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 (WJM) (MF)
- against -	:	
PTC THERAPEUTICS, INC., Defendants.	:	

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AFFIDAVIT OF DIANE M. GOETZ

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

DIANE M. GOETZ, being duly sworn, deposes and says:

1. I am the Director of Patient and Professional Advocacy 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 investigative 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.

3. I joined PTC as Director of Patient and Professional Advocacy in February 2007. In that capacity, I serve as a liaison between PTC and organizations focused on the various medical conditions that the drugs developed by PTC are designed to treat. These organizations provide pharmaceutical companies like PTC with valuable information concerning what it is really like to live with a disease or medical condition. Often, they provide a valuable source of input during the stages of drug development. Another large part of my job and that of people I supervise is answering phone calls and emails from parents and occasionally patients about PTC124 and our clinical trials. It is our approach to be honest and open with families, and to attempt to maintain their hope without raising unrealistic expectations.

4. Since I joined PTC, I have had a number of communications with Cheri Gunvalson on the issue of granting pre-approval access to PTC124 to her son, Jacob. I have told Mrs. Gunvalson on several occasions that the company cannot make a special exception for Jacob and give him PTC124 outside the formal clinical trial setting. It is clear to me that Mrs. Gunvalson has had difficulty accepting what I have told her. Although Mrs. Gunvalson is not unique among the various parents with whom I have dealt, all of whom want to explore every potentially helpful avenue for their children, Mrs. Gunvalson is unique in her insistence that Jacob is different from other children and therefore a more deserving candidate for pre-approval access to PTC124 than others.

5. I understand that the Gunvalsons are claiming that at some time in late 2007 I told them that Jacob would have access to PTC124. Although I had numerous communications with

Mrs. Gunvalson during that timeframe, I have never promised her access to PTC124. To the contrary, I have always been quite candid with Mrs. Gunvalson that, although the company was very interested in exploring ways to make PTC124 available to Jacob and all of the other boys and young adults with DMD who were unable to participate in clinical trials for the drug, the company had not yet adopted any formal program to provide such pre-approval access and that safety considerations as well as overarching fairness concerns precluded granting preferential access to Jacob.

6. On December 21, 2007, I spoke to Mrs. Gunvalson on the phone to discuss pre-approval access to PTC124 for Jacob. After that call, I created the following note in PTC's communications log:

> As we agreed, I spoke to her to give her an answer about why we couldn't start the study that could provide Jacob with drug as soon as we submitted our intent to the FDA. I explained that it wasn't so simple. It was a question of finding the best path forward, the right study design. I explained that we hadn't yet started the 2a extension study and that anything we might be able to do would have to be in the context of a study and that it would be somewhat dependent on how the extension study procedes. We agreed to speak at the end of January.

A copy of the excerpt from PTC's communications log with the Gunvalsons containing this entry is attached hereto as Exhibit A. The 2a extension study referred to in my email is a two-year clinical study in which only those boys and young men who previously participated in an initial 2a clinical trial for PTC124 and received one of three doses of the drug for a period of 28 days are eligible to participate. Because Jacob Gunvalson did not participate in the initial trial, he is ineligible for this study.

7. It is my understanding that the Gunvalsons are claiming that the reason they elected not to have Jacob participate in the initial 2a trial is because PTC executives told Mrs. Gunvalson not to enroll him and encouraged Mrs. Gunvalson to continue treating Jacob with

gentamicin, an antibiotic that is known to be toxic at higher doses. I am very surprised that the Gunvalsons are making this claim. In one of the calls I had with Mrs. Gunvalson at the end of 2007, she was very emotional about the fact that she had elected not to enroll Jacob in this trial, and expressed regret about the decision. At no point during this call did Mrs. Gunvalson state or suggest that anyone at PTC had any role in this decision, and it was quite clear to me that Mrs. Gunvalson was expressing regret over a decision that she herself had made. Mrs. Gunvalson never told me that Dr. Finkel, the primary investigator for the trial, or Cláudia Hirawat, PTC's Senior Vice President, Corporate Development, played any role in the decision not to enroll Jacob in the trial at this or any other time.

8. On December 30, 2007, I responded to another question from Mrs. Gunvalson regarding where things stood in terms of Jacob getting pre-approval access to PTC124. My response is set forth, in pertinent part, below:

> We are trying to figure out whether it would be possible for Jacob and other boys who do not qualify for the 2b study to participate in another study. We won't be able to determine that until we have a better idea of what the 2a extension study will be. As we have recently announced, we are close to initiating the Phase 2b study. We have begun to plan for the extension study but we cannot move ahead with that until the 2b study is launched. That is about as specific as I can be at this point. As for your second question, I'm sorry but I can't answer that until I talk to the clinical team about the exact definition and implications of the term "protocol exception," as it is not one with which I am familiar. It sounds to me like another way of saying "single patient IND," which we have already discussed and which I have explained would not be possible philosophically or practically.

A copy of my December 30, 2007 email to Mrs. Gunvalson is attached hereto as Exhibit B.

9. On January 1, 2008, Mrs. Gunvalson replied to me again asking why a protocol exception could not be made for Jacob. She told me that she had received a letter from Dr. Russell Katz at the FDA suggesting that Jacob could be enrolled in a clinical trial for PTC as a protocol exception. Since that time, I have reviewed Dr. Katz's letter. In his letter, Dr. Katz

summarizes certain FDA guidance concerning pre-approval drug access programs. This guidance makes abundantly clear that pre-approval access is conditioned on the consent of the drug sponsor, in this case, PTC, to provide the drug. Nevertheless, it was clear from Mrs. Gunvalson's email that she had interpreted Dr. Katz's letter as FDA approval for pre-approval access to PTC124 for Jacob. In my response, I tried to explain to Mrs. Gunvalson that, although the company could not agree to provide pre-approval access to Jacob at that time, we were hopeful that we would be able to provide Jacob and all of the other DMD boys and young men who did not have access to PTC124 with a better solution at some point in the future. Copies of Mrs. Gunvalson's inquiry and my response are set forth below:

> Dear Diane,
> My understanding from Dr Katz's email is a protocol exception is that Jacob could be in the 2a trial as a protocal exception ie an exception to the protocal. It is my understanding the FDA wants saftey data on the drug in as many patients as possible in a controlled setting such as the 2a trial so they can evaluate more fully the saftey of the drug. Especially in cases like this when it is a rare subgruop. So it is win win for the FDA to gather more saftey data and for the patient. It is my understanding the FDA is trying to get drug companies to use this avenue more. David Banks from the FDA told me some time ago that he believed that Dr Katz would consider an expidited review of our case for a protocal exception.
> Sincerely,
> Cheri
>
> Dear Cheri,
> I was reading the Wall Street Journal on the way to work today and there was an ad for a book called The Power of a Positive No. One of the blurbs on this book was written by Jim Collins (author of Good To Great), who said, "Ury [the author] teaches us how to say NO - with grace and effect - so that we might create an even better YES." I haven't read the book so I'm not sure how to say no to a protocol exception with grace and effect, but I can definitely tell you we're saying no because we're trying to create an even better yes. When it's all over you'll understand exactly what I mean and why, but we're not there yet. I know it's hard for you but please hold tight. The best path forward is still very much on the agenda here.
> Best,
> Diane

A copy of my email correspondence with Mrs. Gunvalson in early January 2008 is attached hereto as Exhibit C.

10. I understand recently there has been some suggestion that my response was somewhat ambiguous, using both the terms "no" and "yes." However, as set forth below, it is very clear to me that, at the time she received my message, Mrs. Gunvalson understood that I was communicating a denial of her request for pre-approval access to PTC124 for Jacob. The better "yes" I was referring to would be when, either through additional clinical trials or pre-approval access, it would be safe and possible to make PTC 124 available to Jacob and other patients who had not participated in a trial.

11. On January 14, 2008, I received an email from Bettilou Taylor, the staff director of the Senate Labor, Health and Human Services Appropriations Committee on behalf of Mrs. Gunvalson. Ms. Taylor's email to me is reproduced below:

> Diane -- I read with much regret -- you e-mail to Cheri. I know that these decisions are difficult ones -- can you tell me why you won't grant a protocol exception for Jacob? I also would like to know why Cheri thought that Jacob was going to get the drug -- she had called all of her friends and was so excited that PTC finally decided to let him have the drug and then the let down that it was not going to happen. I understand from my discussions with the FDA that they are encouraging companies to grant exceptions to provide as much info as possible about the side effects from the drug to a variety of patients. I know that this is difficult for all involved -- but I am trying to understand. As you can imagine when the Senators get involved – they want to know all of the ins and outs of the issue.
>
> Thanks again for taking time to be so kind to us during our visit. Hope you have a wonderful New Year.
>
> BLT

A copy of Ms. Taylor's January 14, 2008 email to me is attached hereto as Exhibit D. In my response, I explained to Ms. Taylor that the company was continuing to explore avenues to make PTC124 available to a wider population of boys and young men like Jacob, but that the company

could not do anything to jeopardize the approval process for PTC124 so that the drug could be available to everyone who could possibly benefit from it. My email to Ms. Taylor is set forth below:

> Dear Bettilou,
> It's nice to hear from you again. Happy New Year to you, too. I'm sorry for the long delay in responding to you. As you might imagine, it is very difficult to do right by all the patients who could potentially benefit from PTC124, and there are many opinions about the best way to accomplish that. One thing we all agree on: the best thing for all the boys is to get PTC124 approved and to market as quickly as possible. That is, and must be, our primary goal. PTC has not changed its position on trying to find the best way to involve Jacob and other boys in similar circumstances in our studies. It continues to be a priority for us. I hope you will support us, as I know Cheri does, in our mission to make PTC124 available, as expeditiously as possible, to all boys who could potentially benefit from it.
> Best,
> Diane

A copy of my January 25, 2008 email to Ms. Taylor is attached hereto as Exhibit E.

12. Finally, on March 17, 2008, in response to an inquiry from Mrs. Gunvalson as to whether there was any news on Jacob getting access to PTC124, I informed her by email, "I don't have any news about yet about what opportunities there might be for boys and young men like Jacob to access PTC124." A copy of my March 17, 2008 email to Mrs. Gunvalson is attached hereto as Exhibit F.

13. As set forth above, at no time did I promise Mrs. Gunvalson or any other member of the Gunvalson family that Jacob would have pre-approval access to PTC124.

14. I have tremendous sympathy for the Gunvalsons and all of the other families with DMD children who desire pre-approval access to PTC124, but I do not believe it would be in any of their interests if Jacob were allowed to obtain the drug because his parents have filed this lawsuit. As I told Mrs. Gunvalson many times, PTC is firmly committed to finding a way that PTC124 can safely be made available to all of these families prior to obtaining formal FDA

approval for the drug, but until that is possible, it would be unfair to grant pre-approval access to PTC124 to Jacob alone.

Diane M. Goetz

Sworn to before me this
7th day of August, 2008

Notary Public

Sonja Benson
Commission expires 12/17/12

Exhibit A

12/21/2007 03:41 PM DGOETZ INCALL Incoming Phone Call CMPD 12/29/2007
As we agreed, I spoke to her to give her an answer about why we couldn't start the study that could provide Jacob with drug as soon as we submitted our intent to the FDA. I explained that it wasn't so simple. It was a question of finding the best path forward, the right study design. I explained that we hadn't yet started the 2a extension study and that anything we might be able to do would have to be in the context of a study and that it would be somewhat dependent on how the extension study procedes. We agreed to speak at the end of January.

Exhibit B

From: HYPERLINK "mailto:dgoetz@ptcbio.com"Goetz, Diane
To: HYPERLINK "mailto:cgunval@gvtel.com"Cheri Gunvalson
Sent: Sunday, December 30, 2007 4:22 PM
Subject: [Spam] RE: 124 2a extension

Hi, Cheri-
We are trying to figure out whether it would be possible for Jacob and other boys who do not qualify for the 2b study to participate in another study. We won't be able to determine that until we have a better idea of what the 2a extension study will be. As we have recently announced, we are close to initiating the Phase 2b study. We have begun to plan for the extension study but we cannot move ahead with that until the 2b study is launched. That is about as specific as I can be at this point. As for your second question, I'm sorry but I can't answer that until I talk to the clinical team about the exact definition and implications of the term "protocol exception," as it is not one with which I am familiar. It sounds to me like another way of saying "single patient IND," which we have already discussed and which I have explained would not be possible philosophically or practically. I could be wrong about this definition, so I want to be sure I understand the term. I will try to discuss it this coming week but I don't know what people's schedules are so I can't give you a time when I will have an answer. I will try my best to get an answer by Friday.
Best,
Diane

Exhibit C

From: dgoetz@ptcbio.com
Sent: 1/4/2008 11:56:54 AM
To: cgunval@gvtel.com
CC:
BCC:
Subject: RE: [Spam] RE: 124 2a extension

Dear Cheri,
I was reading the Wall Street Journal on the way to work today and there was an ad for a book called The Power of a Positive No. One of the blurbs on this book was written by Jim Collins (author of Good To Great), who said, "Ury [the author] teaches us how to say NO - with grace and effect - so that we might create an even better YES." I haven't read the book so I'm not sure how to say no to a protocol exception with grace and effect, but I can definitely tell you we're saying no because we're trying to create an even better yes. When it's all over you'll understand exactly what I mean and why, but we're not there yet. I know it's hard for you but please hold tight. The best path forward is still very much on the agenda here.
Best,
Diane

From: Cheri Gunvalson [mailto:cgunval@gvtel.com]
Sent: Tuesday, January 01, 2008 12:14 PM
To: Goetz, Diane
Subject: Re: [Spam] RE: 124 2a extension

Dear Diane,
My understanding from Dr Katz's email is a protocol exception is that Jacob could be in the 2a trial as a protocal exception ie an exception to the protocal. It is my understanding the FDA wants saftey data on the drug in as many patients as possible in a controlled setting such as the 2a trial so they can evaluate more fully the saftey of the drug. Especially in cases like this when it is a rare subgruop. So it is win win for the FDA to gather more saftey data and for the patient. It is my understanding the FDA is trying to
get drug companies to use this avenue more. David Banks from the FDA told me some time ago that he believed that Dr Katz would consider an expidited review of our case for a protocal exception.
Sincerely,
Cheri

Exhibit D

From: Taylor, Bettilou (Appropriations)
[mailto:Bettilou_Taylor@appro.senate.gov]
Sent: Monday, January 14, 2008 2:59 PM
To: Goetz, Diane
Subject: Jacob

Diane -- I read with much regret -- you e-mail to Cheri. I know that these decisions are difficult ones -- can you tell me why you won't
grant a protocol exception for Jacob? I also would like to know why Cheri thought that Jacob was going to get the drug -- she had called all of her friends and was so excited that PTC finally decided to let him have the drug and then the let down that it was not going to happen. I understand from my discussions with the FDA that they are encouraging companies to grant exceptions to provide as much info as possible about the side effects from the drug to a variety of patients. I know that this is difficult for all involved -- but I am trying to understand. As you can imagine when the Senators get involved -- they want to know all of the ins and outs of the issue.
Thanks again for taking time to be so kind to us during our visit. Hope you have a wonderful New Year.
BLT

Exhibit E

From: "Goetz, Diane" <dgoetz@ptcbio.com>
To: "Taylor, Bettilou (Appropriations)"
<Bettilou_Taylor@appro.senate.gov>
Cc: "Cheri Gunvalson" <cgunval@gvtel.com>
Sent: Friday, January 25, 2008 6:20 PM
Subject: RE: Jacob

Dear Bettilou,
It's nice to hear from you again. Happy New Year to you, too. I'm sorry for the long delay in responding to you. As you might imagine, it is very difficult to do right by all the patients who could potentially benefit from PTC124, and there are many opinions about the best way to accomplish that. One thing we all agree on: the best thing for all the boys is to get PTC124 approved and to
market as quickly as possible. That is, and must be, our primary goal. PTC has not changed its position on trying to find the best way to involve Jacob and other boys in similar circumstances in our studies. It continues to be a priority for us. I hope you will support us, as I know Cheri does, in our mission to make PTC124 available, as expeditiously as possible, to all boys who could
potentially benefit from it.
Best,
Diane

Exhibit F

From: "Goetz, Diane" <dgoetz@ptcbio.com>
To: "Cheri Gunvalson" <cgunval@gvtel.com>
Sent: Monday, March 17, 2008 8:42 AM
Subject: RE: PTC 124

Dear Cheri,
Plans for the 2a extension are progressing and the trial should be starting over the next several months. I don't have any news about yet about what opportunities there might be for boys and young men like Jacob to have access to PTC124.
Best,
Diane