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UNITED STATES DISTRICT COURT
FOR THE DISTRICT OF NEW JERSEY

JACOB GUNVALSON, CHERI AND JOHN GUNVALSON, AS GUARDIANS FOR JACOB GUNVALSON, AND CHERI AND JOHN GUNVALSON, INDIVIDUALLY, Plaintiffs, v. PTC THERAPEUTICS, INC., Defendant.	Civil Action No. **VERIFIED COMPLAINT** ***Document Filed Electronically***

Plaintiffs Jacob Gunvalson, Cheri and John Gunvalson, as guardians for Jacob Gunvalson, and Cheri and John Gunvalson, individually, by way of complaint against the defendant, PTC Therapeutics, Inc. ("PTC Therapeutics" or "PTC"), allege as follows:

INTRODUCTION

1. Plaintiff Jacob Gunvalson is a sixteen year-old boy with Duchenne Muscular Dystrophy ("DMD"), a rare condition marked by the steady deterioration of muscle tissue that has currently confined Jacob to a wheelchair and will, with the eventual loss of pulmonary function, kill him. Plaintiff Cheri Gunvalson, Jacob's mother, has spent the last six years of her life in getting federal grants and aid to support muscular dystrophy research in general and defendant PTC Therapeutics in particular. Plaintiff John Gunvalson is Jacob's father.

2. Defendant PTC Therapeutics, Inc. manufactures a drug, PTC124, which the company touts as the most promising treatment for Jacob's form of DMD, as well as other diseases. PTC124 has undergone three clinical trials, all of which have been very successful in terms of safety and efficacy.

3. On several occasions, PTC's executives and other representatives promised Cheri Gunvalson that Jacob would have access to PTC124 through enrollment in one of the company's clinical trials. The Gunvalsons reasonably relied on these promises by, for instance, making important medical decisions based upon them. However, Defendant has recently indicated to the Gunvalson family that it will not provide PTC124 to Jacob, either as a part of current clinical trials or pursuant to federal regulations that would permit access under what is known as a compassionate use exception. PTC has said that it does not know if Jacob will ever get access to the drug.

4. Over the past year, Jacob's condition has significantly deteriorated. A year ago, Jacob was able to walk unassisted. Today, he is confined to a wheelchair and cannot lift a glass of water or go to the bathroom without help. Jacob's time is running out. PTC124 is the most promising treatment for Jacob's form of DMD currently being developed. For this reason,

PTC124 represents Jacob's last, best and only chance to slow, stop or even reverse the effects of his condition. Without it, Jacob will not survive.

5. As such, Jacob and his parents respectfully request that the Court enter an Order requiring Defendant PTC Therapeutics to permit Jacob to participate as a "protocol exception" in the Phase IIa study extension currently being commenced in Cincinnati. In the alternative, they ask that the Court order PTC Therapeutics to make the drug available to Jacob's pediatrician, Dr. John Parkin, to be used in a single patient study pursuant to 21 C.F.R. § 312.34(a).

THE PARTIES

6. Plaintiff Jacob Gunvalson is a 16 year-old boy with Duchenne Muscular Dystrophy. Plaintiffs Cheri and John Gunvalson are the parents and guardians of Jacob Gunvalson. All plaintiffs reside in Gonvick, Minnesota.

7. On information and belief, defendant PTC Therapeutics, Inc. is a pharmaceutical manufacturer with its headquarters and principal place of business located at 100 Corporate Court, South Plainfield, New Jersey, 07080, and is incorporated in the state of Delaware.

JURISDICTION AND VENUE

8. This Court has subject matter jurisdiction pursuant to 28 U.S.C. § 1332, as the parties are diverse and the amount in controversy exceeds $75,000.

9. Personal jurisdiction over defendant PTC Therapeutics is present because Defendant is located in this district and a substantial portion of PTC Therapeutics' actions giving rise to the claims herein have occurred within this district.

10. Venue is properly founded in this judicial district under 28 U.S.C. § 1391(a).

BACKGROUND FACTS

11. Plaintiff Jacob Gunvalson is 16 years old, resides in Gonvick, Minnesota, and was born on October 5, 1991. He lives at home with his parents, Cheri and John, his brother Ben (20), and his sister Kelsey (13). Plaintiff Cheri Gunvalson is a registered nurse with a Master of Science Degree in Adult Health Nursing, and Plaintiff John Gunvalson is a farmer.

12. At an early age, Jacob began displaying symptoms of Duchenne Muscular Dystrophy. Jacob was officially diagnosed with DMD in 1999, and since then has been treated with, among other medications, corticosteroids and Gentamicin. Over the last year Jacob's condition has deteriorated significantly and he no longer can walk.

A. Treatment with PTC124 is Jacob's Best Option

13. Duchenne 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 DMD. The life expectancy for an individual with DMD is under 25 years, with death usually caused by deterioration of the pulmonary system. In approximately fifteen percent (15%) of children with DMD, the condition is caused by the presence of a "nonsense mutation" in the dystrophin gene. Therefore, just one in more than 46,000 children has this condition. Jacob's disease is caused by the "nonsense mutation." 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.

14. There is no effective treatment for DMD currently in the marketplace. The most promising treatment being developed is the oral administration of PTC124, a drug that, for

children who have the "nonsense mutation," masks the stop codon and allows the ribosome to direct that the dystrophin be produced to a mature cell.

15. Defendant PTC Therapeutics is a company that was started in 1998. It currently has about 100 employees and has spent over $100 million since it started. Much of the company's revenue stream has been from the federal government, amounting to over $25 million in grants. According to a 2006 registration statement filed with the SEC, PTC124 is the most promising drug of the company. The registration statement makes it clear that funding from the government has been very important to the viability of the company.

16. Although PTC124 is not yet on the market, its value to an individual afflicted with DMD by the nonsense mutation is in the hundreds of thousands, if not millions, of dollars – essentially, this value is the difference between life and death. PTC Therapeutics is currently the only company conducting clinical trials on any drug targeted at the nonsense mutation-caused DMD, and to date, results of clinical studies show PTC124 appears to be far superior to any other treatment available to fight Jacob's illness.

B. Cheri Gunvalson's Work for PTC Therapeutics and its Promises to Treat Jacob

17. Early on, PTC Therapeutics undertook a novel joint venture with a parent group known as the Parent Project for Muscular Dystrophy. ("PPMD"). In 2000, Cheri joined the Government Relations Board of PPMD. In a newspaper article, Jacob's mother has been described as a "heat seeking missile" in the fight against DMD. In 2001, Cheri communicated with every member of Congress and persuaded 235 Representatives and 49 Senators to co-author the Muscular Dystrophy CARE Act, the only disease-specific legislation enacted by the 107th Congress.

18. Prior to passage of the law, 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. The law, sponsored by Cheri's legislators, Minnesota Representative Collin Peterson and the late Senator Paul Wellstone, directed that the National Institutes of Health ("NIH") increase Muscular Dystrophy research, the result of which was that the amount appropriated for research doubled from $14.3 million in 2001 to $28 million in 2003, and doubled again to $54 million in 2006. It also required the NIH to establish and fund Centers of Excellence for treatment of the illness. After the Law was signed on December 18, 2001, Cheri served on the 2003 NIH review board to designate the Centers of Excellence. Over the last five years, Cheri has continued to advocate to Congress, the NIH and the FDA in her effort to find a cure for the disease.

19. Cheri Gunvalson has worked for several years on the preparation of annual Committee Reports issued by the Senate Labor Health and Human Services Appropriations Committee. The Committee Reports give direction to the NIH concerning its funding decisions.

20. In 2005, PTC began its first clinical trial – the Phase I trial – of PTC124, primarily to determine patient tolerance to the drug. The Phase I trial was administered to healthy adults only. As such, Jacob was not enrolled.

21. At this point Cheri was meeting with executives of PTC Therapeutics to urge them to file applications for grants with the National Institutes of Health. Dr. Langdon Miller, PTC's Chief Medical Officer, told her that the applications were complicated and bureaucratic. Having served on a Peer Review Committee with NIH in 2003, Cheri was adamant that the executives were mistaken, that ten percent (10%) of the grant money was supposed to go to private companies and that very few companies were applying for it. Cheri successfully helped

convince PTC to apply for federal grant money, and since that time PTC has received over $25 million in federal grants.

22. In late 2005, PTC commenced a Phase II trial on boys afflicted with Jacob's form of DMD. Under this trial, patients would receive a 28-day dosage of PTC124. Although Jacob was eligible to participate in the Phase II trial, prior to its commencement, Cheri was told by both Dr. Richard Finkel, head of pediatric neurology at the Children's Hospital of Pennsylvania (who was the "primary investigator" for the trial) and Claudia Hirawat, PTC's Senior Vice President, not to take Jacob off of Gentamicin – his then-current treatment – due to the short duration that a trial participant would receive PTC124. At the time, Hirawat also told Cheri there would be **no adverse effects** on Jacob for not participating in the trial. In reliance on these statements, the Gunvalsons did not attempt to enter Jacob in the Phase II trial nor fulfill the prerequisites for it.

23. On March 30, 2006, PTC announced that PTC124 had been granted "fast-track" status by the FDA.

24. Jacob's condition continued to deteriorate, and concerned with this, Cheri went to Dr. John Parkin, Jacob's pediatrician. In late March or early April 2006, Dr. Parkin wrote to Dr. Langdon Miller, PTC's Chief Medical Officer, requesting that the company make the drug available to him for a single patient investigative study. On April 14, 2006, Dr. Miller responded by stating that Phase II clinical trials were being undertaken with PTC124, but that the results of the trials would not be known until the end of 2006. Dr. Miller said that once patient safety was confirmed with this clinical trial, they could then discuss such a proposal.

25. On or about April 24, 2006, Cheri received an email from Dr. Russell Katz, the head of the FDA's neuron-pharmacological division, suggesting that Jacob apply for a protocol

exception or single patient IND in order to receive PTC124 without disturbing any of PTC's clinical trials.

26. On July 13, 2006, the Gunvalsons attended the annual PPMD conference. There, Cheri and her husband John had a private conference with Dr. Miller. Dr. Miller, as with all PTC representatives, expressed great appreciation for Cheri's work in Washington in getting funding which, as noted in *Nature Biotechnology* magazine, was critical to success of the company. During the conversation, Cheri asked Dr. Miller if Jacob would get PTC124. Dr. Miller said that the boys in the Phase II trial metabolized the drug faster than expected and PTC Therapeutics would need to do another 28-day trial at a higher dose. He told Cheri that PTC did not know the right dosage level yet and that it needed to get the safety data back. Dr. Miller reassured Cheri, however, that once positive results were back from the trial Jacob would get PTC124.

27. At this time, Cheri again asked PTC Therapeutics if Jacob should be in the second, Phase IIa 28-day trial at the higher dosage. Claudia Hirawat again instructed Cheri not to discontinue Jacob's Gentamicin treatment, and that Jacob had no better or worse chance to be treated in the future based upon his non-enrollment in the Phase IIa trial. The Gunvalsons did not place Jacob in the Phase IIa trial in reliance on PTC's medical advice, instructions and promises.

28. On September 27, 2006, Cheri was recognized for her work in getting Congress and the NIH to fund DMD research at the Annual Gala of the National Genetic Alliance in Washington, D.C. Claudia Hirawat attended the Gala with Cheri. According to PTC Therapeutics' website, Ms. Hirawat is in charge of corporate development and is directly responsible for fundraising and commercial development of the company. At the event, Ms.

Hirawat expressed great appreciation to Cheri for her work in getting federal funding for PTC's research on DMD. At the event, Cheri explained that she was frustrated with the delay in Jacob receiving access to PTC124, and Ms. Hirawat assured her that Jacob would get access to the medication. The next day Cheri took a train to Philadelphia to attend a conference where PTC released successful results for the clinical trial on those with DMD caused by the nonsense mutation. The drug was reported to not have substantial side effects and appeared to be producing dystrophin, meaning it was effective.

29. The evening that the PTC study was released, a dinner was held at the Loew's Hotel. Dr. Stuart Peltz, the President and Chief Executive Officer of PTC, sat next to Cheri at the dinner. Dr. Peltz thanked Cheri for her leadership in getting federal funding for DMD treatment. She asked if Jacob could get the drug, and Dr. Peltz promised her that Jacob would get it.

30. In October 2006, a telephone conference call was set up between Cheri, Hirawat, and Dr. Parkin. During the conversation, Ms. Hirawat told both Cheri and Dr. Parkin that Jacob would get access to PTC124. It was also noted that Jacob was currently taking Gentamicin and Hirawat explained that, to be enrolled in a PTC124 trial, Jacob would have to off Gentamicin for at least 90 days. In or about March 2007, Jacob lost the ability to walk as a result of DMD and to date has been confined to a wheelchair. Subsequently, Jacob was examined by Dr. Brenda Wong, who headed the Phase IIa study at Cincinnati Children's Hospital. In the summer of 2007, Jacob discontinued his dosage of Gentamicin in reliance that he would soon be enrolled into a PTC124 trial. Jacob had been taking Gentamicin with positive results, but PTC124 was a more promising treatment for his condition because it is more effective than Gentamicin and the side effects of PTC124 are much less severe than those of Gentamicin.

31. On July 11, 2007, PTC and PPMD announced that PTC and the University of Pennsylvania jointly received a $15.4 million research grant from the National Institutes of Health to develop treatments for DMD and Becker muscular dystrophy. The next day Cheri attended the PPMD conference in Philadelphia. At the conference, on July 14, 2007, Cheri again had a conversation with Dr. Peltz, PTC's CEO. Cheri asked Dr. Peltz if Jacob could get PTC124. Dr. Peltz responded that Jacob should have been enrolled in one of the earlier clinical trials, but reiterated that Jacob would get access to the drug. Distressed by Dr. Peltz's comment about how Jacob should have participated in the earlier clinical trials, Cheri then went to Senior Vice President Hirawat, who invited Cheri and Jacob to stay at her home and tour the company headquarters. While there, Ms. Hirawat again represented to Cheri and Jacob that the company was working to put Jacob in a different clinical trial.

32. On October 18, 2007, PTC announced the results of its Phase IIa clinical trial involving patients who were administered PTC124. The drug was reported to be successful, with improved dystrophin presence, increased physical activity and no serious side effects.

33. Bolstered by this news, especially in light of Dr. Miller's earlier promises regarding the safety and efficacy, Cheri contacted PTC's patient liaison, Diane Goetz, about Jacob receiving PTC124 through a compassionate use exception. On November 26, 2007, Cheri had a conversation with Goetz, who told her that Jacob cannot use the drug under an expanded use protocol but that he will be able to get the drug in a different way.

34. By late 2007, PTC announced that it was enrolling DMD patients with the nonsense mutation in a Phase IIb clinical trial, the trial being double-blinded, placebo-controlled, and multi-centered. On December 30, 2007, Diane Goetz sent an email to the Gunvalsons advising them that Jacob did not qualify for the Phase IIb trial because the patients have to be

ambulatory in order to measure their progress, and that she was not aware of the term "protocol exception." She did say, however, that the company was looking at a new clinical trial in which Jacob could enroll.

35. On January 1, 2008, Cheri sent Diane Goetz an email indicating that Dr. Russell Katz, the director of the FDA's neuron-pharmacological division, suggested that Jacob receive PTC124 through a protocol exception.

36. On January 4, 2008, without explanation as to why Jacob could not receive a protocol exception, Diane Goetz, on behalf of PTC, denied Cheri's request.

37. Not satisfied with this response, Cheri contacted Bettilou Taylor, staff director of the Senate Labor, Health and Human Services Appropriations Committee. On January 14, 2008, Taylor sent Goetz an email specifically asking why PTC "won't grant a protocol exception for Jacob", especially because the FDA was "encouraging companies to grant exceptions to provide as much [information] as possible about the side effects from the drug".

38. Eleven days later, on January 25, 2008, Diane Goetz responded to Taylor, again without explanation, that PTC would not provide PTC124 to Jacob in connection with a protocol exception.

C. Current PTC124 Studies in Which Jacob Could Be Treated

39. Enrollment in the Phase IIb trial began in January of 2008 and on April 23, 2008, the Company announced that the trial had begun. Jacob could not be enrolled in the Phase IIb trial because he was not ambulatory as required under the Phase IIb protocol.

40. On information and belief, PTC Therapeutics is also going to imminently conduct a Phase IIa clinical trial extension at the Cincinnati Children's Hospital in which children would

be provided PTC124 on a long-term (96-week) basis. However, the only children eligible for inclusion in this Phase IIa extension are those that participated in the previous Phase IIa study.

41. Because the Gunvalsons were instructed not to enroll Jacob in the Phase IIa study and in reliance on those statements, Jacob is not eligible to participate under the Phase IIa extension protocol. Ironically, other non-ambulatory children with Jacob's form of DMD will be enrolled in this study.

42. Prior to receiving PTC124, each child must undergo a thorough examination by one of the physicians running the study. Included in this examination is an invasive muscle biopsy to determine the child's dystrophin levels.

43. There is a six-month waiting list for an appointment with Dr. Brenda Wong, the lead researcher for the Phase IIa study at Cincinnati Children's Hospital. Jacob has an appointment with Dr. Wong on August 6, 2008. At that time, Dr. Wong will perform a thorough examination of Jacob. She could also perform a muscle biopsy. The Gunvalsons are more than willing to have Dr. Wong perform this invasive procedure if PTC Therapeutics will provide PTC124 for Jacob's treatment. On August 6, therefore, Jacob will be able to have completed the medical prerequisites required to begin treatment on PTC124.

44. On information and belief, there are less than a dozen children – Jacob is perhaps the only one – that are not eligible for inclusion into either the Phase IIa extension or Phase IIb study. Only one in more than 46,000 children has DMD due to the nonsense mutation (the only form of DMD that PTC124 treats). Children with DMD usually die by age 25, with some dying as young as 13. Most DMD children in their early teens are still ambulatory and are therefore eligible for the Phase IIb study. On information and belief, due to the debilitating effects of DMD, many patients over 18 are on a ventilator and therefore would not participate in PTC's

clinical studies. Further, to take part in the studies, a child must not only receive a DMD diagnosis but have his genes sequenced and the nonsense mutation confirmed. Consequently, on information and belief, most, if not all, of those children who meet these criteria are already eligible to participate in either the Phase IIa extension or the Phase IIb study.

D. Jacob Can Receive PTC124 Even if Not Enrolled in a Clinical Trial

45. The FDA allows patients who are not participants in a given clinical trial for unapproved investigational new drugs ("INDs") to gain access to these drugs through the compassionate use exceptions that are known as protocol exceptions or single-patient INDs.

46. Congress and the FDA both encourage compassionate use exceptions, especially in rare diseases where there is a small sample size so that there are more patients in a controlled setting to evaluate the effects of a drug before it goes to market. Compassionate use exceptions are specifically approved of by federal regulations. 21 C.F.R. § 312.34(a) provides:

> During the clinical investigation of the drug, it may be appropriate to use the drug in the treatment of patients not in the clinical trials, in accordance with a treatment protocol or treatment IND. **The purpose of this section is to facilitate the availability of promising new drugs to desperately ill patients as early in the drug development process as possible, before general marketing begins, and to obtain additional data on the drug's safety and effectiveness.**

(emphasis added). This may occur when:

> 1.) The drug is intended to treat a serious or immediately life-threatening disease;
>
> 2.) There is no comparable or satisfactory alternative drug or other therapy available to treat that stage of the disease in the intended patient population;
>
> 3.) The drug is under investigation in a controlled clinical trial under an IND in effect for the trial, or all clinical trials have been completed; and

> 4.) The sponsor of the controlled clinical trial is actively pursuing marketing approval of the investigational drug with due diligence.

21 C.F.R. § 312.34(b)(1). PTC Therapeutics and PTC124 satisfy all four of these conditions.

47. The use of a single patient IND or a "protocol exception" is encouraged by the FDA where the patient otherwise faces death or a serious debilitating condition. In January 2000, an article entitled "Experimental Treatments? Unapproved but Not Always Unavailable" was published in the FDA Consumer Magazine. The article describes the availability of single-patient INDs as follows:

> If enough is known about the drug's safety, and there is some clinical evidence of effectiveness, FDA may allow a patient to become his or her own study. This so-called single-patient IND, or compassionate use IND, virtually ensures that any patient can get access to any investigational new drug.

48. In January 2002, the Best Pharmaceuticals for Children Act ("BPCA") amended section 113 of the Food and Drug Administration Modernization Act of 1997, see 42 U.S.C. § 282(i)(3)(A), to require that clinical sponsors of an investigational new drug ("IND") for serious or life-threatening diseases to provide a description of their policy on compassionate use exceptions to a national data bank maintained by the federal government.

49. In addition to the United States Code, the FDA issued a procedural guidance which acknowledged the need for expanded use and strongly encouraged sponsors to make their expanded use protocols readily accessible to the public by submitting it to the Clinical Trials Data Bank.

50. The Phase IIa and Phase IIb clinical trials for PTC124 are listed on the website www.clinicaltrials.gov. According to the website, the Phase IIb study is currently recruiting participants, and is not scheduled to be completed until August of 2010. As a clinical sponsor of PTC124 -- an investigational new drug for serious or life-threatening diseases -- PTC

Therapeutics is required to provide the description of its policy for compassionate use exceptions. Despite this, the website appears to indicate that PTC may have ignored the above directives and did not submit its expanded use protocol to the federal government.

E. PTC's Denial of a Compassionate Use Exception for Jacob

51. The Gunvalsons have repeatedly requested that PTC Therapeutics enroll Jacob as a protocol exception in the Phase IIa study extension or permit him to undergo a single-patient IND. Unfortunately, notwithstanding PTC's prior promises to the Gunvalsons, these requests have been expressly rejected.

52. The Gunvalsons respectfully submit that Jacob should be enrolled in the Phase IIa study extension as a protocol exception. Such a compassionate use would not adversely affect PTC because Jacob's inclusion would not affect the data derived from the study.

53. Alternatively, if Jacob cannot be enrolled as a protocol exception to the Phase IIa study extension, the Gunvalsons respectfully submit that Jacob should be placed in a single-patient IND under the care of Dr. John Parkin or another physician who could supervise the study.

54. If PTC makes PTC124 available to Jacob for use as either a protocol exception to the Phase IIa study extension or in a single-patient IND, FDA approval is still needed. In that regard, Dr. Russell Katz, the head of the FDA's neuron-pharmacological division, has already indicated that the FDA would likely approve such a request.

55. On April 12, 2008, PTC Therapeutics told Cheri that it did not know if Jacob would ever get the drug. At best, PTC Therapeutics has stated that it is willing to consider Jacob's participation in some future clinical trial, assuming he qualifies under that trial's protocols. However, PTC has admitted that they have not asked the FDA for approval of any

such trials, and that no such trials are currently being planned or contemplated. Given the amount of time necessary to plan and approve a clinical trial and the anticipated completion date of the Phase IIa study extension (96 weeks) and the Phase IIb study of PTC124 (August of 2010), Jacob will probably die before he is eligible to receive PTC124 on the schedule proposed by PTC Therapeutics.

56. Jacob, John and Cheri Gunvalson are willing to pay for PTC124 and its administration, undertake the necessary applications to secure approval from the FDA, sign a fully informed consent and release, and do any other action necessary for Jacob to receive such treatment.

57. On information and belief, the longer Jacob does not have access to PTC124 and the more he deteriorates, the less likely he is to benefit from the drug, and the less benefit he will derive from it.

COUNT I:
PROMISSORY ESTOPPEL

58. Plaintiffs repeat and reallege the allegations in paragraphs 1 through 57 as if fully set forth herein.

59. PTC Therapeutics made multiple promises to Cheri and others that Jacob would get access to PTC124. By way of example only:

- On July 14, 2006, following the annual PPMD conference, Dr. Langdon Miller, PTC's Chief Medical Officer, expressed appreciation for Cheri's work in getting funding for the company and told Cheri that Jacob would get PTC124 once results were back from the Phase IIa trial;
- On September 27, 2006, at the National Generic Alliance's Annual Gala, Claudia Hirawat, PTC's Senior Vice President, told Cheri that Jacob would get access to PTC124;
- On September 28, 2006, at a PPMD conference, Dr. Stuart Peltz, PTC's President and CEO, promised Cheri that Jacob would get PTC124;

- In October 2006, Hirawat again told Cheri and Dr. John Parkin that Jacob would have access to PTC124 during a telephone conference;
- Following a July 2007 PPMD conference, Hirawat told Cheri and Jacob, while they visited her home, that PTC Therapeutics would put Jacob in a clinical trial; and
- On November 26, 2007, Diane Goetz, a PTC patient liaison, told Cheri that Jacob will be able to get access to PTC124 (although not under an expanded use protocol).

In sum, PTC Therapeutics made multiple, repeated promises to Cheri that Jacob would get access to PTC124.

60. Additionally, prior to the commencement of both the Phase II and Phase IIa 28-day trials, Cheri asked PTC Therapeutics whether she should enroll Jacob in those trials. Claudia Hirawat, as well as Dr. Finkel, told Cheri not to take Jacob off his then-current medication to enroll him in either trial. Specifically, twice Ms. Hirawat advised Cheri that there would be no adverse effects on Jacob for not participating in the Phase II or Phase IIa trials. However, PTC now refuses to permit Jacob entry into the Phase IIa trial extension expressly because he was not in the original trials.

61. The Gunvalsons were told Jacob could not receive PTC124 until he was off Gentamicin for 90 days. In reliance on this statement, they discontinued Jacob's dosage of Gentamicin.

62. The promises made to Cheri by PTC Therapeutics and its officers and employees were both clear and definite.

63. PTC Therapeutics knew or should have known that Cheri, John and Jacob Gunvalson would rely on those promises, and, on information and belief, in fact PTC expected that they would do so.

64. The close personal relationship between Cheri Gunvalson and certain PTC Therapeutics executives further led Cheri, John and Jacob Gunvalson to have trust and confidence in the company and to believe and rely on the company's promises.

65. Cheri, John and Jacob Gunvalson's reliance on PTC Therapeutics' promises was reasonable.

66. Throughout this time period, PTC Therapeutics continued to benefit from Cheri's activity, her efforts to procure federal research grants, her discussions with the media, and her discussions with government agencies and elected officials, all of which brought in research dollars for PTC Therapeutics and enhanced the company's credibility. Her efforts also gave the company credibility with regulators and donors. In short, Cheri has been extraordinarily active in her pursuit of research money for PTC, and has greatly assisted PTC Therapeutics in raising government money for its research. Cheri's extensive work was partly attributable to numerous promises made by PTC Therapeutics executives that Jacob would receive access to PTC124.

67. Cheri, John and Jacob Gunvalson reasonably relied on the promises of PTC Therapeutics, in substantial detriment to Jacob. After consulting with and at the direction of Jacob's guardians John and Cheri Gunvalson, Jacob's physician, Dr. Parkin, discontinued treating Jacob with his dosage of Gentamicin in reliance on the company's promise that Jacob could not be on Gentamicin for 90 days previous to enrolling in a clinical study of PTC124. Similarly, John and Cheri Gunvalson made this decision in reliance on PTC Therapeutics' promises.

68. Further, Jacob has suffered great harm due to PTC's direction that Jacob refrain from entering the Phase II or Phase IIa 28-day trials and affirmation that this decision would cause him no harm in the future; significantly, PTC has now refused to permit Jacob to enter the

Phase IIa extension or Phase IIb trials by claiming he does not qualify under those studies' protocols, and Jacob may not survive to the time by which both the Phase IIa study extension and the Phase IIb study are completed.

69. PTC Therapeutics should be estopped from refusing to provide PTC124 to Jacob Gunvalson.

COUNT II:
FRAUDULENT MISREPRESENTATION

70. Plaintiffs repeat and reallege the allegations in paragraphs 1 through 69 as if fully set forth herein.

71. PTC Therapeutics knowingly, intentionally, and falsely misrepresented to Cheri Gunvalson and others that Jacob would be provided access to PTC124.

72. PTC Therapeutics knowingly, intentionally, and falsely misrepresented to Cheri Gunvalson and others that withholding Jacob from the Phase II or Phase IIa 28-day trials would not prejudice, harm or otherwise affect him in the future.

73. The false and fraudulent misrepresentations of PTC Therapeutics, all of which were material, were undertaken with the knowledge and intent that Cheri, John and Jacob Gunvalson would be deceived into believing and relying upon said misrepresentations.

74. Cheri, John and Jacob Gunvalson relied upon these misrepresentations.

75. Jacob individually, and Cheri and John as Jacob's guardians, have been injured due to PTC Therapeutics' fraudulent misrepresentations, and such injury is irreparable. Each day that passes without access to PTC124 constitutes further injury to Jacob. For this reason, PTC Therapeutics should be ordered to immediately provide Jacob access to PTC124.

COUNT III:
NEGLIGENT MISREPRESENTATION

76. Plaintiffs repeat and reallege the allegations in paragraphs 1 through 75 as if fully set forth herein.

77. PTC Therapeutics, at a minimum, negligently misrepresented to Cheri Gunvalson and others that Jacob would be provided access to PTC124.

78. PTC Therapeutics, at a minimum, negligently misrepresented to Cheri Gunvalson and others that withholding Jacob from the Phase II or Phase IIa 28-day trials would not prejudice, harm or otherwise affect him in the future.

79. Cheri, John and Jacob Gunvalson justifiably relied upon PTC Therapeutics' misrepresentations.

80. Jacob individually, and Cheri and John as Jacob's guardians, have been injured due to PTC Therapeutics' negligent misrepresentations, and such injury is irreparable. Each day that passes without access to PTC124 constitutes further injury to Jacob. For this reason, PTC Therapeutics should be ordered to immediately provide Jacob access to PTC124.

WHEREFORE, Plaintiffs demand judgment in their favor against Defendant as follows:

A. Awarding preliminary and permanent injunctions ordering Defendant PTC Therapeutics to make PTC124 available to Jacob Gunvalson under either a "protocol exception" to the Phase IIa study extension at Cincinnati Children's Hospital or, in the alternative, to allow Jacob's personal physician access to the drug for purposes of a "single patient" IND;

B. Ordering that Defendant PTC Therapeutics specifically perform its promises by providing Jacob Gunvalson access to PTC124 through the above means;

C. Awarding Plaintiffs compensatory damages against Defendant PTC Corporation in an amount to be determined at trial;

D. Awarding Plaintiffs punitive damages against Defendant PTC Corporation in an amount to be determined at trial;

E. Awarding Plaintiffs their costs and attorneys' fees;

F. Awarding Plaintiffs pre- and post-judgment interest on any damages; and

G. Granting such other and further relief as this Court deems proper.

DEMAND FOR JURY TRIAL

Plaintiffs hereby demand a trial by jury pursuant to Federal Rule of Civil Procedure 38 as to all issues so triable.

Respectfully submitted,

SAIBER LLC
Attorneys for Plaintiffs

/s/ Marc. E. Wolin
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Tel: (612) 343-3289

Dated: July 16, 2008

VERIFICATION DECLARATION OF CHERI GUNVALSON

CHERI GUNVALSON, of full age, hereby declares as follows:

1. I am the mother of, and along with my husband John guardian of, Jacob Gunvalson, a 16 year-old boy afflicted with Duchenne Muscular Dystrophy. Jacob, John and I are plaintiffs in the within matter.

2. I have read the above Verified Complaint and the matters stated therein are true and correct to the best of my knowledge, except as to those matters therein stated to be upon information and belief, and as to those matters, I believe them to be true.

3. The basis for any knowledge and belief is information provided to me by others and/or documents I have reviewed.

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

Dated: 7/15/08

Cheri Gunvalson
CHERI GUNVALSON

LOCAL CIVIL RULE 11.2 CERTIFICATION

Under Local Civil Rule 11.2, the undersigned counsel for Plaintiffs hereby certifies that this matter is not the subject of any other action asserted by Plaintiffs herein in any court, or of any pending arbitration or administrative proceeding.

Respectfully submitted,

SAIBER LLC
Attorneys for Plaintiffs

/s/ Marc. E. Wolin
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LOCAL CIVIL RULE 201.1 CERTIFICATION

Under Local Civil Rule 201.1, the undersigned counsel for Plaintiffs hereby certifies that, in addition to monetary damages greater than $150,000, Plaintiffs seek injunctive relief, and therefore this action is not appropriate for compulsory arbitration.

Respectfully submitted,

SAIBER LLC
Attorneys for Plaintiffs

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