UNITED STATES DISTRICT COURT
DISTRICT OF NEW JERSEY

JACOB GUNVALSON, CHERI and JOHN GUNVALSON as Guardians for Jacob Gunvalson, and CHERI and JOHN GUNVALSON, Individually,	:	
Plaintiffs,	:	District of New Jersey Index No. 08-cv-3559
- against -	:	
PTC THERAPEUTICS, INC.,	:	
Defendants.	:	

DECLARATION OF PATRICIA FURLONG

STATE OF OHIO)
) ss.:
COUNTY OF BUTLER)

I, PATRICIA FURLONG, pursuant to 28 U.S.C. § 1746, declare as follows:

1. I am the founder and President of Parent Project Muscular Dystrophy ("PPMD"). I submit this declaration in connection with the opposition of defendant PTC Therapeutics, Inc. ("PTC") to the motion of plaintiffs John Gunvalson and Cheri Gunvalson, in their capacity as guardians for Jacob Gunvalson and Jacob Gunvalson, John Gunvalson and Cheri Gunvalson, individually, for a preliminary injunction forcing

PTC to give Jacob Gunvalson access to PTC124. I make this declaration on the basis of my own personal knowledge.

2. PPMD is an organization focused on Duchenne and Becker muscular dystrophy (DMD/BMD). We invest in high risk/high impact research in academia and with industry. We work with healthcare professionals to improve clinical care and develop models to increase awareness for DMD/BMD and improve diagnosis with primary healthcare professionals. One of the most significant aspects of PPMD is our broad advocacy agenda, working with Members of Congress to recognize the importance and impact of muscle and muscle research. With our professional consultants in Washington, DC (Cornerstone Group), we mobilize our families to raise awareness for DMD within Congress and advocate for increased Federal investment in NIH and other federal agencies.

3. I have lost two children to Duchenne muscular dystrophy ("DMD"), in 1995 and 1996. I am very concerned that the Gunvalsons' decision to pursue access to PTC's experimental DMD treatment PTC124 undermines the rationale for safety as well as unbiased access to PTC124 for all individuals who are not eligible to participate in current clinical studies.

4. To me, the issue of pre-approval access to investigational drugs must necessarily concentrate on two areas: safety and fair play. There are many individuals with premature stop mutations unable to participate in clinical studies based on their functional status (non-ambulatory) or their cognitive capability. In my mind, decisions must be made in a very transparent way, with specific criteria, and in an unbiased

manner. Preferential treatment of one individual, when so many are in need, would have a negative impact on all of us (community, industry, regulatory).

5. While I personally want every boy like Jacob with DMD/BMD due to a nonsense mutation to have access to PTC124 as soon as practicable, I do understand why, at this juncture, PTC has not acceded to Mrs. Gunvalson's requests. Data from a dose ranging study of 28 days is insufficient at this time to warrant access to individuals outside of a clinical protocol. In my frequent dealings with PTC employees, they have consistently told me that they are interested in making PTC124 available to all patients who could possibly benefit from it, and that they are committed to do so as safely, rigorously, and quickly as possible. I have never known anyone at PTC124 to act contrary to these goals.

6. At present, I know of many other families who would very much like to have immediate access to PTC124. The mean age of death in DMD is >25 years of age. I do understand that while literal death is not imminent, there are many 'little' deaths, heartbreaking milestones in debilitating conditions where function is lost and the quality of life is diminished. For this reason, I do not believe it is fair or justified to provide access to only one individual; rather when PTC concludes that there is sufficient clinical data to justify safely giving such access, I believe it is essential to grant pre-approval access in a fair way to all who have the potential to benefit from PTC124.

7. Before she filed suit against PTC, Mrs. Gunvalson sent me a draft affidavit in support of a motion for injunctive relief against PTC. I responded to her with comments on the affidavit, by email. A copy of my correspondence with Mrs. Gunvalson is attached hereto as Exhibit A.

8. The complaint reflects some confusion both in PPMD's advocacy agenda as well as funding efforts specifically directed toward PTC Therapeutics. The limited funding PPMD has provided to support PTC124 research was sent directly to the University of Pennsylvania and used to fund animal toxicity studies. PPMD has provided no direct or indirect funding to PTC to support the clinical trials (*i.e.*, trials in human subjects) for PTC124 that have taken place to date. The $15.4 million grant received by Project Catalyst has nothing to do with PTC124, rather involved a capital campaign for drug development with PTC Therapeutics (Project Catalyst) and the submission of a grant to an NIH translation program (U54). The U54 submission was reviewed by the NIH scientific review committee and awarded to the U. Pennsylvania and PTC Therapeutics. Our advocacy efforts were not responsible for this successful application rather the application and review cannot be influenced by Congress or advocates.

9. I continue to disagree strongly with the Gunvalsons' approach in filing this lawsuit. While I can empathize with the fact that they want Jacob to receive any treatment that might help slow the progression of his disease, there are many other boys who need and deserve access and this needs to be accomplished by a process that is safe, fair and unbiased.

I declare under penalty of perjury that the foregoing is true and correct.

Patricia Furlong

Executed this 11th day of August, 2008.

Exhibit A

From: PatFurlong@aol.com [mailto:PatFurlong@aol.com]
Sent: Sun 6/8/2008 5:02 PM
To: cgunval@gvtel.com
Subject: Re: FYI

In a message dated 6/7/2008 6:05:23 PM Eastern Daylight Time, cgunval@gvtel.com writes:

Hello Cheri,

Thanks very much for sending this information. Frankly filing a complaint such as this is devastating. I worry that it will not achieve what you want for Jacob. I know this has been a very painful, difficult road for you and for Jacob. To be honest, I think a better solution would be for you to meet with PTC (rather than phone calls and emails) by yourself and seek a solution, understand what they are thinking, what concerns they may have and what you might expect going forward. I have not been privy to your conversations with Stu, Claudia or Landgon, but I have always found them willing to agree to a meeting to discuss any concerns I may have.

And, I am really worried about these documents as I find some of the statements confusing and some information in error. I think it is critical for you to be entirely accurate about your representation of the information

1. There have been 2 studies thus far with PTC124. Healthy volunteers (Phase I) and a Phase IIa. The phase two A study was a dose ranging study and as mentioned in the documents, had to be extended based on the fact that the boys metabolized the drug faster than first estimated. This information necessitated extension of the study and the inclusion of some non-ambulatory boys. I believe it was during this time that you met with Richard Finkel to discuss Jacob's participation in the extension. While I am not privy to your discussions with Dr Finkel, he did mention that Jacob was eligible to participate in the 2a, but that Jacob would have to d/c the Gentamycin. I understood from you that you were reluctant to take him off the Gentamycin, though I personally thought it would provide a good opportunity to participate and gain some insight into potential benefit and help PTC gain important information about non-ambulatory boys. There is a significant time lapse from your discussions with Finkel and his suggestion to take Jacob off Gentamycin in order to participate in the 2a protocol (oct. 2006) and actually taking Jacob off Gent. (summer, 2007). It was my understanding from Brenda that she recommended discontinuing the Gent. because of concern based on lab tests.

2 These documents suggest that PPMD and PTC Therapeutics were partners in the development of PTC124 This is incorrect. While PPMD did provide support for some of the animal toxicity studies for PTC124 as well as opportunities for company exposure during the annual conferences, our partnership with PTC Therapeutics was initiated on drug discovery for DMD on validated targets that could potentially benefit ALL boys with DMD. Based on PTC Therapeutics obvious interest in the DMD indication, we went to them about partnering on specific targets (myostatin inhibition, igf-1, alpha7integrin, utrophin and most recently Serca2A). This partnership, called "project catalyst" did indeed involve a fundraising campaign whereby 50+/- parents/donors/friends contributed amounts of $25,000 to participate in the Catalyst campaign. PPMD's investment this far is 2.3Million dollars and is expected to increase based on adding new targets. Recognizing drug development requires a signifcant investment, H. Lee Sweeney and PTC Therapeutics agreed to submit a grant to an NIH program directed toward translation. H. Lee Sweeney and PTC Therapeutics were successful and did receive a 15Million dollar award, restricted to Catalyst. It did not have anything to do with nor provide any support for development or trials with PTC124.

8/11/2008

3. Statistics for DMD are inaccurate 1:3500. And cause of death is primarily cardiac. Respiratory management is now Standard of Care (ATS consensus document) and no longer considered primary cause of death

Lastly, I have to say I am concerned about this approach. While I agree that Jacob and frankly every other boy with a stop codon should have PTC124, there is no safety data beyond 28 days at the moment. I am also aware that PTC has had discussions (I have participated in one such discussion) and continues to have discussions about expanded access, whether individual IND or another vehicle. I am also aware that there may be some concern about non-ambulatory boys based on metabolism, potential side effects and excretion of the drug. I do know PTC is committed to help the non-ambulatory boys and trying their best to sort through the pros and cons of these issues.

Cheri, I know you are trying everything in your power to help your son. Your efforts are remarkable for Jacob and for so many others. Like you, I believe we (this community and every dmd boy) are at a pivotal point. Some years ago, Industry was not interested in DMD. Today the landscape has changed. Companies are interested in this indication, promising trials are on the horizion. It must be compared to climibing Mt. Everest and I feel like we are near the peak. We have been waiting for this moment since the day Jacob was diagnosed. I know you are worried to death about Jacob and what the future will hold. I do think it is worth a private meeting with you and Dr. Parkin (no attorneys) with PTC and an honest attempt to air your view, listen to them and map out a plan.

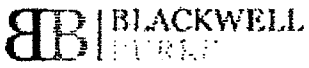
I am asking that you do not go public and I know it is a very big ask. I'm worry this may not be the most appropriate route and I worry it might hurt a lot of others in the process.

Sincerely,
Pat

----- Original Message -----
From: Mike Hatch
To: Cheri Gunvalson
Sent: Saturday, June 07, 2008 12:34 PM

These are hopefully the final versions.

Please note our new address.



431 South 7th Street
Suite 2500
Minneapolis, MN 55415

Mike Hatch
Blackwell Burke P.A.

612.343.3289 Direct
612.343.3205 Fax

mhatch@blackwellburke.com

UNITED STATES DISTRICT COURT
DISTRICT OF NEW JERSEY

JOHN GUNVALSON AND CHERI GUNVALSON, AS GUARDIANS FOR JACOB GUNVALSON,
Plaintiffs,

AFFIDAVIT OF CHERI GUNVALSON

v.

PTC THERAPEUDICS, INC.
Defendant.

STATE OF MINNESOTA)
)ss.
COUNTY OF HENNEPIN)

I, Cheri Gunvalson, being first duly sworn on oath, states as follows:

1. That I reside at 11617 506th Street, Gonvick, Minnesota 56644. I am married to John Gunvalson, and we have three children: Ben (19), Jacob (16) and Kelsey (13). I am a Masters Degreed Registered Nurse and John is a farmer.

2. In 1999 our son Jacob was diagnosed with Muscular Dystrophy. We had two gene sequencing studies undertaken, which both confirmed that Jacob has Duchene Muscular Dystrophy due to a Nonsense Mutation in the dystrophin gene. One study was undertaken at the City of Hope Hospital in Los Angeles on behalf of Ohio State University (Exhibit A) and the other study was undertaken by the University of Utah Medical Center. (Exhibit B)

3. Duchene Muscular Dystrophy is a progressive muscular disorder which is the most prevalent of the muscular dystrophies and the most common lethal genetic disorder diagnosed during childhood. One in every 7,000 children is born with Duchene

Muscular Dystrophy. According to Medline, there is no treatment for muscular dystrophy, and the life expectancy is under 25 years, with death usually caused by deterioration of the pulmonary system. (Exhibit C)

4. Jacob was an A student at school and was ambulatory until a year ago. Attached as Exhibit D is the report on Jacob's physical taken at Cincinnati Children's Hospital in December of 2007.

5. Approximately 15% of the patients with Duchene Muscular Dystrophy have the condition due to the presence of a "nonsense mutation" in the dystrophin gene. (Exhibit E) The nonsense mutation is called a "stop codon" because the mutation instructs the ribosome within the dystrophin gene to prematurely stop the production of the protein which provides the connective structure for muscle tissue. Exhibit F The mutation is in the X chromosome. Because girls have two X chromosomes, and because only one chromosome is necessary to produce the protein, it is a disease that is carried by girls but affects boys. (Exhibit C)

5. There are few approaches, however which have been tried or being evaluated. One approach is the use of gentamicin. Gentamicin is an intravenously administered antibiotic that, in sufficient doses, is believed to allow the ribosome in the gene to read through the "stop codon" and continue to develop the protein for muscular connective tissue. (Exhibit F) Jacob is one of less than ten (10) DMD boys in the country who has been under long term treatment of gentamicin, from January of 2003 to June of 2007.

6. The most promising treatment is PTC124, a drug that masks the stop codon and allows the ribosome to direct that the dystrophin be produced to a mature cell.

(Exhibit F. Exhibit G) Dr Lee Sweeney, a physiology professor at the University of Pennsylvania Medical School who works with PTC Therapeutics, the PPMD and with the clinical trials, has told me that PTC 124 is twelve times as effective as gentamicin and may actually restore dystrophin to functional levels.

7. PTC124 is produced by PTC Therapeutics, Inc. PTC Therapeutics participates in a novel joint venture with a parent group known as Parent Project for Muscular Dystrophy ("PPMD"). Attached as Exhibit H is an article that describes the novel relationship between PPMD and PTC Corporation. I joined PPMD in 2001 and have been very active in the organization. PPMD is composed of approximately 3,000 parents and relatives of muscular dystrophy children. 50 of which have contributed at least $25,000 for research. (Exhibit H) PPMD has contributed at least $3 million to PTC Therapeutics for research. (Exhibit I)

8. PPMD and PTC describe their collaboration as "Project Catalyst". (Exhibit I) According to *Nature Biotechnology*, "Project Catalyst" creates several benefits for PTC, including increased access to government funding and advocacy intervention with the FDA and other regulators. (Exhibit H) According to *Nature Biotechnology*, the PPMD parents have played a critical roll in getting NIH funding for the Corporation.

9. Prior to 2002 muscular dystrophy received very small funding from the National Institute of Health as compared to other diseases of equal morbidity. In 2001 I joined the Government Relations Board of PPMD. I helped draft the Muscular Dystrophy CARE Act, the purpose of which is to equalize funding for muscular dystrophy patients. I also persuaded my two Congressmen at the time to be primary

authors for the legislation, Congressman Collin Peterson and Senator, Paul Wellstone. (Exhibit J)

10. In 2001 I personally met with dozens of Congressmen and Senators and I spent the entire year on the telephone calling Congressional offices. Jacob and I persuaded 235 Congressmen and 49 Senators to co-author the Muscular Dystrophy CARE Act, which was the only disease specific legislation enacted by the 107th Congress. (Exhibit J)

11. Prior to passage of the Muscular Dystrophy CARE Act, muscular dystrophy patients, on a per patient basis, received but a small fraction of grants and funds when compared to other diseases with similar morbidity outcomes. In 2001 only $14.3 million was allocated by the National Institute of Health to Muscular Dystrophy research. The legislation doubled the research in the first year and grew to $54 million by 2006. (Exhibit J)

12. The legislation also required the National Institute of Health establish and fund Centers of Excellence for treatment of muscular dystrophy. After the law was signed on December 18, 2001, I was appointed to the NIH Review Board to designate the Centers of Excellence. (Exhibit J)

13. Since 2001 I have continued to advocate in Congress and with the National Institute of Health in an effort to increase funding for Duchenne Muscular Dystrophy. Each year I worked with Betty Lou Taylor, the staff director of the Senate Labor, Health and Human Services Appropriation Committee from 2003 to 2006, in preparing the annual Committee Report which directs the National Institute of Health as it relates to grants and funding proposals. My mission was to make sure that Muscular

Dystrophy research was funded at least equal to other diseases with similar morbidity outcomes.

14. In 2005, PTC Therapeutics began its first clinical trial of PTC124, primarily to determine patient tolerance to the drug. I recall a meeting with Claudia Hirawat, the Senior Vice President for Corporate Development, at a PPMD meeting, where she told me that one of the hurdles in bringing a drug like PTC124 to market was raising the money necessary to pay for the clinical trials that test the efficacy and safety of a new drug. Thereafter, I urged Dr. Langdon Miller, the Chief Medical Officer of PTC, to file applications for grants from the National Institute of Health. He was skeptical of the suggestion, noting that the applications were very complicated and bureaucratic. Having served on a Peer Review Committee with NIH in 2003, I was adamant that PTC should file an application. I told him that 10% of the NIH grant money has to go to private companies and that very few private companies were applying for it. Within a year and a half PTC Therapeutics received a $15.4 million research grant by NIH to build upon the research paid for by Project Catalyst. (Exhibit I)

15. Jacob was not enrolled in the initial Phase I trial that was undertaken in 2005. The findings of the 2005 study were very promising, and on March 30, 2006 the company announced that the FDA had granted "fast track status" to the development of the drug. The company also initiated Phase II trials in early 2006 (Exhibit E), but once again, Jacob was not enrolled in the clinical trial.

16. Concerned with Jacobs's deterioration, I went to Jacobs's pediatrician in Bemidji, Minnesota, Dr. John Parkin, and asked him if he would apply for a "Compassionate Use" exemption from the FDA on behalf of Jacob. In order to qualify

for a "Compassionate Use" exemption the pharmaceutical company must agree to make the drug available to the physician. (Exhibit K)

16. Accordingly, in April of 2006, Dr. Parkin wrote to Dr. Langford Miller, the Chief Medical Officer of PTC, requesting that the company make the drug available to him for a single patient investigative study. (Exhibit L) On April 14, 2006 Dr. Miller responded by stating that the results of the Phase IIa clinical trials would not be known until the end of 2006. Once patient safety is confirmed with the study, Dr. Miller said the parties could discuss an IND "compassionate use" proposal. (Exhibit M)

17. Claudia Hirawat is Senior Vice President for Corporate Development at Defendant PTC. The website for PTC indicates that she is directly responsible for fund raising and commercial development of the company. I consider Claudia to be my friend.

18. On July 13, 2006 Jacob and I attended the Annual PPMD Conference. At the conference Jacob had a long conversation with the CMO of PTC, Dr. Miller. The next day John and I had a private conference with Dr. Miller. Dr. Miller expressed great appreciation to me for my work in Washington and getting funding. As noted in the *Nature Biotechnology* magazine NIH funding was critical to success of the company. (Exhibit H) During the conversation I asked Dr. Miller if Jacob would get the PTC124. Dr. Miller said that the boys in the first trial metabolized the drug faster than expected and that they will need to do an extended 28 day trial at a higher dose. He told me that the Company did not know the right dosage level yet and they need to get the safety data back. He said that they needed to figure out the right dosage level and to do so they may need to do another short trial at higher doses. He reassured me that Jacob will get the drug.

19. On September 27, 2006 I was recognized for my work in getting funding for muscular dystrophy at the Annual Gala of the National Genetic Alliance in Washington, D.C. Claudia Hirawat, the PTC Senior Vice President in charge of fundraising and corporate development, also attended the Gala. At the event Senior Vice President Hirawat expressed great appreciation to me for my work in getting federal funding for research. At the event I explained to her that I was frustrated with the delay in getting Jacob access to PTC124. She assured me that Jacob would get access to the medication. Senior Vice President Hirawat and a board member of PPMD then invited me to attend a meeting the next day in Philadelphia to hear a presentation on the results of the Phase IIa clinical study.

20. The next day I took a train to Philadelphia where PTC released the successful results of the Phase II clinical study of PTC124, which were eventually released to the media the next month. (Exhibit N) The preliminary findings were that the boys in the study tolerated the medication, that there were no significant adverse effects, and that the boys showed increased development of dystrophin. (Exhibit N)

21. That evening a dinner was held at the Loew's Hotel. Dr. Stewart Peltz, the President and Chief Executive Officer of PTC, sat next to me at the dinner. Dr. Peltz thanked me for my leadership in getting federal funding for muscular dystrophy treatment. I asked Dr. Peltz if Jacob could get the drug. Dr. Peltz agreed that Jacob would have access to it.

22. Thereafter I recall participating in a conference call between Dr. John Parkin of Bemidji and Senior Vice President Hirawat. I believe the call was made in October of 2006. During the conversation Senior Vice President Hirawat made a

commitment that Jacob would be given access to PTC124. During this discussion it was also noted that Jacob was being administered Gentamicin and that, to be enrolled in the PTC124 trial, Jacob would have to get off of Gentamicin for at least 90 days.

24. In May of 2007 Jacob was examined by Dr. Brenda Wong a child neurologist and Cincinnati Children's Hospital. Dr. Wong participates in the PTC trials. Dr. Wong recommended that Jacob discontinue his dosage of Gentamicin in preparation for enrollment in a PTC124 clinical trial. (Exhibit O)

25. On July 11, 2007, PTC and PPMN announced that PTC and the University of Pennsylvania jointly received a $15.4 million research grant to develop the drug. (Exhibit I) The next day I attended the PPMD Conference in Philadelphia. At the conference, on July 14, 2007, I had another conversation with Dr. Peltz, the CEO of PTC. I asked Dr. Peltz if Jacob could get the drug. Dr. Peltz responded that Jacob should have been enrolled in a previous Clinical Trial. Distressed by that comment, I went to Sr. Vice President Claudia Hirawat, who then invited Jacob and me to visit her at her home and tour the company headquarters.

26. We then visited Ms. Hirawat at her home. During the visit Ms. Hirawit again represented to Jacob and I that the company is working to include Jacob in a trial.

27. In October and November of 2007, Ms. Hirawit and I exchanged a number of e-mails. On October 10, 2007 we reminisced about our stay at her home and Jacob's desire to eat a New York pizza. (Exhibit P) An October 25, 2007 e-mail told us that she keeps all of Jacob's photos at the top of her desk, that she considers the family to be her friend, that she and her husband were touched by our visit to their home, and that Jacob's access to PTC124 was seemingly dependent on FDA approval. (Exhibit Q) Throughout

this process I believed that the company was preparing Jacob to be enrolled in a trial or at least to have access to the drug by January of 2008. For instance, on November 26, 2007 I had a conversation with Ms. Diane Goetz, who advised me that Jacob cannot use the drug under an expended use protocol but that he will be able to get the drug in a different way. I was ecstatic and I called a number of people to let them know that we were getting into a Trial.

29. On December 30, 2007 I emailed Ms. Goetz and asked her what she meant by her statement. (Exhibit R) Diane Goetz responded that Jacob does not qualify for the trial because patients have to be ambulatory in order to measure their progress. She began the process of backpedaling off the PTC commitment by stating that the company was looking for another study that Jacob might be able to enroll. (Exhibit S)

30. Finally, on April 12, 2007, I was told that the company did not know when or if Jacob would ever get the drug. On April 23, 2008, PTC announced the commencement of yet another Phase IIb clinical study. (Exhibit T)

31. Over the past two and one half years representatives of PTC repeatedly raised my expectations about Jacob's access to the drug. At several points it was made clear to me that, by word and by deed, that the biggest obstacle to getting Jacob access to the PTC124 was to finance the clinical trials necessary to demonstrate efficacy and safety. They made it clear to me that my work on Capitol Hill was key to getting DMD funding. In other words, my success in getting funding for Muscular Dystrophy would be rewarded with PTC giving my son access to PTC124. I believed that there was an implied agreement between company officials and myself that my efforts would result in Jacob getting access to the drug.

32. On repeated occasions Dr. Miller, Dr. Peltz, Ms. Hirawit, and Ms. Goetz told me that Jacob would have access to the drug.

33. In reliance upon Jacob having access to the drug, his dosage of Gentamicin was terminated in June of 2007.

Further your affiant sayeth not.

_____
Cheri Gunvalson

Subscribed to and sworn before me

this ___ day of May, 2008.

_____
Notary Public