscular dystrophies. Through David's leadership and the organization's efforts, the muscular dystrophy community has successfully increased Congress' awareness of this devastating disease. Today, their voices are being heard here on the floor of the Senate. I have been moved by the number of families in Minnesota and elsewhere who have been affected by this disease, and I have been moved even more by their tenacious response. We can support this community by improving federal research efforts and public programs to address the needs of individuals with muscular dystrophy.

Mr. President, passage of this legislation will improve coordination of research not only into Duchenne's, but into all the various forms of Muscular Dystrophy. It authorizes the National Institutes of Health (NIH) and the Centers for Disease Control and Prevention (CDC) to establish separate Centers of Excellence to promote basic and clinical research, epidemiology, data collection and assessment on the various forms of muscular dystrophy. These steps are needed to ensure a long-term commitment by the federal government to the treatment and cure of muscular dystrophy.

I am neither a scientist nor a physician. But I am told that it is highly probable that sooner or later gene therapy will be able to cure diseases of this nature. For diseases like Duchenne's muscular dystrophy, involving flaws on a single, identifiable gene, the outlook is even more positive. Yet the words 'sooner' and 'later' have profound consequences in the lives of tens of thousands of Americans and their families. With the introduction of the MD CARE Act, we move a step closer to giving those families hope. I encourage my colleagues on the Senate HELP Committee to work steadfastly to move this crucial legislation through the Senate, and I urge all colleagues to support it.

I also think of Eric Anderson who is such a good friend of my son. David and Eric came to Washington. So many of the families who came, and many came with their children, were so young and their children were so young. Time is not neutral for them. There is an excellent chance we can make a real breakthrough in finding a cure. It is not too much that these families ask for and it is not too much to pass this legislation and try and push forward a commitment to the funding, a commitment to this research.

This is one of those diseases. I hate to label, so few are affected, but for these children and these families, they are not too few in number. These are their lives. These are their hopes. These are their dreams. This is their pain. This is their agony. I want to turn this into hope. I ask all of my colleagues to support this legislation.

I am very pleased this has strong bipartisan support.

By Mr. HUTCHINSON:

S. 806. A bill to guarantee the right of individuals to receive full social security benefits under title II of the Social Security Act with an accurate annual cost-of-living adjustment; to the Committee on Finance.

Mr. HUTCHINSON. Mr. President, I ask unanimous consent that the text of Full Social Security Benefits Guarantee Act be printed in the RECORD.

There being no objection, the bill was ordered to be printed in the RECORD, as follows:

S. 806

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the "Full Social Security Benefits Guarantee Act".

SEC. 2. GUARANTEE OF FULL SOCIAL SECURITY BENEFITS WITH ACCURATE ANNUAL COST-OF-LIVING ADJUSTMENT.

(a) IN GENERAL.—Not later than 90 days after the date of enactment of this Act, the Secretary of the Treasury shall issue to each individual who, as of such date, is receiving benefits under title II of the Social Security Act (42 U.S.C. 401 et seq.) and, thereafter, to each individual who applies for such benefits, a certificate representing a legally enforceable guarantee—

(1) of the monthly amount of benefits that the individual will receive under that title, as determined on the date of the issuance of the certificate; and

(2) that the benefits will be adjusted—

(A) not less frequently than annually on the basis of an accurate determination of the increase in the cost-of-living of the individual; and

(B) as a result in a change in the eligibility status of the individual under that title.

(b) ENTITLEMENT.—Any certificate issued under the authority of this section constitutes budget authority in advance of appropriations Acts and represents the obligation of the Federal Government to provide for the payment to the individual to whom the certificate is issued benefits under title II of the Social Security Act (42 U.S.C. 401 et seq.) in the amounts set forth in the certificate and adjusted thereafter as described in subsection (a)(2).

By Mr. CORZINE:

S. 807. A bill to promote youth financial education; to the Committee on Health, Education, Labor, and Pensions.

Mr. CORZINE, Mr. President, today I am introducing the Youth Financial Literacy Act to address an important issue in education: teaching students the basic principles of financial literacy.

Unfortunately, when it comes to personal finances, young Americans do not have the skills they need. Too few understand the details of managing a checking account, for example, or using a credit card. It is time to make sure that our education system teaches our children all the skills they need, including the fundamental principles involved with earning, spending, saving and investing, so that they can manage their own money and succeed in our society.

We have just finished tax season, and a recent survey by the non-profit JumpStart Coalition reveals that the average high school student knows very little about how taxes will affect her take-home pay. The study also found that, on average, only 36 percent of surveyed high school students could correctly answer basic personal finance questions, and only 33 percent of students believed that financial issues strongly impacted their daily lives.

Young people today face an exceedingly complex financial system that is laced with pitfalls. Credit card companies lure naive college students, encouraging them to spend liberally. Music companies offer extraordinary deals such as "8 CDs for one penny!" and then trap customers into purchasing unwanted music every month. Many of our children are simply unaware of the dangers of these kinds of offers.

We also must make sure that the next generation is prepared to deal with the challenges they will find in the workplace. Rather than providing specific benefits, many companies are now encouraging employees to buy

EXHIBIT B

This legislation has strong bipartisan support. It has 310 cosponsors and was unanimously approved by both by the Health Subcommittee and the full Energy and Commerce Committee.

I call on my colleagues to join me in supporting this legislation. What we are doing here this evening is giving hope to Don and Joyce and Ben Carpenter and many others who suffer from Duchenne and other devastating forms of muscular dystrophy in this nation and across the world. We can work miracles when we really try.

Mr. PETERSON of Minnesota. Mr. Speaker, I rise today in support of H.R. 717, the Muscular Dystrophy Community Assistance, Research and Education Act.

Representative WICKER and I introduced H.R. 717, after being inspired by testimonies from our constituents. I am inspired by an extraordinary 9-year-old boy, Jacob, who has Duchenne Muscular Dystrophy.

For those of you who don't know about Duchenne Muscular Dystrophy: Duchenne is typically diagnosed in boys between the ages of 3 and 5 years; the disease is characterized by progressive weakness, with a gradual deterioration of muscle capacity, first in the legs, then in the arms, back, lungs, and heart; and children affected by Duchenne typically do not live to see their 20's

Currently, Jacob uses a motorized scooter to get around, but soon he will need a ventilator to breathe. There is no treatment for Duchenne Muscular Dystrophy. The life expectancy of a child with Duchenne has not changed since 1859 when it was first identified. It is time for us to focus our efforts and target funds to Muscular Dystrophy research at NIH and CDC.

H.R. 717, will fight childhood muscular dystrophy by boosting research funding and raising public awareness. Less than 1/2000 of the NIH budget is focused on research linked to Muscular Dystrophy. Time is running out.

I asked Jacob, if he could trade places with anyone in the world who would he be; I expected him to say a famous athlete or movie star, but he simply answered his older brother, so he can play football with his friends. You see his biggest wish is to be a regular boy.

Today, lets do what we can to help this little boy grow up to play football with his friends. I hope all of you are as inspired as I am by the courage of Jacob and other children who suffer from this, terrible disease.

I urge you to support H.R. 717.

Mr. STRICKLAND. Mr. Speaker, I yield back the balance of my time.

Mr. BILIRAKIS. Mr. Speaker, I have no further requests for time, and I yield back the balance of my time.

The SPEAKER pro tempore (Mr. MILLER of Florida). The question is on the motion offered by the gentleman from Florida (Mr. BILIRAKIS) that the House suspend the rules and pass the bill, H.R. 717, as amended.

The question was taken.

The SPEAKER pro tempore. In the opinion of the Chair, two-thirds of those present have voted in the affirmative.

Mr. BILIRAKIS. Mr. Speaker, on that I demand the yeas and nays.

The yeas and nays were ordered.

The SPEAKER pro tempore. Pursuant to clause 8 of rule XX and the Chair's prior announcement, further proceedings on this motion will be postponed.

MESSAGE FROM THE SENATE

A message from the Senate by Mr. Lundregan, one of its clerks, announced that the Senate has passed without amendment a bill of the House of the following title:

H.R. 2603. An act to implement the agreement establishing a United States-Jordan free trade area.

REPORT ON H.R. 2944, DISTRICT OF COLUMBIA APPROPRIATIONS ACT, 2002

Mr. KNOLLENBERG, from the Committee on Appropriations, submitted a privileged report (Rept. No. 107–216) on the bill (H.R. 2944) making appropriations for the government of the District of Columbia and other activities chargeable in whole or in part against the revenues of said District for the fiscal year ending September 30, 2002, and for other purposes, which was referred to the Union Calendar and ordered to be printed.

The SPEAKER pro tempore. Pursuant to clause 1, rule XXI, all points of order are reserved on the bill.

RECESS

The SPEAKER pro tempore. Pursuant to clause 12 of rule I, the Chair declares the House in recess until 5:30 p.m.

Accordingly (at 4 o'clock and 6 minutes p.m.), the House stood in recess until 5:30 p.m.

□ 1730

AFTER RECESS

The recess having expired, the House was called to order by the Speaker pro tempore (Mr. FOLEY) at 5 o'clock and 30 minutes p.m.

APPOINTMENT OF CONFEREES ON H.R. 2500, DEPARTMENTS OF COMMERCE, JUSTICE, AND STATE, THE JUDICIARY, AND RELATED AGENCIES APPROPRIATIONS ACT, 2002

Mr. WOLF. Mr. Speaker, I ask unanimous consent to take from the Speaker's table the bill (H.R. 2500) making appropriations for the Departments of Commerce, Justice, and State, the Judiciary, and related agencies for the fiscal year ending September 30, 2002, and for other purposes, with a Senate amendment thereto, disagree to the Senate amendment, and agree to the conference asked by the Senate.

The SPEAKER pro tempore. Is there objection to the request of the gentleman from Virginia? The Chair hears none and, without objection, appoints the following conferees: Messrs. WOLF, ROGERS of Kentucky, KOLBE, TAYLOR of North Carolina, REGULA, LATHAM, MILLER of Florida, VITTER, YOUNG of Florida, SERRANO, MOLLOHAN, Ms. ROYBAL-ALLARD, and Messrs. CRAMER, KENNEDY of Rhode Island, and OBEY.

There was no objection.

MAKING IN ORDER AT ANY TIME CONSIDERATION OF H.J. RES. 65, CONTINUING APPROPRIATIONS, FISCAL YEAR 2002

Mr. YOUNG of Florida. Mr. Speaker, I ask unanimous consent that it be in order at any time without intervention of any point of order to consider in the House the joint resolution (H.J. Res. 65) making continuing appropriations for the fiscal year 2002, and for other purposes; that the joint resolution be considered as read for amendment; the joint resolution shall be debatable for 1 hour equally divided and controlled by the chairman and ranking member of the Committee on Appropriations; and the previous question shall be considered as ordered on the joint resolution to final passage without intervening motion except one motion to recommit.

The SPEAKER pro tempore. Is there objection to the request of the gentleman from Florida?

There was no objection.

GENERAL LEAVE

Mr. YOUNG of Florida. Mr. Speaker, I ask unanimous consent that all Members may have 5 legislative days in which to revise and extend their remarks on H.J. Res. 65, and that I may include tabular and extraneous material.

The SPEAKER pro tempore. Is there objection to the request of the gentleman from Florida?

There was no objection.

CONTINUING APPROPRIATIONS, FISCAL YEAR 2002

Mr. YOUNG of Florida. Mr. Speaker, pursuant to the order of the House of today, I call up the joint resolution (H.J. Res. 65) making continuing appropriations for the fiscal year 2002, and for other purposes, and ask for its immediate consideration.

The Clerk read the title of the joint resolution.

The text of House Joint Resolution 65 is as follows:

H.J. RES. 65

Resolved by the Senate and House of Representatives of the United States of America in congress assembled, That the following sums are hereby appropriated, out of any money in the Treasury not otherwise appropriated, and out of applicable corporate or other revenues, receipts, and funds, for the several departments, agencies, corporations, and other organizational units of Government for fiscal year 2002, and for other purposes, namely:

SEC. 101. (a)(1) Such amounts as may be necessary under the authority and conditions provided in the applicable appropriations Act for fiscal year 2001 for continuing

EXHIBIT C

binding. It must issue a new rule if the personal dust monitoring devices prove successful. And I hope that they understand that the miners of our States and their representatives in the Congress will be watching, and that we will continue to oppose any effort to circumvent the 1969 Mine Act with regard to dust levels.

I urge Senators to take this opportunity to improve the credibility of the Mine Safety and Health Administration. I urge Senators to recall the findings of the Congress that were contained in the 1969 Mine Act:

The first priority and concern of all in the coal mining industry must be the health and safety of its most precious resource—the miner.

Mr. KENNEDY. It is a privilege to join Senator BYRD and Senator SPECTER on this amendment to increase health and safety protections for the Nation's coal miners.

Coal mining is difficult and dangerous work. Miners daily face the risk of mine collapse, mine fires, and the debilitating illness called black lung disease. Each year, over 4,000 coal miners lose time at work because of injuries on the job. We all remember the near-tragedy last summer at Quecreek Mine in Pennsylvania, when nine miners were trapped underground for 3 days. Miraculously, they were rescued but many other miners are not so fortunate.

Congress passed the Federal Mine Safety and Health Act in 1969 to protect miners from these hazards. One of the most critical parts of the Mine Safety Act is its requirement that mine operators reduce and control the level of dust that miners inhale during their shifts.

Since then, we have made great progress in reducing the number of cases of black lung, but this battle is far from over. Over 100,000 former miners and their dependents are receiving Federal benefits today because they or their family members have had black lung. Each year, more than 1,000 workers die from the disease—and hundreds of new cases of black lung are reported each year.

This amendment deals with MSHA's proposed regulations on dust levels and dust monitoring. Many of us are deeply troubled by the administration's proposal. The proposed regulation reduces the protections of the Mine Safety Act. It would allow coal mine operators to raise dust levels up to four times the amount now permitted by the act. It would also reduce the number of samples taken in mines to measure coal dust exposure.

It makes no sense to roll back the current protections. Instead of increasing the number of inspections and tightening the dust standard, the administration's regulation would allow coal mines to raise the amount of dust miners are exposed to. The new regulation directly contradicts the recommendations of the National Institute for Occupational Safety and Health, under which the permitted level of coal dust would be cut in half.

Senator BYRD and Senator SPECTER have proposed this amendment to require MSHA to consider incorporating the new Personal Dust Monitor technology, once testing is completed. This amendment is supported by both the coal mining industry and by the United Mineworkers.

These Personal Dust Monitors have been developed in conjunction with labor and industry. They would be worn by individual miners at all times and could measure more accurately than any existing technology how much coal dust each miner is exposed to.

MSHA itself has acknowledged the role of Dust Monitors in miner safety. It has extended the current rulemaking period in order to include comments based upon Dust Monitor testing. This amendment would go one step further, by requiring MSHA to consider reproposing the rule to incorporate Personal Dust Monitors as part of the required safeguards in the Nation's mines.

Incorporating these technological advances into the rules on coal dust monitoring is a very important step. The Nation's miners risk their lives every day to provide critical domestic sources of energy, and we need to do all we can to protect their lives and health.

I strongly urge my colleagues to support this amendment to use all available technologies to protect the Nation's hard-working coal miners.

The PRESIDING OFFICER. The Senator from Iowa is recognized.

AMENDMENT NO. 1645

Mr. HARKIN. Mr. President, I ask the indulgence of the Senate for a brief statement I am going to make.

As we all know, Senator Paul Wellstone, his wife Sheila, and his daughter Marcia, three staff members, and two pilots perished in a tragic plane accident nearly a year ago near Eveleth, MN. The Senate lost an honest, passionate public servant, and we all lost a friend.

Senator Wellstone's life was a testament to his compassion and commitment to serve the less advantaged. He was a tireless advocate for people in need. That was never more true than when he began working with children with Duchenne muscular dystrophy and their families.

In 2001, Senator Wellstone introduced the Muscular Dystrophy Community Assistance, Research, and Education Amendments of 2001 to intensify Federal investment in Duchenne and other forms of muscular dystrophy. The MD CARE Act asked the Director of the National Institutes of Health to create centers of excellence to conduct basic and clinical research into Duchenne and other muscular dystrophies. That bill was signed into law in December of 2001. About a year later, Senator Wellstone was tragically killed.

While we cannot replace the colleague and friend who served with us in this Chamber, we can commemorate his work on behalf of Jacob Gunvalson and others who inspired the late Senator to see this law enacted. In September of this year, the National Institutes of Health will announce the first grantees of its newly created Muscular Dystrophy Cooperative Research Centers Program.

In addition, three NIH institutes—the National Institute of Arthritis and Musculoskeletal Diseases, the National Institute of Neurological Disorders and Stroke, and the National Institute of Child Health and Human Development—also set aside $1 million in the MDCRC program as the "Senator Paul D. Wellstone Muscular Dystrophy Cooperative Research Centers."

I have an amendment that would rename the NIH Muscular Dystrophy Cooperative Research Center program in honor of the late Senator Paul D. Wellstone of Minnesota. I will not read all of it. In part, it says that the designation of the NIH Muscular Dystrophy Cooperative Research Centers program shall be known and designated as the "Senator Paul D. Wellstone Muscular Dystrophy Cooperative Research Centers" in honor of Paul D. Wellstone, who was deceased on October 25, 2002. There is no cost involved.

I send the amendment to the desk and ask for its immediate consideration.

The PRESIDING OFFICER. The clerk will report.

The legislative clerk read as follows:

The Senator from Iowa [Mr. HARKIN] proposes an amendment numbered 1645.

Mr. HARKIN. I ask unanimous consent that further reading of the amendment be dispensed with.

The amendment is as follows:

(Purpose: To rename the NIH Muscular Dystrophy Cooperative Research Center (MDCRC) program in honor of the late Senator Paul D. Wellstone of Minnesota)

Add at the appropriate place:

SECTION 1. DESIGNATION OF SENATOR PAUL D. WELLSTONE NIH MDCRC PROGRAM

(a) FINDINGS.—Congress finds the following:

(1) On December 18, 2001, Public Law 107-84, otherwise known as the Muscular Dystrophy Community Assistance, Research and Education Amendments of 2001, or the MD CARE Act, was signed into law to provide for research and education with respect to various forms of muscular dystrophy, including Dechenne, Becker, limb girdle, congenital, facioscapulohumeral, myotonic, oculopharyngeal, distal, and EmeryDreifuss myscular dystrophies.

(2) In response to the MD CARE Act of 2001, in September 2002, NIH announced its intention to direct $22.5 million over five years to its newly created Muscular Dystrophy Cooperative Research Centers (MDCRC) program.

(3) Senator Paul D. Wellstone was a driving force behind enactment of the MD CARE Act, which led to the establishment of the MDCRC program.

(b) DESIGNATION.—The NIH Muscular Dystrophy Cooperative Research Centers (MDCRC) program shall be known and designated as the "Senator Paul D. Wellstone Muscular Dystrophy Cooperative Research Centers," in honor of Senator Paul D. Wellstone who was deceased on October 25, 2002.

(c) REFERENCES.—Any reference in a law, regulation, document.

The PRESIDING OFFICER. The Senator from Pennsylvania.

Mr. SPECTER. Mr. President, I thank the Senator from Iowa for what he has done. I am delighted to join him in sponsoring the amendment to name the Muscular Dystrophy Operative Research Center after Senator Wellstone. Senator Wellstone attended many of our appropriations subcommittee hearings on neurological disorders and was a tireless advocate for medical research regarding the cause of muscular dystrophy. He was the original sponsor of the Muscular Dystrophy Care Act of 2001. This was the first disease-specific piece of legislation in the 107th Congress, the first major piece of health care legislation signed by President George Bush, and the first piece of legislation to ever address a very lethal childhood disorder.

Just a few weeks before he died, Senator Wellstone visited a little boy named Jacob who was afflicted with muscular dystrophy. He and Jacob made some television ads, which sadly didn't air because of Senator Wellstone's tragic death. Senator Wellstone made a difference in the lives of thousands of children afflicted with this deadly disease. He leaves a legacy of unprecedented Federal commitment to muscular dystrophy research and development. I have no doubt that someday soon we will win the fight against muscular dystrophy because of Paul Wellstone's work.

Mr. President, I yield to my distinguished colleague from Pennsylvania, Senator SANTORUM.

The PRESIDING OFFICER. The Senator from Pennsylvania.

Mr. REID. Will the Senator withhold?

Mr. SANTORUM. I will be happy to withhold.

The PRESIDING OFFICER. Is there further debate on amendment No. 1645? If not, the question is on agreeing to the amendment.

The amendment (No. 1645) was agreed to.

The PRESIDING OFFICER. The Senator from Pennsylvania.

AMENDMENT NO. 1623

Mr. SANTORUM. Mr. President, earlier today, I offered an amendment to increase the amount of money in the Aid for Africa Program to the number that met the authorization level this Senate passed just a couple months ago. It was an additional $350 million which would be needed to do the $2 billion in bilateral aid to which we committed, plus the $400 million we would need to match roughly $700 million-plus that has now been put forward by other countries for the Global Fund.

This bill is, in combination with the foreign operations bill, $350 million short for what I believe is the most important health crisis facing the world and one I would argue is, as far as dollars spent, going to save more lives and improve the quality of life for more people than probably any other single dollar item we are doing in this bill.

I believe this is the highest priority. That is why I was willing to offer an amendment to fund this program fully and to do so with an across-the-board cut. Having seen the success of an across-the-board cut in a previous amendment, I have not been necessarily encouraged by my colleagues to continue this effort.

I thank the Senator from Pennsylvania, my colleague, in sharpening his pencil to come up with enough money to at least meet the President's request on his program, which is an additional $61 million. It is an amendment Senator DEWINE offered earlier for mother-to-child transmission and for non-mother-to-child transmission.

Senator DEWINE has been doing some great work, along, I understand now, with Senator DURBIN, to come up with this money.

In exchange for the acceptance of that amendment by the managers, I am going to withhold my amendment. I yield to the Senator from Ohio to explain what his amendment does.

Mr. DEWINE. I thank my colleague from Pennsylvania. I also thank my other colleague from Pennsylvania, the chairman of the committee, for his good work on this bill and for his willingness to work with us on this very important issue.

I know the hour is late, Mr. President. I am just going to take literally 2 minutes to explain this amendment. I will offer the amendment and then I believe the amendment will be accepted.

This amendment is very simple. What it does is it will restore the money to this bill the President has requested this Congress to provide for a program that literally will save tens of thousands, maybe hundreds of thousands, of lives of innocent children. We have the ability today to see a pregnant mother who is HIV positive and to provide her with the care and the drugs to ensure she will not give birth to a child who is HIV positive.

The statistics are very simple and the facts are very simple. If a woman today is pregnant and is HIV positive, the odds are—the percentage is about 30 percent—she will give birth to a child who is HIV positive. In sub-Saharan Africa or in Haiti or in Guyana, there are programs today that will reduce those odds from 30 percent down to 5 or 10 percent for as little as $3 to $4 per woman. That is not per day. That is per woman per child. It is almost a miracle.

My colleague in the Chair and other Members of the Senate who just came back from a trip, led by Majority Leader BILL FRIST, to Africa saw these programs in place. They work. What this extra $60 million will do is to help ensure there will be tens of thousands of more children who will be born HIV negative.

I thank my chairman for allowing this money to come into the bill.

I call up my amendment on behalf of Senator SANTORUM and Senator DURBIN, who went to bat, as he has many times in the past, for children and those who are HIV positive and who might be HIV positive. I thank Senator DURBIN and Senator SANTORUM. I now call up my amendment No. 1623.

The PRESIDING OFFICER. The clerk will report.

The legislative clerk read as follows:

The Senator from Ohio [Mr. DEWINE], for himself, Mr. SANTORUM, and Mr. DURBIN, proposes an amendment numbered 1623 to amendment No. 1542.

Mr. DEWINE. Mr. President, I ask unanimous consent that the reading of the amendment be dispensed with.

The PRESIDING OFFICER. Without objection, it is so ordered.

The amendment is as follows:

(Purpose: To increase funding for activities to prevent the mother-to-child transmission of HIV)

On page 61, between lines 14 and 15, insert the following:

SEC. ____. (a) MOTHER-TO-CHILD HIV TRANSMISSION PREVENTION.—In addition to any amounts otherwise made available under this Act to carry out mother-to-child HIV transmission prevention activities, there shall be made available an additional $60,000,000 to carry out such activities and $1,000,000 for non-mother-to-child activities.

(b) REDUCTION IN AMOUNTS.—Amounts made available under this Act for the administrative and related expenses for departmental management for the Department of Labor, the Department of Health and Human Services, the Department of Education, shall be reduced on a pro rata basis by $61,000,000.

The PRESIDING OFFICER. The Senator from Pennsylvania.

Mr. SPECTER. Mr. President, I congratulate the Senator from Ohio for his outstanding work. I congratulate my colleague from Pennsylvania for his contribution. The Senator from Ohio only talked to me about this amendment about 79 times during the course of the last 2 days. To say that he was persistent would be a vast understatement.

We are prepared to accept this $61 million for the global AIDS for the CDC, of which $60 million is for the mother-to-child transmission prevention initiative and $1 million is for other global AIDS activities. This offset will be made from the administrative costs of the Departments of Labor, Health and Human Services, and Education.

During the course of the debate, we have had a great many AIDS amendments offered, and we wish we could have accepted more of them. But this particular one is very precisely targeted. I know the Senator from Ohio just came back from Africa and feels very deeply about this issue.

We are pleased to accept this amendment, with our compliments to the Senator from Ohio, Mr. DEWINE, the Senator from Pennsylvania, Mr. SANTORUM, and the Senator from Illinois, Mr. DURBIN.

I yield the floor.

The PRESIDING OFFICER. Is there further debate? The Senator from Iowa.

EXHIBIT D

U.S. Food and Drug Administration

June 20, 2001

STATEMENT

BY

ROBERT J. TEMPLE, M.D.

ASSOCIATE DIRECTOR FOR CENTER FOR DRUG EVALUATION AND RESEARCH

FOOD AND DRUG ADMINSTRATION

BEFORE

THE COMMITTEE ON GOVERNMENT REFORM,

U.S. HOUSE OF REPRESENTATIVES

JUNE 20, 2001

Introduction

Mr. Chairman, Members of the Committee, I am Robert Temple, M.D., Associate Director for Medical Policy, Center for Drug Evaluation and Research (CDER), Food and Drug Administration (FDA or the Agency). Also with me today is Ms. Patricia Delaney from FDA's Office of Special Health Issues (OSHI), Cancer Liaison Program.

Background

FDA would like to thank the Chairman for drawing attention to the important issue of the availability of investigational drugs for what is commonly referred to as compassionate use. First, let me say that while the phrase "compassionate use" is commonly used to describe some of the ways of making unapproved products available to patients, there is no FDA regulation or policy defining a "compassionate use." Compassion, an intent to help, should be, and is, an element of all drug investigation activities. In general, we describe these uses of drugs as "treatment uses," because their intent is to provide treatment of patients, not primarily to evaluate the safety and effectiveness of the drugs, the primary and usual purpose of studies under an Investigational New Drug Application (IND). FDA refers to compassionate use requests for individual patients as a "single patient IND" study, wider use would usually take place under a "treatment IND" or "treatment protocol" under an existing commercial IND.

We are very much aware of the impact FDA's processes and decisions have on the public we serve. Under the Federal Food, Drug, and Cosmetic (FD&C) Act and related statutes, the Government has a

vitally important role in helping to ensure that the marketed medical products upon which patients and their health care practitioners rely are shown to be both safe and effective. Just as important, we have critical responsibilities in helping to ensure that the use of investigational drugs is carried out safely, and that the limitations of current information on the drug is conveyed to the patient. We are particularly aware that even before a drug is approved for marketing, there may be enough information to support varying degrees of treatment use for people with serious illness when there is no effective treatment available. In various ways, FDA has attempted to make it possible for investigational drugs to be available in these situations, but availability must bear a relation to how much information we have. The safeguards provided by FDA's activities are particularly important for our most vulnerable citizens, those who are seriously ill.

We understand that patients and their family members are often unfamiliar with FDA's legal and regulatory responsibilities. Often they are unaware that FDA cannot compel a company to supply an individual patient with an investigational drug outside of its planned clinical trials. The manufacturer or sponsor makes the final decision to provide an experimental drug or therapy to a patient. The sponsor may consider many factors, including the amount of information available about the drug, the amount of drug available, and how best to use its resources to optimize development of the drug for marketing. This maximizes the availability of the drug to patients who can benefit from it. In some cases, the sponsor is unwilling to provide the product outside of clinical trials, especially relatively early in drug development. Patients are sometimes confused or angered by this situation and misinterpret the company's unwillingness to provide the product as an FDA action.

FDA may not allow treatment uses because of safety concerns. Generally, however, if a physician makes a request for treatment use of an experimental drug, in a patient for whom no effective therapy exists, and there is an ongoing study of the drug and a sponsor agrees to provide the product, FDA does not object to the treatment use.

There have been cases in which treatment use has been considered appropriate, despite relatively little evidence supporting the usefulness of the drug for the particular indication. Generally, when there was no effective alternative drug or treatment for the particular condition and there was sufficient information about safety, treatment use be justified. Physicians may always contact FDA to propose such a use for a specific patient when they believe circumstances warrant this use.

It is apparent that many manufacturers of promising drugs do not have standard operating procedures in place for handling requests for single patient INDs, especially when the promise of the drug is just becoming appreciated. This has created confusion and, in some cases, led to perceptions of an unfair system, in which some people can gain access to therapies while others, who appear similarly situated, cannot. The patients seeking these drugs are frequently cancer patients who have exhausted standard treatment. They, and their relatives, are often desperately seeking a last chance to prolong their lives. Any impediment to obtaining the drugs would be most unwelcome. Actual or perceived unfairness would seem intolerable.

Recent FDA Activities on Access

FDA is generally satisfied with how the current system of access to INDs and single patient INDs is working, but there are problems and some inherent limitations of the system. We realize that the experience of some individuals has not been satisfactory and has seemed unfair and there is a public perception of a very convoluted system to gain access to these drugs.

Let me bring you up to date on some recent FDA activities that have addressed issues of access to

experimental drugs and single patient INDs.

At the December 13 and 14, 2000, and the June 7, 2001, Oncologic Drugs Advisory Committee (ODAC) meetings, FDA solicited advice from the committee on when it is appropriate for FDA to allow investigational drugs to be used for treatment of individual cancer patients. An important additional objective of the meeting was to educate the public, physicians, and ODAC on the issues surrounding access to investigational cancer drugs for single patient treatment use.

The individual presentations and discussions at the meeting were wide-ranging, very thoughtful and, it is fair to say, somewhat surprising. These were patients or relatives of patients who spoke feelingly of their difficulties and frustrations in seeking potentially useful treatment for cancers that had not responded to other therapy. A number of patient groups spoke with equal feeling on the need to develop treatment rationally, to defer treatment use until adequate information supports it (and only then to make it available) and to correct the widespread misimpression that there are magic bullets available for treatment of refractory malignancies. They also emphasized the need for widely available information about drugs under study and those drugs for which more widespread availability was appropriate and available. It was clear that the overall situation was one of great complexity, but these groups did not believe that wide use of toxic drugs without known benefit was a service to seriously ill patients.

Industry Concerns About Treatment use of Investigational Drugs

Commercial sponsors are not always willing to supply drug for treatment uses. A number of industry concerns about the use of experimental drugs were discussed at the June 7, 2001, ODAC meeting.

(1) There may be a limited drug supply early in drug development. The batches prepared for early drug studies are usually small; making larger amounts available is expensive and not considered reasonable until there begins to be evidence that the drug is of value.

(2) There may be competition between expanded access programs and the regulatory programs that will lead to drug approval. Competition can be either for patients entering trials or for internal company resources. The process of individualized packing and shipping of drugs for single patient use on an emergent basis can be very disruptive to departments that are organized to pack and ship drugs in a scheduled manner for clinical trials. There is significant concern that availability of all investigational drugs outside a formal protocol will decrease participation in the formal study. In fact, FDA rules allow open studies to be put on hold if they are interfering with the conduct of clinical trials

(3)The use of an investigational drug in less controlled setting, in patients with very advanced disease could lead to adverse reactions that might raise difficult to resolve but spurious safety concerns about the drug.

(4)Industry seems to learn little about a drug from single patient use. FDA expects very low response rates in patients who have received multiple previous therapies and a low rate in such patients would not damage the drug's chance for approval.

At the conclusion of the meeting, it was obvious that this issue deserved further discussion and exchange of views. FDA has suggested that a consensus development meeting be convened in the near future, involving industry, academia, patient advocacy groups and regulators to discuss these issues. FDA and the National Cancer Institute could play an important role in organizing and facilitating the conference. We will be glad to provide the committee with the written statements offered at the ODAC meeting and

a transcript of the June 7, 2001, discussions.

Current Access Procedures

We would like to clarify FDA's role in making INDs available for treatment uses. First, to put the subject into context, I would like to briefly address the public health system of getting unapproved drugs to patients.

Clinical Trials

FDA's primary obligations are those vested in us by Congress in the FD&C Act and the Public Health Service Act, that ensure that marketed medical products are, safe, effective, properly labeled and that experimental drug studies are designed to protect the patient volunteers.

Before being approved by FDA for marketing, new drugs and biological products must be proven effective in controlled clinical trials and shown to be safe. FDA is directed, under the FD&C Act, to rely on evidence of effectiveness based upon adequate and well-controlled studies. The persons who participate in any trials under an IND must be fully informed of the risks and possible benefits of their participation and studies must be designed to adequately protect the patients from harm. Patients must be informed about alternative medical treatments, whether approved or investigational. This is possible only when there is adequate pre-clinical data from animal studies or from other sources to provide the information upon which informed consent can be based.

Access to a Clinical Trial

The access process starts with a drug sponsor, a pharmaceutical company or a research scientist at a university or at the National Institutes of Health (NIH), seeking to develop a new drug. Before clinical testing begins, researchers analyze the drug's main physical and chemical properties in the laboratory and study its pharmacologic and toxic effects in laboratory animals (pre-clinical studies). If the laboratory and animal study results show promise, the sponsor must submit an IND to FDA for review before beginning a trial in people.

After a study passes FDA review and a local Institutional Review Board (IRB) (a panel of scientists and non-scientists that oversees clinical research) approves the protocol for clinical trials, experienced clinical investigators give the drug to a small number of patients. These Phase I studies are designed to assess the most common acute adverse effects and examine the amount of drug that patients can take safely without unacceptable side effects. It is unusual in this setting to see important patient benefits. Initial clinical studies are also designed to better understand what happens to a drug in the human body, how it is changed (metabolized), how much of it (or a metabolite) gets into the blood and various organs, how long it stays in the body, and how the body gets rid of the drug and its effects.

If Phase I studies do not reveal major problems, such as unacceptable toxicity, Phase II studies are conducted to determine the effectiveness of the drug in patients who have the medical condition that is intended to be affected by the drug. Researchers then assess whether the drug has a favorable effect, for example, in cancer patients by seeing whether the tumor size is reduced.

In some cases the Phase II studies reveal results so impressive that these studies alone are the basis for approval, generally for treatment of refractory disease. This is often done under FDA's accelerated approval rule (similar to the fast-track provision under FDA's Modernization Act of 1997 [FDAMA]) which allows FDA to approve drugs on the basis of a surrogate endpoint (effect on a measurement such

as a tumor size likely to lead to a real patient improvement) on condition that post-marketing studies demonstrate a tangible patient benefit. In most cases, if Phase II studies show desirable responses, Phase III studies are conducted. Those are concurrently controlled studies in which two therapies are compared. These usually are 1) a comparison of standard treatment alone, or 2) a comparison of the new treatment alone with an older treatment to show that the new treatment is not worse than the older treatment or is its superior.

It is generally believed that it would be in everyone's interest if more patients participated in trials of new cancer treatments. We recommend that anyone interested in participating in a clinical trial discuss the idea with his or her physician. Doctors are generally aware of investigational drugs that might be of benefit to their patients and of clinical trials involving these drugs. Detailed information can be obtained from a variety of sources, including drug sponsors, FDA (if the information is public), a new website www.clinicaltrials.gov , and NIH. Clinical trials are carried out at major medical research centers such as teaching hospitals, at NIH, and even in doctors' offices. Although they often involve hospitalized patients, many clinical trials can be conducted on an outpatient basis, with participants more or less going about their normal activities. The center or institution where a study is to be carried out often runs newspaper ads recruiting potential participants for clinical studies that tell readers where to call or write for further information.

The full implications of taking part in a clinical trial must be fully explained to potential patient subjects in advance by the people conducting the trial and patients must agree to the conditions before they can participate. The hope of personally benefiting from a new drug, or the desire to take part in research that might one-day benefit millions, is what makes people volunteer for clinical trials. These hopes and desires should not prevent them, however, from finding out all they can about being a part of the process. Many seemingly promising agents prove not to be helpful or too toxic to use and, especially in early trials, major benefits are clearly the exception.

Protocol Exception/Exemptions

In cases where a patient cannot be enrolled in an existing protocol because of some factor that makes the patient ineligible to participate in the study, research sponsors or investigators often can make a protocol exception to treat such a patient. The data from that patient would not be part of the report of the original study. Usually such special exceptions arise in the same institutions that are conducting the original study, where investigators are familiar with the drug.

Access to Investigational New Products

The ideal way for a patient to receive a promising but unproven drug is as a participant in a controlled clinical trial. Such trials provide appropriate patient protections and potential benefits (for example, IRB review, informed consent, free product or treatment, and FDA review of pre-clinical data and the protocols for the clinical trials) and maximize the gathering of useful information about the product, potentially benefiting the entire patient population. It is not possible, however, for all patients who might benefit from the drug to enroll in controlled clinical trials.

FDA believes that it is appropriate to make certain promising, but not yet approved, products available to patients with serious and life-threatening illnesses who lack alternative treatment. This should be done in a way that does not interfere with recruitment to the clinical trials needed to support the effectiveness and safety of the drug. It should also be done fairly. A major goal of the treatment IND proposed in 1982, and made final in 1987, was to make unapproved but promising drugs with appropriate evidence of effectiveness widely available prior to marketing. In the past such drugs often had been available but

only at selected sites.

Single Patient INDs

The paperwork reporting responsibilities a sponsor must submit for a single patient IND or single patient use under an existing IND is modest. If a patient is treated under an existing IND, the sponsor must collect and report adverse reactions and include such events in its annual reports. A single investigator wanting to treat a patient will refer to the commercial IND for most information and will have to provide additional information about the patient to be treated to obtain informed consent and local IRB approval. Exactly what to do is described in the oncology part of FDA's website and the Agency's role in the process www.fda.gov/cder/cancer/singleIND.htm.

FDA's role in the process

One may ask why FDA is involved in this process at all. That is, why should not the physician and patient decide on the appropriateness of treatment? We believe that the independent scientific consideration provided by the Agency is critical and is an essential component of patient protection, when one is considering drugs about which relatively little is often known and especially when potential toxicity is great. In the typical single patient IND situation, especially those involving emergency IND requests, the patient's physician generally has only very limited information about the investigational therapy being requested.

FDA has set up internal procedures to facilitate single patient IND requests. Physicians are put in touch with a Consumer Safety Officer (CSO) within the relevant reviewing division; the CSO helps the physician understand the IND process to facilitate completion of the IND application.

Progress Since FDAMA

Section 402 of FDAMA codified certain FDA regulations and practices regarding expanded patient access to experimental drugs and devices. FDAMA addresses three expanded access procedures with respect to: 1) emergency situations; 2) individual patient access to investigational products intended for serious diseases; and 3) treatment IND applications and treatment investigational device exemptions (IDE). The Agency continues to review current regulations and practices in light of FDAMA and is currently developing new regulations to codify current practices. FDAMA continues to emphasize for all those cases, including individual uses that the appropriateness of expanded access depends on available data, i.e., "sufficient evidence of safety and effectiveness to support the [proposed] use."

For the past four years, Agency efforts have included: 1) expediting approval of cancer therapies; 2) encouraging new uses of marketed products in cancer treatment; 3) expanding access to investigational cancer therapies that have been approved in other countries; and 4) appointing cancer patients to our Oncologic Drug Advisory Committee, which reviews new cancer therapies.

Expediting Development, Review, and Approval of New Products

An important means of providing access to new cancer therapy is the rapid development and approval of new agents. FDA has implemented mechanisms designed to increase access to new drugs and biologics by expediting their development, review and approval. All of these programs have been instrumental in shortening the time to marketing approval for cancer drugs and biologics. FDA programs include:

- Expedited development under Title 21, Code of Federal Regulations (CFR) Part 312, Subpart E

expedites the development, evaluation, and marketing of new therapies intended to treat persons with life-threatening and severely debilitating illnesses.

- Priority Review to speed the review of NDAs, biologics license applications (BLAs), and effectiveness supplements that could have important therapeutic impacts. The standard review time of ten months. Since 1996, five biologics and 31 drugs (20 NDAs and 11 supplements) for cancer therapies have received priority review and approval.

- Fast Track section 112 of FDAMA, amends the FD&C Act to consolidate the various provisions intended to facilitate the investigational development and approval of drugs and biologics that provide significant advances in the treatment of serious diseases. This codified FDA's accelerated approval regulations. Perhaps the most important, yet under appreciated aspect of fast track is FDA's commitment to work closely with sponsors throughout the drug development process to agree on study designs and appropriate outcome measures, etc. This allows companies to plan and carry out the most rapid possible responsible development. FDA meets constantly with sponsors taking advantage of this opportunity.

FDA's overall goal is to improve significantly patient access to promising cancer treatments and treatments for other life-threatening illnesses without compromising patient safety. When we do this we seek optimal development and use pre-marketing access when this is safe and appropriate. Importantly, FDA regulations emphasize safeguards for the protection of human subjects, including the requirement for informed consent.

The Office of Special Health Issues

FDA is mindful of the frustrations that patients with life threatening illnesses and their families experience when trying to obtain information about potentially helpful therapies, especially when there is no standard therapy. In addition to offices within FDA's Center for Biologics Evaluation and Research and CDER that routinely provide assistance and information to consumers, OSHI provides information and works with cancer patients and their advocates on cancer-related issues. Most activity in OSHI is on behalf of patients with life threatening diseases, most often cancer and AIDS.

Usually, callers want information about treatments currently being researched. Although we cannot disclose proprietary information about products under development, we are able to talk with patients about any treatment that appears in public access databases.

In response to Section 113 of FDAMA, FDA has worked with NIH to develop a data bank of clinical trials of therapies for serious or life-threatening diseases. The data bank, www.clinicaltrials.gov, currently lists over 5000 trials sponsored by NIH, other Federal agencies, universities, and the pharmaceutical industry. We anticipate that many more industry trials will be included after the final guidance document is available.

Our goals in serving patients with life-threatening diseases and their family members are straightforward:

(1) Promptness (returning patients' and family members' calls within 24 hours);

(2) Accessibility (listening to the caller's concerns and giving him or her as much time as he or she needs);

(3) Education (about the drug approval process and his or her options); and

(4) Assistance (providing additional information to the patient or family member that may be helpful, e.g. other sources of information).

Conclusion

Even as they provide high standards and protection of patients, the laws and regulations are flexible and allow patients with no alternatives access to promising, but not yet unapproved treatments while preserving the system of well-controlled clinical trials that provides the information necessary to determine the safety and effectiveness of proposed new products.

Protection of public health, compassion and respect for individuals, can, and do, co-exist.

Thank you for the opportunity to testify. I will be happy to answer any questions the Committee might have.

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Web page updated by jch 2001-JUL-09.

EXHIBIT E

PTC Therapeutics Announces Interim Phase 2 Results of PTC124 in Cystic Fibrosis

SOUTH PLAINFIELD, N.J., April 4, 2006 - PTC Therapeutics, Inc. (PTC), a biopharmaceutical company focused on the discovery, development, and commercialization of orally administered, proprietary small-molecule drugs that target post-transcriptional control processes, today announced interim results from Phase 2 clinical trials of PTC124 in patients with cystic fibrosis (CF) due to a nonsense mutation. These interim data suggest that PTC124 may have pharmacological activity that addresses the underlying cause of CF in these patients.

PTC124 is an orally delivered investigational product candidate that PTC is developing for the treatment of genetic disorders due to nonsense mutations. Nonsense mutations are alterations in the genetic code that prematurely halt the translation process, producing a shortened, non-functional protein. Phase 2 clinical trials are ongoing in the two initial indications being pursued, CF and Duchenne muscular dystrophy (DMD) caused by nonsense mutations.

PTC is conducting two comparable Phase 2 clinical trials of PTC124 in CF, one at the Hadassah University Hospital in Jerusalem, Israel, and the other at four sites in the US (University of Alabama at Birmingham Hospital and Clinics, AL; Johns Hopkins Hospital, Baltimore, MD; Rainbow Babies' and Children's Hospital, Cleveland, OH; and Denver Children's Hospital, Denver, CO). In each study, patients receive two sequential two-week courses of treatment, first at a lower and then at a higher PTC124 dose level.

The primary endpoint of these trials is the change in the CFTR chloride channel activity (also known as chloride conductance). Chloride conductance is evaluated using a standardized nasal transepithelial potential difference (TEPD) procedure. Cystic fibrosis patients lack sufficient CFTR protein and therefore have an abnormal TEPD chloride conductance.

Fifteen patients have completed two cycles of treatment and data from these patients were available for inclusion in the interim analysis. Of these patients, three were from the U.S. trial and 12 were from the Israeli trial. All patients had a nonsense mutation and multiple signs and symptoms of CF, with most patients having lung dysfunction, chronic bacterial infection of the lungs, pancreatic insufficiency, and low body weights.

In these 15 patients, at both dose levels, statistically significant results were observed in all three ways in which the TEPD endpoint was assessed, including mean improvement in chloride conductance, percentage of patients with a chloride conductance response, and percentage of patients with a chloride conductance improvement into the normal range. Statistically significant improvements in other endpoints, including lung function and weight were also observed. Several patients also reported improvement in well-being, decrease in cough, decreased mucus thickness, and easier clearing of mucus.

"We believe that these results suggest that PTC124 has meaningful pharmacological activity that is consistent with our hypothesis that treatment with PTC124 can restore the production and function of CFTR protein in patients with cystic fibrosis caused by a nonsense mutation," said Stuart W. Peltz, Ph.D., President and Chief Executive Officer of PTC. "We also believe that this is the first time such activity has been observed in a clinical trial of an oral therapy for cystic fibrosis."

PTC124 was generally well tolerated among the 15 patients included in the interim analysis. All adverse events that were potentially drug-related were mild in severity; there were no safety concerns identified in patients' physical examinations, vital sign measurements, or electrocardiograms; and no meaningful changes in laboratory safety parameters were observed. There were no dosing interruptions or trial discontinuations due to toxicity. Evaluation of treatment compliance indicated that patients took more than 98% of the intended total drug treatment at both the lower and higher dose levels. Pharmacokinetic data indicated that PTC124 was readily absorbed and desired plasma concentrations were achieved and maintained at the first and fourteenth days of both the lower-dose and higher-dose treatment courses.

PTC's Phase 2 CF and DMD clinical trials are ongoing. The interim results do not necessarily predict favorable final results from these ongoing CF or DMD trials or any future trial.

ABOUT PTC THERAPEUTICS, INC.

PTC is a biopharmaceutical company focused on the discovery, development, and commercialization of orally administered, proprietary small-molecule drugs that target post-transcriptional control processes. Post-transcriptional control processes regulate the rate and timing of protein production and are of central importance to proper cellular

function. PTC has assembled proprietary technologies and extensive knowledge of post-transcriptional control processes that it applies in its drug discovery and development activities. PTC's current pipeline of clinical and preclinical product candidates addresses multiple indications, including genetic disorders, oncology, and infectious diseases.

ABOUT PTC124

PTC124 is an orally delivered investigational product candidate in development for the treatment of genetic disorders due to nonsense mutations. Nonsense mutations are single-point alterations in the genetic code that prematurely halt the translation process, producing a shortened, non-functional protein. PTC124 has demonstrated activity in preclinical genetic disease models harboring nonsense mutations allowing the restoration of the production of full-length, functional proteins. In Phase 1 clinical trials, PTC124 was generally well tolerated, achieved target plasma concentrations that have been associated with activity in preclinical models, and did not induce ribosomal readthrough of normal stop codons. Pharmacokinetic modeling of the Phase 1 results allowed development of a dosing regimen for the Phase 2 studies in cystic fibrosis (CF) and Duchenne muscular dystrophy (DMD).

It is estimated that 10% of the cases of CF and 13% of the cases of DMD are due to nonsense mutations. PTC believes that PTC124 is potentially applicable to a broad range of other genetic disorders in which a nonsense mutation is the cause of the disease. The FDA has granted PTC124 Fast-Track designations and Orphan Drug designations for the treatment of CF and DMD due to nonsense mutations. PTC124 has also been granted orphan drug status for the treatment of DMD and CF by the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMEA). PTC124's development is supported by grants from the Muscular Dystrophy Association (MDA), Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT), FDA's Office of Orphan Products Development (OOPD), and by General Clinical Research Center grants from the National Center for Research Resources (NCRR).

ABOUT CYSTIC FIBROSIS (CF)

CF is among the most common life-threatening genetic disorders worldwide. According to the Cystic Fibrosis Foundation, CF affects approximately 30,000 adults and children in the United States and, according to the European Cystic Fibrosis Foundation, it affects a similar number of patients in Europe. CF occurs in approximately one of every 3,500 live births, with approximately 1,000 new cases diagnosed each year in the United States. There is a commercially available genetic test to determine if a patient's CF is caused by a nonsense mutation and it is estimated that nonsense mutations are the cause of CF in approximately 10% of patients in the United States. There is currently no available therapy to correct defective CFTR production and function. Instead, available treatments for CF are designed to alleviate the symptoms of the disease. These treatments include chest physical therapy to clear the thick mucus from the lungs, antibiotics to treat lung infections, and a mucus-thinning drug designed to reduce the number of lung infections and improve lung function. In addition, the majority of cystic fibrosis patients take pancreatic enzyme supplements to assist with food absorption in digestion. There is a significant unmet medical need for a treatment for the underlying cause of CF. More information regarding CF is available through the Cystic Fibrosis Foundation (www.cff.org).

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EXHIBIT F

PTC Therapeutics Announces Encouraging Additional Phase 2 Results of PTC124 in Cystic Fibrosis

- New Pediatric Data Presented at 21st North American Cystic Fibrosis Conference Confirm Previous Findings in Adult population -

SOUTH PLAINFIELD, N.J., Oct 05, 2007 /PRNewswire via COMTEX News Network/ -- PTC Therapeutics, Inc. (PTC), a biopharmaceutical company focused on the discovery, development and commercialization of small-molecule drugs targeting post-transcriptional control mechanisms, today announced encouraging data from a Phase 2 clinical trial of PTC124 in pediatric patients with cystic fibrosis (CF) due to a nonsense mutation. These pediatric results and additional information emerging from long-term studies support the existing data from prior short- term studies in adult CF patients. These studies show that treatment with PTC124 results in statistically significant improvements in a measure of the function of the cystic fibrosis transmembrane conductance regulator (CFTR) protein. These data were highlighted today in a plenary session entitled "CF Drug Development: What's New?" given by Dr. Felix Ratjen, University of Toronto Professor of Pediatrics and Respiratory Medicine Division Chief, at the 21st North American Cystic Fibrosis Conference in Anaheim, California.

(Logo: http://www.newscom.com/cgi-bin/prnh/20010919/PTCLOGO)

Patients with CF lack the CFTR protein, a chloride channel that maintains proper hydration of epithelial cells in the lung, pancreas, and liver. PTC has completed multi-site, open-label, dose-ranging Phase 2 clinical trials in adult CF patients to determine whether PTC124 can induce production of active CFTR protein. Studies in the U.S. and Israel evaluated nasal transepithelial potential difference (TEPD) as a surrogate for CFTR protein production in adult CF patients. Across the two studies, at both PTC124 dose levels tested, TEPD assessments showed statistically significant ($p<0.03$) improvements of mean CFTR-dependent chloride secretion in the airways.

PTC is currently conducting a third, open-label, dose-ranging Phase 2 clinical trial in pediatric CF patients at l'Hopital Necker-Enfants Malade, Paris, France to determine whether PTC124 can induce production of active CFTR protein. In the trial, patients receive two sequential two-week courses of treatment. Patients have been randomized to receive either a low or high dose of PTC124 followed by two weeks of rest and then are crossed over to the other dose level for an additional two weeks of therapy. Eleven patients have completed the study and data from these patients were available for inclusion in the initial analysis. Across both dose levels, statistically significant improvements ($p<0.05$) were seen in CFTR chloride-channel function as measured by TEPD.

"Our initial observations in a pediatric population confirm the findings from the previous studies in older CF patients," said Isabelle Sermet- Gaudelus, M.D., Ph.D., principal investigator at l'Hopital Necker-Enfants Malade, Paris, France. "Normalization of CFTR-mediated chloride secretion was observed in several of the children, indicating that PTC124 continues to demonstrate significant potential as a treatment for patients with CF. Based on the enthusiasm generated by these data, we have recently added two new study sites in Belgium in order to expand the evaluation of PTC124 across a larger pediatric population."

Eitan Kerem, M.D., Head of Pediatrics and the CF Center at the Hadassah University Hospital in Mount Scopus, Jerusalem also commented on longer-term studies that he has been leading in Israel. "Based on the positive results we observed during our initial study with two-week treatment periods, we have analyzed preliminary data from a three-month extension study evaluating the longer-term effect of PTC124 in patients with nonsense-mutation-mediated CF. We have seen encouraging evidence of sustained CFTR chloride-channel function and improvements in symptoms of CF, such as coughing, which we believe may be predictive of longer-term clinical benefit. We look forward to presenting the full data from this trial next year."

"We are very pleased to see the evidence of drug activity reported at last year's North American Cystic Fibrosis Conference reproduced by additional investigators in a pediatric population," said Langdon Miller, M.D, Chief Medical Officer of PTC. "We are also encouraged by the findings of the Israeli three-month study. We believe these confirmatory results, coupled with supportive safety data in more than 50 patients participating in the Phase 2 trial program, can lead to initiation of longer-term trials to evaluate the clinical benefit of PTC124 in patients with CF."

About Cystic Fibrosis

Cystic fibrosis (CF) is among the most common life-threatening genetic disorders worldwide. According to the Cystic Fibrosis Foundation, CF affects approximately 30,000 adults and children in the United States and, according to the European Cystic Fibrosis Foundation, it affects a similar number of patients in Europe. CF occurs in approximately one of

every 3,500 live births, with approximately 1,000 new cases diagnosed each year in the United States. There is a commercially available genetic test to determine if a patient's CF is caused by a nonsense mutation and it is estimated that nonsense mutations are the cause of CF in approximately 10% of patients in the United States. There is currently no available therapy to correct defective CFTR production and function. Instead, available treatments for CF are designed to alleviate the symptoms of the disease. These treatments include chest physical therapy to clear the thick mucus from the lungs, antibiotics to treat lung infections and a mucus-thinning drug designed to reduce the number of lung infections and improve lung function. In addition, the majority of cystic fibrosis patients take pancreatic enzyme supplements to assist with food absorption in digestion. There is a significant unmet medical need for a treatment for the underlying cause of CF. More information regarding CF is available through the Cystic Fibrosis Foundation (www.cff.org).

About PTC124

PTC124 is an orally delivered investigational new drug in Phase 2 clinical development for the treatment of genetic disorders due to nonsense mutations. Nonsense mutations are single-point alterations in the genetic code that prematurely halt the translation process, producing a shortened, non- functional protein. PTC124 has restored production of full-length, functional proteins in preclinical genetic disease models harboring nonsense mutations. In Phase 1 clinical trials, PTC124 was generally well tolerated, achieved target plasma concentrations that have been associated with activity in preclinical models and did not induce ribosomal read through of normal stop codons. PTC is currently conducting Phase 2 clinical trials of PTC124 in nonsense-mutation-mediated cystic fibrosis (CF) and Duchenne muscular dystrophy (DMD).

It is estimated that 10% of the cases of CF and 13% of the cases of DMD are due to nonsense mutations. PTC believes that PTC124 is potentially applicable to a broad range of other genetic disorders in which a nonsense mutation is the cause of the disease. The FDA has granted PTC124 Fast-Track designations and Orphan Drug designations for the treatment of CF and DMD due to nonsense mutations. PTC124 has also been granted orphan drug status for the treatment of CF and DMD by the European Commission. PTC124's development is supported by grants from the Muscular Dystrophy Association (MDA), Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT), Parent Project Muscular Dystrophy (PPMD), FDA's Office of Orphan Products Development (OOPD) and by General Clinical Research Center grants from the National Center for Research Resources (NCRR).

About PTC Therapeutics, Inc.

PTC is a biopharmaceutical company focused on the discovery, development and commercialization of orally administered, proprietary, small-molecule drugs that target post-transcriptional control processes. Post-transcriptional control processes regulate the rate and timing of protein production and are of central importance to proper cellular function. PTC's internally-discovered pipeline addresses multiple therapeutic areas, including genetic disorders, oncology and infectious diseases. In addition, PTC has developed proprietary technologies and extensive knowledge of post-transcriptional control processes that it applies in its drug discovery and development activities, including the Gene Expression Modulation by Small-molecules (GEMS) technology platform, which has been the basis for collaborations with leading pharmaceutical and biotechnology companies such as Celgene, Pfizer, CV Therapeutics and Schering- Plough.

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EXHIBIT G

PTC Therapeutics Announces Pharmacokinetic and Safety Results from Phase 2 Study of PTC124 in Duchenne Muscular Dystrophy

- Data Presented at 36th Child Neurology Society Annual Meeting -

SOUTH PLAINFIELD, N.J., Oct 11, 2007 /PRNewswire via COMTEX News Network/ -- PTC Therapeutics, Inc. (PTC), a biopharmaceutical company focused on the discovery, development and commercialization of small-molecule drugs targeting post-transcriptional control mechanisms, today announced pharmacokinetic and safety data from a Phase 2 clinical trial of PTC124 in patients with Duchenne muscular dystrophy (DMD) due to a nonsense mutation. The results, which include data from the third and final cohort of the study, show that treatment with PTC124 appeared well tolerated at all three dose levels and target plasma concentrations were achieved at the mid- and high-dose levels. These data were presented today at the 36th Annual Meeting of the Child Neurology Society (CNS) in Quebec City, Canada.

(Logo: http://www.newscom.com/cgi-bin/prnh/20010919/PTCLOGO)

In the study, patients received 28 days of PTC124 treatment at one of three dose levels. All clinical trial participants are boys with a nonsense mutation in the dystrophin gene, substantially elevated serum creatine kinase levels, and symptoms associated with DMD. The analysis presented today showed that PTC124 appeared well tolerated among the 38 boys included in the study. Adverse events were infrequent, mild to moderate in severity, and did not result in therapy interruptions or discontinuations. There were no concerns based on physical examinations, vital sign measurements, electrocardiograms or laboratory parameters. Compliance with PTC124 treatment was excellent at all three dose levels. Target plasma concentrations associated with activity in a preclinical model of DMD were achieved at the mid- and high-dose levels.

"DMD is a disorder with a significant need for better treatment options and we are encouraged by the results we have seen to date with PTC124," said Brenda Wong, M.D., Associate Professor of Pediatrics and Neurology, Cincinnati Children's Hospital Medical Center, Cincinnati, OH, who presented the data today at CNS and is one of the trial's lead investigators. "Based on the findings from this study, we believe that the safety profile of PTC124 supports continued testing in longer-term studies."

"These results add to the growing body of safety data for PTC124, which has now been evaluated in more than 150 subjects, including patients with both DMD and cystic fibrosis. The safety profile has consistently shown that PTC124 appears well tolerated," said Langdon Miller, M.D., Chief Medical Officer of PTC. "We are looking forward to presenting additional activity data from this study next week at the World Muscle Society meeting in Italy."

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a progressive muscle disorder that causes the loss of both muscle function and independence. DMD is perhaps the most prevalent of the muscular dystrophies and is the most common lethal genetic disorder diagnosed during childhood today. Each year, approximately 20,000 children worldwide are born with DMD (one of every 3,500 male children). More information regarding DMD is available through the Muscular Dystrophy Association (www.mdausa.org) and the Parent Project Muscular Dystrophy (www.parentprojectmd.org).

About PTC124

PTC124 is an orally delivered investigational new drug in Phase 2 clinical development for the treatment of genetic disorders due to nonsense mutations. Nonsense mutations are single-point alterations in the genetic code that prematurely halt the translation process, producing a shortened, non- functional protein. PTC124 has restored production of full-length, functional proteins in preclinical genetic disease models harboring nonsense mutations. In Phase 1 clinical trials, PTC124 was generally well tolerated, achieved target plasma concentrations that have been associated with activity in preclinical models and did not induce ribosomal read through of normal stop codons. PTC is currently conducting Phase 2 clinical trials of PTC124 in nonsense-mutation-mediated cystic fibrosis (CF) and Duchenne muscular dystrophy (DMD).

It is estimated that 10% of the cases of CF and 13% of the cases of DMD are due to nonsense mutations. PTC believes that PTC124 is potentially applicable to a broad range of other genetic disorders in which a nonsense mutation is the cause of the disease. The FDA has granted PTC124 Fast-Track designations and Orphan Drug designations for the treatment of CF and DMD due to nonsense mutations. PTC124 has also been granted orphan drug status for the treatment of CF and DMD by the European Commission. PTC124's development is supported by grants from the Muscular Dystrophy

Association (MDA), Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT), Parent Project Muscular Dystrophy (PPMD), FDA's Office of Orphan Products Development (OOPD) and by General Clinical Research Center grants from the National Center for Research Resources (NCRR).

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EXHIBIT H

PTC Therapeutics Announces Additional Results from Phase 2 Study of PTC124 in Duchenne Muscular Dystrophy

Data Presented at the World Muscle Society International Congress

SOUTH PLAINFIELD, N.J., Oct 18, 2007 /PRNewswire via COMTEX News Network/ -- PTC Therapeutics, Inc. (PTC), a biopharmaceutical company focused on the discovery, development, and commercialization of small-molecule drugs targeting post-transcriptional control mechanisms, today announced additional data from a Phase 2 clinical trial of PTC124 in patients with Duchenne muscular dystrophy (DMD) due to a nonsense mutation. The results, which include data from all three cohorts of the study, show that administration of PTC124 is associated with qualitative increases in muscle dystrophin expression and with reductions in serum creatine kinase values. These data were presented today at the World Muscle Society (WMS) International Congress in Taormina, Italy.

(Logo: http://www.newscom.com/cgi-bin/prnh/20010919/PTCLOGO)

Patients with DMD are boys and young men who lack dystrophin, a protein that is critical to the structural stability of muscle fibers. This Phase 2 multi-site, open-label, dose-ranging clinical trial enrolled 38 boys with loss of dystrophin due to a nonsense mutation in the dystrophin gene. Participants also had substantially elevated serum creatine kinase levels due to the disease, and symptoms associated with DMD. Boys enrolled in the trial received 28 days of PTC124 treatment at one of three dose levels, with the primary endpoint of the trial being an increase in dystrophin expression in muscle. Pre- and post-treatment muscle biopsies and blood analyses to assess muscle-derived creatine kinase were available from all 38 patients.

An in vitro analysis demonstrated PTC124-induced dystrophin expression in cultured muscle cells from all 35 (100%) of the boys with samples evaluable in this analysis. The in vivo data indicated that, across all three dose levels of PTC124, 18/38 (47%) of patients demonstrated visible improvement in the staining for dystrophin from muscle biopsies. Response did not appear to be dependent on type of nonsense mutation.

Blood levels of muscle-derived creatine kinase were also measured as assessments of muscle integrity. Statistically significant reductions in the concentrations of muscle-derived creatine kinase were observed during PTC124 treatment. In addition, several parents and teachers reported that boys participating in the study had improvements in terms of greater activity level and increased endurance during the study duration.

"We are very encouraged by these results, which show improvements in critical biomarkers of DMD," said presenter and study investigator, Carsten Bonnemann, M.D., Assistant Professor Neurology and Pediatrics, Children's Hospital of Philadelphia and University of Pennsylvania School of Medicine and Co-Director of the Neuromuscular Program, Children's Hospital of Philadelphia. "The combined in vitro and in vivo evidence of enhanced dystrophin expression and reduced muscle fragility offer signals of pharmacological activity that we hope to translate into potential clinical benefit for patients with DMD."

"Coupled with the emerging safety profile of PTC124, these data provide the impetus for moving forward rapidly to initiate longer-term studies for boys with DMD," said Langdon Miller, M.D., Chief Medical Officer of PTC. "We are actively working with our clinical investigators and the regulatory agencies to finalize plans for additional clinical trials and we look forward to commencing these studies in the coming months."

Stuart W. Peltz, Ph.D., President and Chief Executive Officer of PTC Therapeutics added, "These results, combined with the data presented earlier this month at the Child Neurology Society meeting and North American Cystic Fibrosis Conference, further support our belief that PTC124 represents a paradigm shift in the treatment of genetic disorders. Our future plans for PTC124 include the initiation of longer-term studies in DMD and cystic fibrosis (CF) as well as additional proof of concept studies in other indications. We hope that PTC124 will one day offer an improved treatment option for patients with nonsense-mediated DMD, CF, and a broad range of genetic disorders."

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a progressive muscle disorder that causes the loss of both muscle function and independence. DMD is perhaps the most prevalent of the muscular dystrophies and is the most common lethal genetic disorder diagnosed during childhood today. Each year, approximately 20,000 children worldwide are born with DMD (one of every 3,500 male children). More information regarding DMD is available through the Muscular Dystrophy Association

(www.mdausa.org) and the Parent Project Muscular Dystrophy (www.parentprojectmd.org).

About PTC124

PTC124 is an orally delivered investigational new drug in Phase 2 clinical development for the treatment of genetic disorders due to nonsense mutations. Nonsense mutations are single-point alterations in the genetic code that prematurely halt the translation process, producing a shortened, non- functional protein. PTC124 has restored production of full-length, functional proteins in preclinical genetic disease models harboring nonsense mutations. In Phase 1 clinical trials, PTC124 was generally well tolerated, achieved target plasma concentrations that have been associated with activity in preclinical models and did not induce ribosomal read through of normal stop codons. PTC is currently conducting Phase 2 clinical trials of PTC124 in nonsense-mutation-mediated cystic fibrosis (CF) and Duchenne muscular dystrophy (DMD).

It is estimated that 10% of the cases of CF and 13% of the cases of DMD are due to nonsense mutations. PTC believes that PTC124 is potentially applicable to a broad range of other genetic disorders in which a nonsense mutation is the cause of the disease. The FDA has granted PTC124 Fast-Track designations and Orphan Drug designations for the treatment of CF and DMD due to nonsense mutations. PTC124 has also been granted orphan drug status for the treatment of CF and DMD by the European Commission. PTC124's development is supported by grants from the Muscular Dystrophy Association (MDA), Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT), Parent Project Muscular Dystrophy (PPMD), FDA's Office of Orphan Products Development (OOPD) and by General Clinical Research Center grants from the National Center for Research Resources (NCRR).

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EXHIBIT I

PTC Therapeutics Announces Initiation of Phase 2b Registration-Directed Clinical Trial of PTC124 in Duchenne/Becker Muscular Dystrophy

First Registration Study of an Investigational Drug for Duchenne/Becker Muscular Dystrophy

SOUTH PLAINFIELD, N.J., April 23, 2008 /PRNewswire via COMTEX News Network/ -- PTC Therapeutics, Inc. (PTC), today announced the initiation of an international pivotal trial of PTC124 in patients with Duchenne/Becker muscular dystrophy (DMD/BMD) due to a nonsense mutation. The primary objective of this registration-directed Phase 2b trial is to demonstrate the efficacy of PTC124 as measured by improvements in the walking ability of patients with this progressive genetic disease.

(Logo: http://www.newscom.com/cgi-bin/prnh/20010919/PTCLOGO)

"DMD/BMD is a disorder with a significant need for better treatment options, and we are very encouraged by the promising results we have seen to date with PTC124," said Brenda Wong, M.D., Associate Professor of Pediatrics and Neurology, Cincinnati Children's Hospital Medical Center, Cincinnati, who was involved in the Phase 2a study and is one of the trial's lead investigators. "We believe that the safety profile of PTC124 and activity we have seen in the Phase 2a studies clearly support the initiation of this longer-term, registration-directed efficacy and safety study. We are very pleased to be a part of this groundbreaking trial."

Patients with DMD and BMD are boys and young men who lack dystrophin, a protein that is critical to the structural stability of muscle fibers. Patients develop progressive muscle weakness that leads to loss of ambulation, wheelchair dependency, and eventual decline in respiratory and cardiac function. It is estimated that one in 10 DMD patients are likely to have a Becker presentation, a milder form of the disease that is associated with later manifestation of symptoms. In essence, DMD and BMD represent a continuum of the same disease.

PTC124 is a novel, orally delivered drug in development for the treatment of patients with genetic disorders due to a nonsense mutation, a type of mutation found in approximately 13% of patients with DMD. In this double-blind study, patients will be randomized to receive placebo, or one of two dose levels of PTC124, three times per day. Eligible patients will be boys with nonsense-mutation-mediated DMD/BMD who are at least 5 years of age and are able to walk at least 75 meters or approximately 80 yards in six minutes. PTC expects to enroll a total of 165 patients at approximately 35 investigational sites; all study subjects will undergo 48 weeks of blinded treatment. Thereafter, all participants, including those who have been receiving placebo, will be eligible to enroll in an open-label PTC124 extension study.

The primary outcome measure is the total distance walked during a 6-minute walk test, a test of ambulation that has now been standardized for boys with DMD/BMD through a collaboration with noted investigator, Craig McDonald, M.D., at University of California at Davis. Other outcome measures in the Phase 2b study will evaluate activity at home, muscle and heart function, strength, cognitive ability, muscle integrity, and muscle dystrophin expression. Safety parameters, compliance, and PTC124 blood levels also will be monitored.

"We are very pleased to announce the initiation of the Phase 2b trial for PTC124 in boys with DMD/BMD," said Langdon Miller, M.D., Chief Medical Officer of PTC. "We applaud the patients, parents, and clinicians who have committed themselves to this effort. The design of this trial reflects our ongoing collaboration with the advocacy community, investigators at leading neuromuscular centers, and the U.S. and European regulatory agencies. We hope that PTC124 will soon offer a treatment that addresses the underlying cause of the disease for patients with nonsense-mediated DMD/BMD and that the development of PTC124 will set the stage for improving therapeutic options in this disabling and life-threatening disorder."

Stuart W. Peltz, Ph.D., President and Chief Executive Officer of PTC Therapeutics added, "Initiation of the Phase 2b trial is an important milestone for PTC. The trial builds on the results we have achieved to date in DMD and cystic fibrosis (CF) and constitutes a major step forward in establishing the potential for PTC124 as a paradigm shift in the treatment of genetic disorders. Our future plans for PTC124 include the initiation of longer-term studies in CF, as well as additional proof-of-concept studies in other indications."

About Duchenne and Becker Muscular Dystrophy

Duchenne and Becker muscular dystrophy (DMD/BMD) are progressive muscle disorders that cause the loss of both muscle function and independence. DMD/BMD is perhaps the most prevalent of the muscular dystrophies and is the most

common lethal genetic disorder diagnosed during childhood today. Each year, approximately 20,000 children worldwide are born with DMD (one of every 3,500 male children). It is estimated that one in 10 DMD patients are likely to have a Becker presentation, a milder form of the disease that is associated with later manifestation of symptoms. In essence, DMD and BMD represent a continuum of the same disease. More information regarding DMD and BMD is available through the Muscular Dystrophy Association (www.mdausa.org), the Parent Project Muscular Dystrophy (www.parentprojectmd.org), and the Association Francaise contre les Myopathies (www.afm-france.org).

About PTC124

PTC124 is an orally delivered investigational new drug in Phase 2 clinical development for the treatment of genetic disorders due to nonsense mutations. Nonsense mutations are single-point alterations in the genetic code that prematurely halt the translation process, producing a shortened, non- functional protein. PTC124 has restored production of full-length, functional proteins in preclinical genetic disease models harboring nonsense mutations. In Phase 1 clinical trials, PTC124 was generally well tolerated, achieved target plasma concentrations that have been associated with activity in preclinical models and did not induce ribosomal read through of normal stop codons. PTC124 has demonstrated pharmacodynamic proof of concept in Phase 2a clinical trials in nonsense-mutation-mediated Duchenne muscular dystrophy (DMD) and cystic fibrosis (CF).

It is estimated that 13% of the cases of DMD and 10% of the cases of CF are due to nonsense mutations. PTC believes that PTC124 is potentially applicable to a broad range of other genetic disorders in which a nonsense mutation is the cause of the disease. The FDA has granted PTC124 Subpart E designation for expedited development, evaluation, and marketing and has granted Orphan Drug designations for the treatment of CF and DMD due to nonsense mutations. PTC124 has also been granted orphan drug status for the treatment of CF and DMD by the European Commission. PTC124's development has been supported by grants from the Muscular Dystrophy Association (MDA), Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT), Parent Project Muscular Dystrophy (PPMD), FDA's Office of Orphan Products Development (OOPD) and by General Clinical Research Center grants from the National Center for Research Resources (NCRR). For additional information on the PTC124 clinical trial, please visit www.clinicaltrials.gov and search using the keyword: PTC124.

About PTC Therapeutics, Inc.

PTC is a biopharmaceutical company focused on the discovery, development and commercialization of orally administered, proprietary, small-molecule drugs that target post-transcriptional control processes. Post-transcriptional control processes regulate the rate and timing of protein production and are of central importance to proper cellular function. PTC's internally-discovered pipeline addresses multiple therapeutic areas, including genetic disorders, oncology and infectious diseases. In addition, PTC has developed proprietary technologies and extensive knowledge of post-transcriptional control processes that it applies in its drug discovery and development activities, including the Gene Expression Modulation by Small-molecules (GEMS) technology platform, which has been the basis for collaborations with leading pharmaceutical and biotechnology companies such as Pfizer, Celgene, CV Therapeutics and Schering- Plough. For more information, visit the company's website, www.ptcbio.com.

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EXHIBIT J

PTC THERAPEUTICS ANNOUNCES DATA FROM ADDITIONAL CLINICAL STUDIES OF PTC124 IN CYSTIC FIBROSIS CONFIRMING ACTIVITY

- Data Presented at the 31st European Cystic Fibrosis Conference -

South Plainfield, NJ and Prague, Czech Republic- June 12, 2008 - PTC Therapeutics, Inc. (PTC) today announced promising new data from two studies of PTC124 in cystic fibrosis (CF). Results from an Israeli Phase 2a extension study evaluating three months of oral PTC124 treatment in adult patients with nonsense-mutation-mediated CF demonstrated statistically significant improvements in the function of the cystic fibrosis transmembrane conductance regulator (CFTR) protein and a statistically significant mean [28%] decrease in the frequency of cough, one of the most prominent and burdensome CF-related symptoms. Results from the Hadassah University Hospital in Israel were presented today by Michael Wilschanski, M.D., Director, Pediatric Gastroenterology, and by Eitan Kerem, M.D., principal investigator and head of the Department of Pediatrics and Cystic Fibrosis Center. Separately, results from a European study evaluating 14-day courses of PTC124 in pediatric patients with nonsense-mutation-mediated CF confirmed the CFTR activity observed in previous short-term studies in adult patients. Data from the European study were presented today by Isabelle Sermet-Gaudelus, M.D., Ph.D., principal investigator at l'Hôpital Necker-Enfants Malade.

Patients with CF lack adequate levels of the CFTR protein, a chloride channel that maintains proper hydration of epithelial surfaces in the lung, pancreas, and liver. Patients with nonsense-mutation-mediated CF generally have a severe form of CF because virtually no CFTR protein is produced. Previous studies of PTC124 adult patients with CF evaluated nasal transepithelial potential difference (TEPD) as a surrogate for the presence and activity of the CFTR protein. Across the short- and long-term clinical trials at high and low doses of PTC124, TEPD assessments showed statistically significant improvements of mean CFTR-dependent chloride secretion in the airways.

The Phase 2a extension study in Israel assessed 3 months of oral PTC124 therapy at two different dose levels in 19 adult men and women with nonsense-mutation-mediated CF who had participated in a prior short-term PTC124 Phase 2a study. More than 90% of the patients had chronic CF-related lung infection and also had CF-induced pancreatic insufficiency. Results from the study showed that treatment with PTC124 resulted in statistically significant ($p<0.001$) improvements in CFTR function as measured by TEPD in both dose groups. The proportion of patients showing improvement in TEPD chloride secretion increased over time in the extension study. Trends towards improvements in mean FEV1 and FVC values were observed. Baseline data showed that CF patients cough a remarkable 643 times per day on average, with a range of 324 to 1,569 coughs per day. In comparison, healthy individuals generally cough fewer than 16 times per day, according to the European Respiratory Journal (Hsu 1994). Patients receiving PTC124 experienced a mean [28%] decrease in cough frequency by the end of three months of therapy ($p<0.01$). PTC124 was generally well tolerated, resulting in excellent mean compliance with the treatment regimen (>90%).

"Three months of treatment with PTC124 in nonsense-mutation-mediated CF patients was associated with time-dependent improvements in nasal TEPD chloride conductance, pulmonary function and cough – important markers suggesting the potential for benefit in patients with CF," stated Dr. Wilschanski. "PTC124 increases CFTR-mediated chloride secretion in patients with a variety of nonsense mutation types, which suggests a broad spectrum of activity across one of the major subpopulations in CF."

Dr. Kerem added, "The impact on cough that we observed in this study by objective measurement is particularly notable given that cough is one of the major symptomatic manifestations of the underlying disease process in CF. The reduction in cough achieved with PTC124 in this three-month study suggests secondary clinical effects of drug activity. Based on these findings, we believe that objective measurement of cough may offer a meaningful new way to evaluate drug efficacy in future CF clinical trials."

In a separate Phase 2a study in Europe, data are currently available from 21 children who received two 14-day treatment courses of oral PTC124 therapy at two different dose levels. Patients ranged in age from six to 18 years. All had nonsense-mutation-mediated disease, pathological lung infection, and pancreatic insufficiency. Statistically significant ($p<0.05$) increases in the proportion of epithelial cells showing surface staining with the CFTR protein were observed. In addition, statistically significant ($p<0.05$) improvements in CFTR-mediated chloride conductance as measured by TEPD were evident. PTC124 was generally well tolerated in pediatric patients and mean compliance with treatment was excellent (>95%).

"We are encouraged to see the activity of PTC124 observed in adults reproduced in a pediatric population in France and Belgium," commented Dr. Sermet-Gaudelus. "PTC124 causes the missing protein to be made, to be located in the right place in the cell, and to have functional effect. Combined with the generally well-tolerated profile of PTC124, we believe

these data support inclusion of pediatric patients in future clinical trials."

"We are gratified by these results from our Israeli, French, and Belgian investigators, which demonstrated the activity of PTC124 in patients across a variety of age ranges, geographies, and nonsense mutation types," stated Langdon Miller, M.D., Chief Medical Officer of PTC. "These data offer the basis for initiating a randomized, controlled Phase 2b study later this year to evaluate the clinical benefit of PTC124 in adults and children with nonsense-mutation-mediated CF."

ABOUT CYSTIC FIBROSIS
Cystic fibrosis (CF) is among the most common life-threatening genetic disorders worldwide. According to the Cystic Fibrosis Foundation, CF affects approximately 30,000 adults and children in the United States and, according to the European Cystic Fibrosis Foundation, it affects a similar number of patients in Europe. There is a commercially available genetic test to determine if a patient's CF is caused by a nonsense mutation, and it is estimated that nonsense mutations are the cause of CF in approximately 10% of patients in the United States. There is currently no available therapy to correct defective CFTR production and function. Instead, available treatments for CF are designed to alleviate the symptoms of the disease. These treatments include chest physical therapy to clear the thick mucus from the lungs, antibiotics to treat lung infections and a mucus-thinning drug designed to reduce the number of lung infections and improve lung function. In addition, the majority of cystic fibrosis patients take pancreatic enzyme supplements to assist with food absorption in digestion. There is a significant unmet medical need for treatments that address the underlying cause of CF. More information regarding CF is available through the Cystic Fibrosis Foundation (www.cff.org).

ABOUT PTC124
PTC124 is an orally delivered investigational new drug for the treatment of genetic disorders due to nonsense mutations. Nonsense mutations are single-point alterations in the genetic code that prematurely stop the translation process, preventing production of a functional protein. In Phase 2a clinical trials in nonsense-mutation-mediated cystic fibrosis (CF) and in nonsense-mutation-mediated Duchenne muscular dystrophy (DMD), PTC124 has demonstrated the ability to produce functional protein across a variety of nonsense mutation types. Across all clinical studies to date, PTC124 has been generally well tolerated and has achieved target plasma concentrations that have been associated with activity in preclinical models. PTC124 is currently in Phase 2b development with the goal of demonstrating that increasing functional protein levels in patients with nonsense-mediated genetic disorders may provide clinical benefits.

ABOUT PTC THERAPEUTICS INC.
PTC is a biopharmaceutical company focused on the discovery, development and commercialization of orally administered, proprietary, small-molecule drugs that target post-transcriptional control processes. Post-transcriptional control processes regulate the rate and timing of protein production and are of central importance to proper cellular function. PTC's internally discovered pipeline addresses multiple therapeutic areas, including genetic disorders, oncology, and infectious diseases. In addition, PTC has developed proprietary technologies and extensive knowledge of post-transcriptional control processes that it applies in its drug discovery and development activities, including the Gene Expression Modulation by Small-molecules (GEMS) technology platform, which has been the basis for collaborations with leading pharmaceutical and biotechnology companies such as Pfizer, Celgene, CV Therapeutics and Schering-Plough. For more information, visit the company's website, www.ptcbio.com.

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