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.	:	

AFFIDAVIT OF CLÁUDIA HIRAWAT

STATE OF NEW JERSEY)
) ss.:
COUNTY OF MIDDLESEX)

CLÁUDIA HIRAWAT, being duly sworn, deposes and says:

1. I am the Senior Vice President, Corporate Development, at defendant PTC Therapeutics, Inc. ("PTC"). I submit this affidavit on behalf of PTC in opposition 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 either (i) pursuant to a "protocol exception" permitting him to participate in an ongoing clinical trial for which he is ineligible; or (ii) for use in a proposed "single patient study" by his pediatrician, Dr. John Parkin.

2. I make this affidavit on the basis of my own personal knowledge, on information I have learned through conversations with other PTC personnel, and on my review of certain business records maintained by PTC.

General Background Information

3. I joined PTC in September 2000 as the Director of Corporate Development. In that role, I was tasked with building various functions of PTC (including patient advocacy, business development and public relations). At the time I joined the company, our initial research indicated the potential to identify a drug that might represent a treatment for approximately 1,800 distinct genetic disorders. This drug is now known as PTC124.

4. Early on, I worked closely with Stuart Peltz, PTC's President and Chief Executive Officer, to prioritize specific disorders for which a drug such as PTC124 should be investigated as a potential treatment. As part of this process, Dr. Peltz and I met with the leadership of the National Organization for Rare Disorders ("NORD") and many patient advocacy groups. Frequently, patient advocacy groups include medical professionals, patients, and their family members. Thus, early on, I spoke to a number of patient families who were interested in obtaining access to PTC124.

5. Ultimately, PTC decided that it would initially pursue preclinical and clinical studies for PTC124 as a potential treatment for two separate indications: cystic fibrosis ("CF") and Duchenne muscular dystrophy/Becker muscular dystrophy ("DMD/BMD").

6. From my initial contacts with NORD and patient advocacy groups, I witnessed the desperation felt by families whose children have been diagnosed with severe disorders. I also heard of a number of instances in which scientists and physicians were overly optimistic about the possible benefits of potential new treatments for these disorders and provided families with

false hope. Occasionally, unwarranted optimism about the availability of treatments or "cures" for life-threatening conditions that later proved to be false resulted from carelessness, but, more often that not, it resulted from sheer excitement about scientific progress in difficult fields.

7. At PTC, we believe that a company can have a close and honest interaction with patient families and our efforts to foster such interactions have been extremely successful. Hundreds of families have expressed their gratitude for our style of communication, and in 2008 PTC received an Art of Industry Partnership Award from the Genetic Alliance, honoring it for modeling "the benefits of creative partnerships between consumer advocates and industry to advance understanding and treatment of genetic conditions, disorders, and diseases." A copy of the announcement of the award PTC received is attached hereto as Exhibit A.

8. As a result of PTC's growth, as of late 2007, my responsibilities at PTC have focused solely on business development – specifically collaborations with other pharmaceutical and biotechnology companies. I began to transition my responsibilities for interacting with patients and their families to other PTC employees, in particular Diane Goetz, in 2007. The transition took several months, during which time I worked closely with Ms. Goetz to ensure an efficient and complete transfer of information.

9. As part of my ongoing commitment to DMD/BMD, I also am a member of the Board of Directors of Parent Project Muscular Dystrophy ("PPMD"), an organization dedicated to supporting muscular dystrophy research, models of care, and awareness.

10. People with DMD/BMD cannot manufacture sufficient quantities of or appropriately functional dystrophin – a protein needed to ensure muscular structure and stability. Approximately 15% of the individuals with DMD/BMD have the condition as the result of what is known as a nonsense mutation in their DNA. This mutation prevents their bodies from

manufacturing complete dystrophin molecules. PTC124 is designed to enable the body to read through the genetic material that prevents complete protein production and facilitate complete dystrophin production.

11. I understand that Mrs. Gunvalson is claiming that I told her on several occasions that Jacob would receive access to PTC124, although she does not claim that I ever promised her that Jacob would receive access to PTC124 at any particular point in time. This is untrue. At various times, I have discussed with Mrs. Gunvalson that PTC is committed to considering access opportunities for patients who do not quality for participation in its current studies at the appropriate time. However, I never promised Mrs. Gunvalson that Jacob would be placed in any future trial for PTC124, or that pre-approval access to PTC124 would be assured for Jacob. To the contrary, I have consistently told Mrs. Gunvalson, and was careful to capture this statement in writing many times, that PTC was not in a position to grant pre-approval access to Jacob or any other child with DMD/BMD outside the formal clinical trial setting because of the early state of clinical development and limited data.

The Gunvalsons' Inaccurate Descriptions of My Communications With Cheri Gunvalson

12. I understand that Mrs. Gunvalson is claiming that I instructed her to forgo enrolling Jacob in a Phase 2a clinical trial for PTC124 and, instead, urged her to continue to treat Jacob's condition with an antibiotic called gentamicin. This is untrue. I recall communicating with Mrs. Gunvalson about the possibility of enrolling Jacob in the Phase 2a trial both before the trial began in late 2005 (before the enrollment criteria was known), and in late 2006, after a decision had been made to open the trial to non-ambulatory subjects.

13. As set forth more fully below, in the first round of communications, I advised Mrs. Gunvalson in writing to discuss the possibility of enrollment with Jacob's treating

physician, and that *she* would need to weigh the benefits of enrollment against discontinuing gentamicin therapy. I also spoke to Mrs. Gunvalson by telephone. During our discussion, Mrs. Gunvalson expressed great concern about discontinuing Jacob's gentamicin therapy because she believed that gentamicin was helping his condition. As an employee of the clinical sponsor for the trial, I, of course, had an interest in making sure that the trial was fully subscribed, but it is also my responsibility to support patients in their decision process without influencing their decisions, so all I was able to do was suggest that Mrs. Gunvalson discuss the decision with Jacob's treating physician as well as the clinical trial investigator. I always emphasize in these instances that there are no right or wrong decisions, and that the family needs to make a joint decision based on their best assessment. I told Mrs. Gunvalson that she should work with Jacob's treating physician, Dr. Parkin, to address her concerns about whether enrollment in this 28-day trial was the right decision for her family if Jacob was truly benefiting from taking gentamicin. I suggested that Dr. Parkin work with DMD/BMD experts such as Dr. Brenda Wong and Dr. Richard Finkel to help evaluate her concerns about taking Jacob off gentamicin.

14. In my communications with Mrs. Gunvalson in late 2006, Mrs. Gunvalson informed me that Dr. Finkel, the primary investigator for this trial in Philadelphia, had made the decision not to enroll Jacob in a later phase of the trial. Mrs. Gunvalson repeated to me her concerns about taking Jacob off gentamicin due to her belief that it was effective in treating his condition.

15. I of course played no role in prescribing gentamicin for Jacob. I am not aware of who originally prescribed gentamicin to Jacob, nor am I aware of any DMD/BMD experts who use this drug as a treatment for DMD/BMD. But Mrs. Gunvalson was always adamant that gentamicin was in fact helping Jacob and reluctant to stop the treatment.

16. Mrs. Gunvalson also claims that I assured her that Jacob's non-enrollment in the Phase 2a trials would have no adverse impact on his ability to gain access to PTC124 in a future clinical trial. This allegation is inaccurate. Being precluded from PTC's future trials as a result of non-enrollment in the Phase 2a trials was a common concern among patient families that I was frequently called upon to address. When Mrs. Gunvalson raised the concern with me, I gave her the same information I gave many other parents. I informed Mrs. Gunvalson that Jacob's non-enrollment in Phase 2a trials would not by itself preclude him from participating in all of PTC's anticipated future clinical trials for PTC124, assuming he satisfied the eligibility requirements for those trials, and I also told her that participants in the initial 2a trials would likely have a preference over others in terms of future studies.

Mrs. Gunvalson's Aggressive Pursuit of Access to PTC124

17. I first met Cheri Gunvalson at a PPMD conference several years ago. She told me that she was interested in having immediate access to PTC124. I informed her that such access was not possible because PTC124 was still in the first phase of clinical trials, which involved introducing PTC124 into healthy adult volunteers, and there was no safety or efficacy data available for the drug as a DMD/BMD treatment.

18. Since my initial meeting with the Gunvalsons, Mrs. Gunvalson has aggressively pursued access to PTC124 for Jacob, and I have had a number of other communications with her and others about PTC's inability to provide such access within her desired timeframe and outside of a clinical trial. Despite the repeated efforts that I have made to explain to Mrs. Gunvalson why it would be inappropriate and irresponsible for PTC to allow her son access to PTC124 for his own long-term personal use before there was sufficient data indicating that it would be appropriate to do so, and without consideration to all of the other young men who want it, she

has continued to press the issue with me, other PTC executives, and various third parties. As set forth below, in many instances, Mrs. Gunvalson has mischaracterized what people at PTC have told her and created impressions that are not true.

19. In my experience, Mrs. Gunvalson is unique among the parents of children with DMD/BMD in her desire for special treatment, her sense of entitlement based on her political connections and fundraising activities, and her single-minded focus on Jacob. Generally speaking, parents of children with severe disorders such as DMD/BMD understand and appreciate that the goal of clinical research is to determine if a drug is in fact a viable treatment, not just to treat their own children even in the absence of this information. There is an extremely strong sense of community and fellowship among these parents and they understand that, insofar as experimental drugs like PTC124 are concerned, there are no guarantees. While I understand and in many ways respect Mrs. Gunvalson's determination, it has become apparent to me that Mrs. Gunvalson has reached such a level of desperation that she is hearing only what she wants to hear, despite what I and others at PTC have repeatedly told her.

20. I understand that the Gunvalsons are contending that Jacob may be the *only* young man suffering from DMD/BMD who is ineligible for inclusion in either PTC's ongoing Phase 2a extension study or its Phase 2b trial. This is absolutely not the case. There are many young men just like Jacob who would like access to PTC124, but have not participated in any trial, many of whom are in significantly more advanced stages of DMD/BMD than Jacob. I have personally spoken to patient families who do not currently have access to PTC124 and would like access to the drug.

21. Early on in my own personal interactions with Mrs. Gunvalson, she approached Dr. Russell Katz from the FDA. A copy of Mrs. Gunvalson's February 20, 2006 Email to Dr. Katz is attached hereto as Exhibit B.

22. In response, Dr. Katz sent Mrs. Gunvalson an unsigned letter that makes it abundantly clear that it is up to PTC to decide whether to grant pre-approval access to PTC124 to Jacob Gunvalson, or to anyone else. A copy of Dr. Katz's response letter is attached hereto as Exhibit C. In particular, Dr. Katz's letter states that the "FDA cannot compel a company to supply an individual patent with an investigational drug outside of its planned clinical trials," and acknowledges that an investigational drug sponsor may be "unwilling to provide the product outside of clinical trials, especially relatively early in drug development." *See* Ex. C at 2.

23. A few months later, on August 11, 2006, in response to a request from Mrs. Gunvalson to provide her with talking points for potential questions from various sources about Jacob's access to PTC124 that she anticipated having to answer, I suggested the following:

> - Phase 2 data is expected before the end of 2006
>
> - Phase 3 is expected to start mid-2007
>
> - PTC hopes to work with FDA and patient groups to design a program that would allow pre-approval drug access for the patients who do not qualify for participation in the study. We don't know what the criteria for participation would be, so we need the design of Phase 3 to be in place, and agreed upon with the FDA before we can pursue such a project, but it is a priority for us.

A copy of my August 11, 2006 email to Mrs. Gunvalson is attached hereto as Exhibit D. As it turned out, data from Phase 2 was not fully analyzed until 2007, and the eligibility criteria for PTC's Phase 2b/3 trials were not announced until April 23, 2008.

24. The Gunvalsons allege that, just six weeks after I sent Mrs. Gunvalson this email, I "assured" her on September 27, 2006, that Jacob "would get access to" PTC124. Compl. ¶ 28.

They further allege that some time in October 2006, I again told Mrs. Gunvalson, and also Jacob's pediatrician, Dr. John Parkin, that Jacob "would get access to PTC124." *Id.* ¶ 30. These allegations are untrue.

25. In fact, on January 29, 2007, I had a conversation with Mrs. Gunvalson during which I reminded her that we still did not have sufficient safety data for PTC124 to make the drug available as part of a pre-approval access program. I memorialized that conversation in an email I sent to Mrs. Gunvalson the next day. In pertinent part, my email provides:

> As we discussed yesterday, while this topic is a great priority to us, we don't have a developed plan for pre-approval drug access (whether it is expanded access or any other form such as an investigator-initiated IND) at this time. There are several elements of the development of PTC124 that would need to be addressed, including:
>
> - determining the best dose (studies ongoing)
>
> - determining the enrolment criteria for next studies, which would tell us which patients would be included or excluded of future trials (these efforts are ongoing)
>
> - securing agreement from the regulatory agencies about the design of the study, including inclusion/exclusion criteria and end points, which would allow us to determine how many patients would be required for the trial as well as feedback regarding the safety data (at this point no patient has received PTC124 longer than 28 days).
>
> Until this information is available to us, we are not in a position to move forward with any form of pre-approval drug access. As we discussed, this type of program is typically conducted during Phase 3, and in most instances when the enrollment for the trial has been completed.
>
> I realize the waiting is very difficult, it is very difficult for us too. but we just can't move ahead of the science or the safety data. We are working diligently to advance this in the best possible manner.

A copy of my January 30, 2007 email to Mrs. Gunvalson is attached hereto as Exhibit E. My use of the phrase "Phase 3" in this email refers to the anticipated large controlled clinical trials

that I mentioned earlier. Approximately 15 months after I wrote this email, PTC announced that it intended to conduct a large controlled clinical trial, which it called a "Phase 2b trial."

26. Next, in early February 2007, after Mrs. Gunvalson had again contacted the FDA in an effort to get support for her request that PTC make PTC124 available to Jacob outside the clinical trial setting, I again reminded her by email that the company was not in a position to give pre-approval access of PTC124 to Jacob. Set forth below is what I wrote Mrs. Gunvalson on this topic:

> We are currently working with our advisors to develop the regulatory strategy for PTC124 (what are the next studies, duration/end-points, etc.) and hope to meet with the FDA within the first half of 2007. This meeting should provide us guidance for the path forward for PTC124 and we would anticipate being able to provide you a better sense of a timeline after these discussions take place. We will contact Mr. Banks as you suggested, because we are always interested in studying mechanisms for pre-approval drug access, but must emphasize that at this time we don't have enough safety or efficacy data to support your request for continuous treatment with PTC124.

A copy of my February 2, 2007 email to Mrs. Gunvalson is attached hereto as Exhibit F.

27. Shortly after I wrote this email, I had a conversation with David Banks of the FDA. Prior to placing a call to Mr. Banks, Mrs. Gunvalson reported to me that Mr. Banks had told her that special access was frequently granted when clinical drugs were in the stage of development that PTC124 was in at the time, and that he believed "Jacob's situation would fit the criteria" for granting such access. A copy of Mrs. Gunvalson's email to me summarizing her conversation with Mr. Banks is attached hereto as Exhibit G.

28. When I contacted Mr. Banks on February 6, 2007, he told me that he understood from Mrs. Gunvalson that PTC124 was very close to receiving marketing approval from the FDA. I explained to Mr. Banks that this was not true and told him where the drug was in the approval process – at that time, we were still in the process of accruing and analyzing the data

from our Phase 2a trials and still had no clinical data to support the conclusion that PTC124 was safe for long-term use. I recall that Mr. Banks was very surprised to learn that we were still in the fairly early stages of clinical trials for PTC124. He was supportive of PTC's plan, and told me that he felt that Mrs. Gunvalson had mischaracterized the facts in her conversation with him. An excerpt from PTC's log of communications with Mr. Banks that summarizes this conversation is attached hereto as Exhibit H.

29. I spoke to Mr. Banks again about seven weeks later, on March 30, 2007. During our conversation, Mr. Banks told me that he had tried to counsel Mrs. Gunvalson to the best of his ability, but given the limited information he had, he was not in a position to make any judgments or give Mrs. Gunvalson any advice. An excerpt from PTC's log of communications with Mr. Banks that summarizes this conversation is attached hereto as Exhibit I. Also during this conversation, Mr. Banks commended PTC for its open and honest communication style with Mrs. Gunvalson. *See* Ex. I.

30. About two weeks before my second conversation with Mr. Banks, on March 12, 2007, I responded to another request from Mrs. Gunvalson about access to PTC124 for her son. I again reminded her that the clinical data we had at that time did not support making the drug available to DMD patients outside of the clinical trial setting:

> At this point, considering the information available to us, our only plan for any form of pre-approval drug access is after the enrollment of Phase 3 patients. At that point we feel we will have had the opportunity to discuss the Phase 3 plan with the FDA, including the safety and dosing data, and that we would be in a position to consider requests such as yours.
> I hope this is clear to you, our position continues to be the same. If anything, the extensive research we continue to conduct about pre-approval drug access (including the perspective from Drs. Banks and Katz) supports our strategy.
>
> We are eager for next steps, but we simply can't move ahead of the clinical data.

A copy of my March 12, 2007 email to Mrs. Gunvalson is attached hereto as Exhibit J.

31. Finally, on November 27, 2007, I participated in another call with Mrs. Gunvalson about granting Jacob access to PTC124. Per company policy, I created a summary of that call shortly after it ended. That summary is set forth below:

> Spoke to her via conference call with Diane to explain there are no firm plans for a trial for patients who dont qualify for the Phase 2B study, but we continue to discuss the topic and will keep her informed.

An excerpt from PTC's log of communications with Mrs. Gunvalson containing this summary is attached hereto as Exhibit K.

32. Following this telephone call, I have had very limited contact with Mrs. Gunvalson as a result of a restructuring of my job responsibilities.

Jacob's Enrollment/Non-Enrollment In Phase 2a Clinical Trials for PTC124

33. When the opportunity for Jacob to seek enrollment in the Phase 2a clinical trial for PTC124 presented itself in 2005 and again in late 2006, I did not tell Mrs. Gunvalson to keep her son on gentamicin. Nor did I at that time, or any other, promise her that Jacob would be enrolled in future clinical trials for PTC124.

34. Phase 2a trials for PTC124 were open only to boys and young men with a DMD diagnosis, and not boys and young men with a BMD diagnosis. These trials were conducted in three stages. First, clinical investigators administered the drug to a very small number of ambulatory boys – only 6 – with DMD at a low dose for a period of 28 days. Second, a medium dose of PTC124 was administered to 20 ambulatory DMD patients for a period of 28 days, and finally, a high dose of PTC124 was administered to a group of 12 ambulatory and non-ambulatory DMD patients for a period of 28 days. The decision to include non-ambulatory participants with DMD in the last stage of the Phase 2a trials was made after PTC concluded that

there was a strong scientific rationale for evaluating the proper dose of PTC124 in heavier patients than had been included in the first two stages of the trial. Because including heavier patients necessarily meant including older patients, the trial was opened to non-ambulatory participants.

A. *I Told Mrs. Gunvalson that She Should Decide Whether to Remove Jacob from Gentamicin to Participate in the Phase 2a Trials*

35. Before enrollment for the Phase 2a trials for PTC124 in DMD patients began, I spoke to Mrs. Gunvalson about the trials. Some time in the middle of October 2005, Mrs. Gunvalson described to me at length the pros and cons from her perspective, of pursuing enrollment in the Phase 2a trials. Mrs. Gunvalson told me that she was reluctant to pursue enrollment because it meant that she would have to take Jacob off of gentamicin which she believed was benefiting her son. Mrs. Gunvalson also told me that she was concerned that following the period off of gentamicin, Jacob could still be determined not to be eligible for the study if his biopsy results showed a large amount of dystrophin. I told Mrs. Gunvalson that she should work with Jacob's treating physician, Dr. Parkin, to address her concerns about whether enrollment in this 28-day trial was the right decision for her family if Jacob was truly benefiting from taking gentamicin. I suggested that Dr. Parkin work with DMD/BMD experts such as Dr. Brenda Wong and Dr. Richard Finkel to help evaluate her concerns about taking Jacob off gentamicin. I was sympathetic to Mrs. Gunvalson's concerns about disrupting a treatment she believed benefited her son, and frank about the fact that doing so would not guarantee long-term access to PTC124. However, at no point during this conversation did I instruct Mrs. Gunvalson that Jacob should not participate in the trial. Rather, I consistently advised her that she needed to make the participation decision in consultation with her family and Jacob's physicians.

36. On October 31, 2005, I sent Mrs. Gunvalson an email that attached the enrollment criteria for the Phase 2a trials. In that email, I reiterated to Mrs. Gunvalson that, assuming Jacob was eligible for the trial, the decision of whether or not the benefits of enrolling Jacob in the trial outweighed the potential consequences of discontinuing gentamicin therapy was hers to make. I wrote:

> We enclose the detailed criteria for enrollment from the protocol for the Phase 2 trial of PTC124 for DMD.
>
> * * *
>
> Please discuss the enclosed criteria with Jacob's treating physician, who should be able to help you determine if Jacob may qualify. *As we had discussed by phone, **you will need to make the decision of whether, assuming Jacob does fit the criteria, it is worth discontinuing gentamicin treatment for a four-week treatment of PTC124.***

A copy of my October 31, 2005 email to Mrs. Gunvalson is attached hereto as Exhibit L (emphasis supplied).

37. Mrs. Gunvalson and I next communicated about the possibility of enrolling Jacob in the Phase 2a trial at the end of 2006, after the trial was opened to non-ambulatory participants. I do not understand how the Gunvalsons can claim that I had anything to do with the decision not to seek to enroll Jacob in the Phase 2a trials at this time, especially in light of the emails she sent to me in December of 2006 and January of 2007.

38. On December 8, 2006, Mrs. Gunvalson forwarded me a message she had sent to Pat Furlong the same day stating "I got a call from Dr Finkle telling me he did not select Jacob for the trial." A copy of Mrs. Gunvalson's December 8, 2006 email to me is attached hereto as Exhibit M. Dr. Finkel was the primary investigator at one of the sites for the Phase 2a trials and, as such, had ultimate discretion as to whether or not to include Jacob as a trial participant at that site, subject to the formal eligibility requirements imposed by the protocol for that trial.

39. In another email I received from Mrs. Gunvalson on December 8, 2006, she stated that she was very concerned that the Phase 2a trial "might be the last chance for [Jacob] to be in a long term trial without placebos." A copy of Mrs. Gunvalson's second December 8, 2006 email to me is attached hereto as Exhibit N.

40. Subsequent to that, Mrs. Gunvalson informed me and others on January 30, 2007 that Jacob could not participate in the Phase 2a trials "due to his dystrophin production." A copy of Mrs. Gunvalson's January 30, 2007 email is attached hereto as Exhibit O.

B. ***My Statements Concerning the Impact of Jacob's Non-Participation in the Phase 2a Trials***

41. I understand the Gunvalsons are also claiming that I told Mrs. Gunvalson that the failure to enroll Jacob in the last stage of the Phase 2a trial would not have any adverse effect on Jacob in terms of enrollment in upcoming clinical trials for PTC124. These allegations are not accurate. PTC had communicated to the community that participants in those clinical trials could have priority in terms of future trial enrollment; and that non-enrollment would not preclude future participation in all of the future trials for PTC124 that were contemplated at the time, provided that patients satisfied the requisite enrollment criteria, which may be different for each trial. At that time, there was no determination as to what the eligibility criteria for the future trials might be, but it was understood that those trials would be larger than their predecessors, and therefore include patients who had not participated in those earlier trials.

42. The fact that Jacob is ineligible to participate in the controlled Phase 2b trials PTC is now conducting is entirely consistent with this message. Jacob's ineligibility for the Phase 2b trials has nothing whatsoever to do with his non-participation in the Phase 2a trials. Rather, Jacob cannot participate in the Phase 2b trials because is no longer ambulatory and the study is designed, in part, to measure the effect of PTC124 on ambulation.

43. Finally, it bears mention that PTC has always been, and remains, fully committed to exploring ways to make PTC124 available to boys like Jacob who cannot, for one reason or another, participate in clinical trials for the drug, but I have always indicated such consideration would take place at the appropriate time.

Providing Access Only to Jacob Would be Irresponsible and Unfair

44. While I am extremely sympathetic to the Gunvalsons' concerns for Jacob, it would be wholly irresponsible and unfair for PTC to make PTC124 available to Jacob simply because the Gunvalsons have filed this lawsuit.

45. In addition, it would be unfair to the other families whose children also do not qualify for the current ongoing trials for PTC124, some of who are in far more advanced stages of DMD/BMD than Jacob, if we were to make PTC124 available to Jacob and not to them. I cannot imagine how we could justify making PTC124 available to Jacob and continuing to withhold it from the many other children and young men (some of whom are in much worse health than Jacob) who would also like access to the drug. I believe this type of decision needs to be made on scientific and clinical considerations, not legal or political grounds.

Funding for PTC124 Research

46. Finally, I understand that the Gunvalsons are claiming that Mrs. Gunvalson was instrumental in encouraging PTC to apply for a grant from the National Institutes of Health ("NIH") to fund PTC124. I am not aware of any NIH grants that involved any recommendation or advice from Mrs. Gunvalson.

47. Insofar as the clinical trials for PTC124 that have been conducted to date are concerned, those trials have been funded by various sources, including grants from the Muscular Dystrophy Association, Cystic Fibrosis Foundation Therapeutics, Inc., the FDA's Office of

Orphan Products Development, and the National Center for Research Resources, with the vast majority of funds coming from PTC's private investors. Mrs. Gunvalson was not involved in obtaining any of this funding on PTC's behalf.

48. The $15.4 million NIH grant that the Gunvalsons claim Mrs. Gunvalson was instrumental in helping to procure has nothing to do with PTC124. In 2003, after PTC had commenced preliminary research on PTC124, PPMD and PTC decided to collaborate on additional research into other muscular dystrophy treatments – that is, treatments for forms of DMD/BMD that were not caused by nonsense mutations. Only about 15% of the individuals with DMD/BMD have nonsense mutations and it was hoped that this collaboration, known as Project Catalyst, would foster the development of treatments that could be of use to the other 85% of DMD/BMD patients. The recently awarded $15.4 million NIH grant to the University of Pennsylvania will help fund this additional research. None of the proceeds from this grant will be used to fund PTC124 research, and I do not believe Mrs. Gunvalson had any involvement in securing this grant.

49. Finally, to the extent that the Gunvalsons are suggesting that they provided any direct funding to PTC for PTC124, this too is inaccurate.

50. Although the entire DMD/BMD community appreciates the efforts and involvement of patient families, including the Gunvalsons, the idea that those efforts or Mrs. Gunvalson's relationships with Congressmen or women should entitle Jacob to preferential

treatment over others is a proposition that both PTC and I personally strongly and resoundingly reject.

Cláudia Hirawat

Sworn to before me this
12 day of August, 2008

~~Notary Public~~
An Attorney of the State of New Jersey

Exhibit A

Events

Conference 2009

Genetics Day 2008

Film Screening

Conference 2008

Program
Awards
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Session Materials

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Genetics Day 2007

Gala 2006

Genetics Day 2006

Conference 2006

Conference 2005

Conference 2004

Previous Award Winners

TRANSFORMATIONAL LEADERSHIP

GENETIC ALLIANCE 2008 ANNUAL CONFERENCE JULY 11-13th, 2008

Celebrate the 2008 Genetic Alliance Award recipients:

Art of Transformational Leadership
As Francis Collins leaves his position at National Human Genome Research Institute (NHGRI), Genetic Alliance honors his compassionate, visionary leadership. His constant focus on health, his creation of novel partnerships and his commitment to the research commons, are only a few of the attributes we will celebrate!

Art of Transformational Leadership Award Winner
Francis Collins, MD, PhD
National Human Genome Research Institute, Bethesda, MD.

The **Founder's Service Award** was a new award at 2008 conference. It recognizes an individual who has given deeply, consistently and over a long period to Genetic Alliance.

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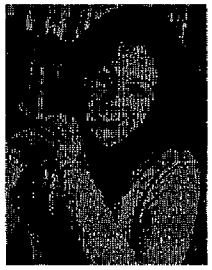
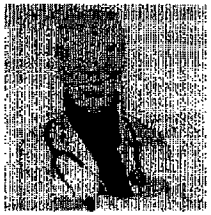
Founder's Service Award Winner
Jannine Cody, PhD
Chromosome 18 Registry, San Antonio, TX.

The **Art of Advocacy Award** pays tribute to a visionary grassroots leader who is harnessing his or her knowledge and experience to improve the quality of research, healthcare, information and support services for a specificcondition or for a coalition of grassroots organizations.



Art of Advocacy Award Winner
Clare Dunsford, PhD
Boston College, Boston, MA.

The **Art of Listening Award** honors a health professional who is a caring, receptive professional in the lives of individuals and families living with genetic conditions.



Art of Listening Award Winner
Joann N. Bodurtha, MD MPH
Virginia Commonwealth University, Richmond, VA

The **Art of Industry Partnership Award** honors a for-profit Biotechnology, Pharmaceutical, or genetics company whose track record models the benefits of creative partnerships between consumer advocates and industry to advance understanding and treatment of genetic conditions, disorders, and diseases.

Art of Industry Partnership Award Winner
PTC Therapeutics, Inc.
Plainfield, NJ

The winners were honored at the Genetic Alliance 2008 Annual Conference: *Transformational Leadership* at the Saturday awards banquet, on Saturday, July 12th, 2008 at the Bethesda North Marriot in Rockville, Maryland.

Graphic Design by
Melissa Allen Design

4301 Connecticut Avenue NW - Suite 404
Washington, DC 20008-2369
Tel: 202.966.5557 Fax: 202.966.8553
info@geneticalliance.org

The Combined Federal Campaign changed the numbers for organizations. Genetic Alliance's NEW CFC is 80146.
Please notify your workplace that your donations should go to this new number. Thank you!

Exhibit B

From: Cheri Gunvalson [mailto:cgunval@gvtel.com]
Sent: Monday, February 20, 2006 12:51 PM
To: Katz, Russell G
Cc: Cheri Gunvalson
Subject: Single Patient IND for Compassionate Use

Dr Russell Katz
Director, Neuropharmacological Drug Products
Food and Drug Administration, FDA 120
1451 Rockville Pike, Room 4037
Rockville, MD 20852

Dear Dr Katz,

Our son Jacob has a terminal form of Duchenne Muscular Dystrophy. He doesn't qualify for the PTC 124 Ph II trial.
http://www.ptcbio.com/big/indexhome.html This drug holds great promise for a subset of boys with a premature stop codon. Jacob has a premature stop codon on exon 5. There is no other treatment available for this deadly disease.

The soonest this drug might be available (with everything perfect in the FDA approval process) is mid to late 2007 if there is a phase
III side trial for those boys not eligible for the regular trial.Jacob was 14 in October and is still ambulatory for short distances. He's deteriorating and will not be ambulatory in mid to late 2007. I am sure you are fully aware of not only the medical but also psychosocial problems that face Jacob as he deteriorates from this deadly disease.

We would like to apply for a single patient IND for compassionate use of this drug PTC 124. As you know the first step is to approach the drug company. I believe if you could you provide assurance to the drug company similar to the response by Dr Crawford below, the drug company would be much more willing to consider our request. I have attached the letter to PTC.

"The FDA, categorically, does not attach special significance to adverse events reported from such expanded access programs as Ms. Bacon has tried to join. We recognize that these programs involve less-controlled use of new drugs, and we assess the reported data accordingly. The development of a new medication is not slowed by side effects occurring outside clinical trials".

What ever assistance you might be in this would be most appreciated!

If you have any questions feel free to contact myself or Jacob's physician, John Parkin MD at johnparkin@meritcare.com or
218-333-4710.

Respectfully Yours,

Cheri Gunvalson,

cgunval@gvtel.com

Wall Street Journal Letter to the Editor December 20, 2002

To the Editor: Cancer patient Edie Bacon's letter (November 29, 2002) about her inability to obtain a potentially life-saving experimental drug was poignant and her deep concern about her lack of access to a treatment that she believes to be beneficial is understandable. The policies of the Food and Drug Administration, however, are not an obstacle to the use of experimental treatments outside clinical trials. Such use would not prompt, as Ms. Bacon appears to believe, an FDA request for additional studies. The FDA, categorically, does not attach special significance to adverse events reported from such expanded access programs as Ms. Bacon has tried to join. We recognize that these programs involve less-controlled use of new drugs, and we assess the reported data accordingly. The development of a new medication is not slowed by side effects occurring outside clinical trials. The FDA strongly supports expanded use of promising therapies for serious and otherwise untreatable diseases. In the last two years alone, 10,000 patients with myeloid leukemia have been given access to Gleevec, and Iressa, a medication that's currently being reviewed for lung cancer, has been made available to 15,000 patients. So far, 67 patients have been given outside-trial access to ET-743, the drug that Ms. Bacon wants to use. The FDA encourages patients to participate, when appropriate, in well-designed clinical trials.

Information about studies is available at http://clinicaltrials.gov. To obtain an experimental drug under expanded access, patients should first contact the manufacturer. If the result is unsatisfactory, our Office of Special Health Issues (301/827-4460) can often provide helpful information about access to experimental drugs.

Lester M. Crawford, D.V.M., Ph.D.,
Deputy Commissioner, FDA

Exhibit C

Dear Mrs. Gunvalson,

I am writing in response to your inquiry concerning the possible availability of PTC 124 under a single patient IND to treat your son, Jacob, for Duchenne Muscular Dystrophy. I am sorry to hear of your son's progressive difficulties despite his current therapy with gentamycin.

As you know, a new potential therapy such as PTC 124 is ideally first given to patients as part of a clinical trial. From your inquiry, I understand that your son did not meet the inclusion criteria for the clinical trial now in progress for the treatment of Duchenne muscular dystrophy. In my opinion, the best option for your son would be to request a protocol exception. If approved by the sponsor, a protocol exception allows some patients who are ineligible to participate in a study to be treated with the investigational drug under the existing study IND at one of the study centers participating in the trial. Thus, the new therapy is given under the supervision of a study investigator who has had at least some experience with this new therapy and who will be informed by the sponsor of the ongoing clinical experience of the other study investigators conducting the trial of the new therapy. The dose and length of treatment allowable would depend on safety information currently available and would not exceed those allowable under the study protocol.

The alternative to the protocol exception would be the single patient IND which also requires the approval of the sponsor. In general, the FDA is supportive of a physician's filing a single patient IND to allow treatment with an investigational drug of a patient with a serious illness when no alternative effective therapy exists, when clinical studies of the drug are ongoing, and when a sponsor agrees to provide the drug. The length of treatment allowable under the single patient IND would again depend on the safety information currently available.

The physician who would propose to treat your son under either the protocol exception mechanism or the single patient IND mechanism should contact Katherine Needleman, the Consumer Safety Officer in our Division who is responsible for PTC 124. She will work with the physician to explain and assist in the process. Ms Needleman's maybe contacted by email katherine.needleman@fda.hhs.gov or by phone 301-796-1125.

An explanation of the single patient IND is posted on the FDA internet public website (http://www.fda.gov/ola/2001/compassionateuse0620.html) and may be useful in your understanding this type of IND. It also briefly discusses protocol exception mechanism.

The following excerpts from this explanation are specifically relevant to your request:

Background

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 are 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 can be justified. Physicians may always contact FDA to propose such a use for a specific patient when they believe circumstances warrant this use. . . .

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.

I hope that this information is helpful to you and to your son.

Sincerely,

Exhibit D

From: "Hirawat, Claudia " <chirawat@ptcbio.com>
To: "Cheri Gunvalson" <cgunval@gvtel.com>
Sent: Friday, August 11, 2006 12:34 PM
Subject: RE: Hi

Hi Cheri,

Thank you so much for the photos again, they are great!!!

Here are some talking points:

- Phase 2 data is expected before the end of 2006
- Phase 3 is expected to start mid-2007
- PTC hopes to work with FDA and patient groups to design a program that would allow pre-approval drug access for the patients who do not qualify for participation in the study. We don't know what the criteria for participation would be, so we need the design of Phase 3 to be in place, and agreed upon with the FDA before we can pursue such a project, but it is a priority for us.
- This is an exciting project because it attempts to address the cause of the disease directly as opposed to just treat symptoms. As a drug that is first in class and first in disease, the development process is challenging, but PTC has a very experienced clinical development team with a long and well established track record of success (and we are all fans of Jacob!!!).

Hope this is helpful!

Warmest wishes,

Cláudia

Exhibit E

From: "Hirawat, Claudia " <chirawat@ptcbio.com>
To: "Cheri Gunvalson" <cgunval@gvtel.com>
Cc: <John.Parkin@meritcare.com>; <finkel@email.chop.edu>; "Miller, Langdon " <lmiller@ptcbio.com>
Sent: Tuesday, January 30, 2007 4:17 PM
Subject: RE: Following up regarding Jacob G.

Hello Cheri,
Yes, we are familiar with this document and believe that there are positive changes being made in this area, which of course we monitor closely. As we discussed yesterday, while this topic is a great priority to us, we don't have a developed plan for pre-approval drug access (whether it is expanded access or any other form such as an investigator-initiated IND) at this time. There are several elements of the development of PTC124 that would need to be addressed, including:
- determining the best dose (studies ongoing)
- determining the enrolment criteria for next studies, which would tell us which patients would be included or excluded of future trials (these efforts are ongoing)
- securing agreement from the regulatory agencies about the design of the study, including inclusion/exclusion criteria and end points, which would allow us to determine how many patients would be required for the trial as well as feedback regarding the safety data (at this point no patient has received PTC124 longer than 28 days).
Until this information is available to us, we are not in a position to move forward with any form of pre-approval drug access. As we discussed, this type of program is typically conducted during Phase 3, and in most instances when the enrollment for the trial has been completed. I realize the waiting is very difficult, it is very difficult for us too, but we just can't move ahead of the science or the safety data. We are working diligently to advance this in the best possible manner.
I am always here if you want to talk!
All the best,
Cláudia

Exhibit F

From: CHirawat@ptcbio.com
Sent: 2/2/2007 5:33:34 PM
To: cgunval@gvtel.com
CC: John.Parkin@meritcare.com;finkel@email.chop.edu;?????????;
BCC: ????????m;?????????m;
Subject: RE: Following up regarding Jacob G.

Dear Cheri,
Sorry it took me a couple of days to get you a more detailed response. I was traveling. The study you are referring to will not be completed in the next few weeks, but we anticipate its completion within the next few months. Even if the target plasma concentrations are achieved, that does not demonstrate activity of PTC124. In order to assess activity, a significant amount of additional work is required which includes reviewing the biopsies as well as analyzing all of the other data collected during the trial. The analysis of all of these results will hopefully allow us to characterize the activity of PTC124 at the higher dose and allow for the planning of longer-term studies. We are currently working with our advisors to develop the regulatory strategy for PTC124 (what are the next studies, duration/end-points, etc.) and hope to meet with the FDA within the first half of 2007. This meeting should provide us guidance for the path forward for PTC124 and we would anticipate being able to provide you a better sense of a timeline after these discussions take place. We will contact Mr. Banks as you suggested, because we are always interested in studying mechanisms for pre-approval drug access, but must emphasize that at this time we don't have enough safety or efficacy data to support your request for continuous treatment with PTC124.
Please to let me know if you have any additional questions.
Warmest wishes,
Cláudia

Exhibit G

From: Cheri Gunvalson [mailto:cgunval@gvtel.com]
Sent: Tuesday, January 30, 2007 6:16 PM
To: Hirawat, Claudia
Cc: John.Parkin@meritcare.com; finkel@email.chop.edu; Miller, Langdon
Subject: Re: Following up regarding Jacob G.

Hello,
In the next couple of weeks when you finish the higher dose boys I believe you will have a good idea on dosage. Jacob produces some dystrophin which is truncated and 1/2 the size of normal dystrophin (on biopsy at dx) he so he cannot be in the trial. Since he cannot be in the trial there is no value for him to have to wait until all of the boys have been enrolled into ph2b or 3 in 9-12 months. He will not be walking at that time. I would encourage you visit with David Banks in the office of special access at the FDA 301-827-4460. He is the one who gave me the web site and told me that special access is granted often at this sage of the clinical trial process and Jacob's situation would fit the criteria. All I am asking is for access to the drug so we could attempt a single pt IND with all the same controls as the boys in the trial. If this is not in Jacobs best interest the FDA will turn us down but we don't know until we try. I believe this might even help you with the next steps you need to take with the FDA.
Cheri

Exhibit H

2/6/2007 12:06 PM CHIRAW INCALL Incoming Phone Call CMPD 3/30/2007
returning my call. He was wonderful, he had not understood the stage of the drug, had a very different impression from this discussin with Cheri. In short, he supports our strategy and agrees with our decisions. he was just great

Exhibit I

3/30/2007 12:17 PM CHIRAW OUTCAL Outgoing Call CMPD 3/30/2007
called him again based on Cheri's latest letter to Langdon and my conversation with her, where she made it sound like Mr. Banks supports the strategy of a single patient IND and in fact had told her this is easy to do, it is done all the time, it takes about one week to process. Explained the context to Mr. Banks, apologized for bothering him, but explained we want to do a great job at this and if has any advice we would welcome his thoughts. He was surprised by what Cheri had communicated. He said he is not in a position to make any judgment on this process, and does not "even have enough information for which to base an opinion." I typed his actual words: "I have tried to counsel her to the best of my ability but from a very limited range of issues that I can even speak to because, you know, I don't know. So I want to be clear about this, that I am not making judgments or even advising her on what I think should happen or even could happen at this time or at any time, because I don't know." I explained we understand his position completely, just wanted to circle back with him because she asked us to. "I told her that I am astounded by the openness with which your firm is dealing with her, this is way beyond what I have experienced dealing with other manufacturers and I also told her that the issues you had raised when you spoke regarding the regulatory and scientific concerns regarding the current status of the development of this drug, that I thought it was quite possible that some of those issues remained unresolved and could potentially preclude use of this drug beyond its current sphere of use. So I want to be cleat about this, I have talked to her, but I have respected the limits of my knowledge and my authority." he goes on: "I am thankful that you are dealing with somebody like Dr. Katz who is a brilliant guy and a long time good friend of mine and I know you will be well advised by him, I question my capacity to add value beyond what he can do, but I am at your service." He said he understands we have a very tough job, and congratulated us on taking this approach of being open communications, he encouraged us to keep the agency abreast of our efforts so they can appreciate how hard we are working on helping patients understand where we are in the process.

Exhibit J

From: "Hirawat, Claudia " <chirawat@ptcbio.com>
To: "Cheri Gunvalson" <cgunval@gvtel.com>
Cc: "Goetz, Diane" <dgoetz@ptcbio.com>
Sent: Monday, March 12, 2007 9:53 AM
Subject: RE: Thinking about you

Hi Cheri,
We don't have another year of safety data, patients have only been exposed to the drug for 28 days; we also do not have additional dosing information, we are conducting those studies now. At this point, considering the information available to us, our only plan for any form of pre-approval drug access is after the enrollment of Phase 3 patients. At that point we feel we will have had the opportunity to discuss the Phase 3 plan with the FDA, including the safety and dosing data, and that we would be in a position to consider requests such as yours. I hope this is clear to you, our position continues to be the same. If anything, the extensive research we continue to conduct about pre-approval drug access (including the perspective from Drs. Banks and Katz) supports our strategy.
We are eager for next steps, but we simply can't move ahead of the clinical data.
Please let me know if you would like to discuss it by phone.
All the best,
Cláudia

Exhibit K

11/27/2007 06:23 PM CHIRAW NOTES Notes CMPD 11/27/2007
Spoke to her via conference call with Diane to explain there are no firm plans for a trial for patients who dont qualify for the Phase 2B study, but we continue to discuss the topic and will keep her informed.

Exhibit L

From: Claudia Hirawat
Sent: Monday, October 31, 2005 8:17 PM
To: 'cgunval@gvtel.com'
Cc: Kerri Donnelly
Subject: Criteria for enrolment

Dear Cherie,
We enclose the detailed criteria for enrollment from the protocol for the Phase 2 trial of PTC124 for DMD.
We have also spoken to our clinical team about the protocol change you mentioned. They have informed us that no amendments have been made to the enrollment criteria. Please discuss the enclosed criteria with Jacob's treating physician, who should be able to help you determine if Jacob may qualify. As we had discussed by phone, you will need to make the decision of whether, assuming Jacob does fit the criteria, it is worth discontinuing gentamicin treatment for a four-week treatment of PTC124. Provided of course, that the timelines would work out.
Independently of the PTC124 trial, Dr. Brenda Wong said she would be wiling to see Jacob and try to help determine/quantify his response to gentamicin. She can be contacted at 513-636-4222, brenda.wong@cchmc.org.
As for the CF trial, patients may receive inhaled tobramycin, as tobramycin is considered a standard of care for this disease. Also, current data indicates that tobramycin does not suppress nonsense mutations.
I hope I was able to answer your questions. As always, please feel free to contact me with any additional questions.
All the best and our warmest wishes to you and Jacob,
Claudia
Cláudia Hirawat
Vice President
Corporate Development
PTC Therapeutics, Inc.
100 Corporate Court
South Plainfield, NJ 07080
Tel: 908.222.7000 x106
Fax: 908.222.7231
Cell: 908.294.8399
CHirawat@ptcbio.com
www.ptcbio.com

Exhibit M

From: Cheri Gunvalson [mailto:cgunval@gvtel.com]
Sent: Friday, December 08, 2006 11:17 AM
To: Hirawat, Claudia
Subject: Fw: Jacob /Trial

Claudia,
Thank you for your help! I'm sorry I was a mess yesterday. Other than Jacobs diagnosis this news has been the worst. Please let me know if there is anything else I can do! I know this is not easy for you and I truly appreciate all you have done for Jacob!
Cheri

----- Original Message -----
From: HYPERLINK "mailto:cgunval@gvtel.com"Cheri Gunvalson
To: HYPERLINK "mailto:PatFurlong@aol.com"PatFurlong@aol.com
Sent: Friday, December 08, 2006 9:40 AM
Subject: Jacob /Trial

Pat,
I can't begin to tell how hard it is for me to share this with you. While you were flying yesterday I got a call from Dr Finkle telling me he did not select Jacob for the trial. He asked me Jacobs DOB, who his MD was and seemed surprised Dr Day had seen him and that he was walking at age 15. I don't believe he ever ordered or reviewed Jacobs file. He said he had enough kids in his practice to be in the trial and that he knew them well which made it easier to monitor side effects. I offered to stay in Philly during the trial with Jacob and he said that wasn't necessary. I called Claudia and Lee and it is my understanding you all will be together this weekend if you can brain storm on what to do next. They think the best thing would be for you to call Brenda Wong and see if Jacob could get in there. If that doesn't work now that there is data would PTC release the drug for expanded use?
I will be home until 12:30 CST 218487-5788. Then on my cell. 218-556-6980. at 2:15 on I will be with the kids and they do not know about this and I cannot talk about it with them in the car.

Exhibit N

From: cgunval@gvtel.com
Sent: 12/8/2006 12:42:57 PM
To:
CC:
BCC:
Subject: Re: Jacob /Trial

Claudia,
It is not the 28 day that worries me so much but it is my understanding that group might be the last chance for him to be in a long term trial without placebos. His walking has been getting notably worse and it is very hard to see this knowing there is a drug that could stop the deteriation and placebos will make this worse. He doesn't want to talk about this but he is very smart and knows it is happening. Its not a question of if but when without intervention he will be in a chair full time. This is a great worry to Jacob and I could type all day and not be able to articulate it well enough but for example he is very modest and the thought of someone having to toilet him is very hard.
Cheri

Exhibit O

From: Cheri Gunvalson [mailto:cgunval@gvtel.com]
Sent: Tuesday, January 30, 2007 1:53 PM
To: cgunval@gvtel.com; Hirawat, Claudia ; finkel@email.chop.edu
Cc: cgunval@gvtel.com; John.Parkin@meritcare.com
Subject: RE: Following up regarding Jacob G.

Hello,
If possible could you please take a look at
http://www.fda.gov/bbs/topics/NEWS/2006/NEW01520.HTML
It is my understanding that since Jacob cannot be in the trial due to his dystrophin production he would be eligible for expanded access now and not have to wait the 9-12 months till the other boys were all enrolled in ph 2b or 3.
Cheri