Marc E. Wolin (mew@saiber.com)
Jakob B. Halpern (jbh@saiber.com)
SAIBER LLC
One Gateway Center
Newark, N.J. 07102
Telephone: (973) 622-3333
Facsimile: (973) 622-3349

Michael A. Hatch (MHatch@blackwellburke.com)
BLACKWELL BURKE P.A.
431 South Seventh Street, Suite 2500
Minneapolis, MN 55402
Telephone: (612) 343-3289
Facsimile: (612) 343-3205

Attorneys for Plaintiffs

UNITED STATES DISTRICT COURT
FOR THE DISTRICT OF NEW JERSEY

JACOB GUNVALSON, CHERI AND JOHN GUNVALSON, AS GUARDIANS FOR JACOB GUNVALSON, AND CHERI AND JOHN GUNVALSON, INDIVIDUALLY, Plaintiffs, v. PTC THERAPEUTICS, INC., Defendant.	Civil Action No. 08-3559 (WJM) (MF) **DECLARATION OF DR. JOHN PARKIN, M.D.** ***DOCUMENT FILED ELECTRONICALLY***

DR. JOHN PARKIN, M.D., of full age, declares as follows:

1. I am a physician who specializes in pediatric medicine. My practice is located in Bemidji, Minnesota. I am affiliated with MeritCare Clinic, which has its headquarters in Fargo, North Dakota.

2. Jacob Gunvalson has been my patient since he was a small boy. In 1999 Jacob was observed to have symptoms that later were diagnosed to be Duchenne Muscular Dystrophy ("DMD"). There are various causes of Duchenne Muscular Dystrophy, and in 15% of the cases

the cause is related to a "nonsense mutation" in the dystrophin gene which interferes with the development of a muscle-building protein. I understand that Jacob's medical condition is caused by this "nonsense mutation" in the dystrophin gene. A true and correct copy of a portion of the website of PTC Therapeutics, Inc. ("PTC Therapeutics" or "PTC") which discusses this medical condition is attached hereto as Exhibit A.

3. Since 1999 Jacob has been treated with, among other medications, corticosteroids and Gentamicin. He was treated with Gentamicin for approximately four years, from January of 2003 to June of 2007. The administration of Gentamicin was "off label," meaning that its use in the treatment of Duchenne Muscular Dystrophy was investigational.

4. Cheri Gunvalson, Jacob's mother, is a registered nurse. She is extremely active in muscular dystrophy organizations and has been in frequent contact with PTC Therapeutics, Inc. ("PTC Therapeutics" or "PTC"), a pharmaceutical company that is developing a drug called PTC124 for the treatment of Jacob's form of DMD. PTC Therapeutics indicates on its website and in published journals that PTC124 is promising in the treatment of Duchenne Muscular Dystrophy.

5. In March or April 2006, at the request of Ms. Gunvalson, I wrote to PTC Therapeutics and requested that I be provided access to PTC124 so that I could apply to the FDA for a single-patient investigative study with Jacob Gunvalson. A true and correct copy of my letter to the company is attached hereto as Exhibit B.

6. On April 14, 2006, Dr. Langdon Miller, the Chief Medical Officer of PTC, responded to me by letter and essentially stated that the company would have to complete a Phase II clinical trial, which was scheduled to commence toward the end of 2006, prior to

considering my request. A true and correct copy of Dr. Miller's reply letter is attached hereto as Exhibit C.

7. In the fall of 2006 (I believe it may have been in October of 2006), I participated in a telephone conference call with Cheri Gunvalson and a women who identified herself as Ms. Claudia Hirawat, the Senior Vice President of Corporate Development at PTC Therapeutics. During the conversation Ms. Hirawat made two statements that I recall. First, she stated that Jacob would have access to PTC124. Second, that Jacob had to be off Gentamicin for 90 days before being administered PTC124. In reliance on this statement, Jacob was taken off Gentamicin in approximately June of 2007.

8. PTC Therapeutics reports on its website that it completed the Phase II clinical study and that, as of May of 2007, interim data indicated that the study was a success with regard to safety and efficacy. Exhibit A hereto discusses the success of the Phase IIa clinical trial. As it relates to safety, the company states:

> There are no safety concerns based on physical examinations, vital sign measurements, electrocardiograms, or laboratory parameters.

It also reported that 50% of the boys who were administered PTC124 showed an increase in muscular dystrophin levels, and that the parents reported that the boys had increased physical endurance and improvement in physical activity.

9. It is my understanding that the company is currently enrolling patients in a Phase IIa study extension at Cincinnati Children's Hospital. It is my further understanding that participation in the extended study is limited to patients who participated in a previous study. It is my understanding that Jacob is not eligible to participate in the study under its protocol because he was not enrolled in the prior study.

10. It is also my understanding that, with regard to other PTC124 clinical studies, patients who are enrolled must be ambulatory; that is, the patient must be able to walk approximately 80 yards unassisted within a six-minute time period. One of these studies is a Phase IIb international study. A true and correct copy of page from PTC's website discussing this study is attached hereto as Exhibit D. It is my understanding that the purpose of requiring the patient to be ambulatory is to create a mechanism through which progress can by measured. At present Jacob is non-ambulatory, which means he is ineligible to participate in the Phase IIb clinical trial.

11. It is my understanding that there are several vehicles through which a patient can get access to an investigational drug through a "compassionate use exception" if they are not already enrolled in or eligible for including in a clinical trial. One approach is through a "protocol exception" in which the pharmaceutical company will approve the participation of a patient who otherwise is not qualified to participate in the clinical study if, without access to the drug, the patient may die or suffer serious consequences. Another approach is to obtain approval from the FDA for a single patient IND (a single-patient study), which also requires the approval of the pharmaceutical company.

12. If PTC Therapeutics will not permit Jacob to participate as a protocol exception in the extended Phase IIa study at the Cincinnati Children's Hospital, I am prepared to apply to the FDA for approval to conduct a single patient IND. In order to treat Jacob under a single patient IND, however, PTC Therapeutics must agree to allow me access to PTC124. I submit this declaration in support of the Gunvalson's request that PTC Therapeutics be ordered to provide access to PTC124.

13. I have spoken with Cheri Gunvalson about this matter, and I have advised her that, prior to such a single patient IND being commenced, John (Jacob's father), Cheri and Jacob will need to sign the requisite authorizations, waivers and releases to PTC Therapeutics, and to the physician conducting the study. Cheri told me that the Gunvalsons will do so. I assume any order issued by the Court would make reference to these requirements.

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

John Parkin MD

DR. JOHN PARKIN, M.D.

Dated: 7/15/08

EXHIBIT A

Clinical and Preclinical Programs

Genetic Disorders: PTC124

-Indications

-Additional Resources

Oncology: PTC299

Infectious Diseases: Hepatitis C

Drug Discovery Programs

Genetic Disorders: PTC124

PTC124 is a novel, orally administered small-molecule compound that targets a particular genetic alteration known as a nonsense mutation. Genetic disorders occur as a consequence of mutations in an individual's DNA. Nonsense mutations are single-point alterations in the DNA that, when transcribed into mRNA, introduce a premature translation termination codon. This change halts the ribosomal translation process at an earlier site than normal, producing a truncated, non-functional protein. PTC is developing PTC124 for the treatment of genetic disorders in which a nonsense mutation is the cause of the disease.

Click here to read Frequently Asked Questions about PTC124.

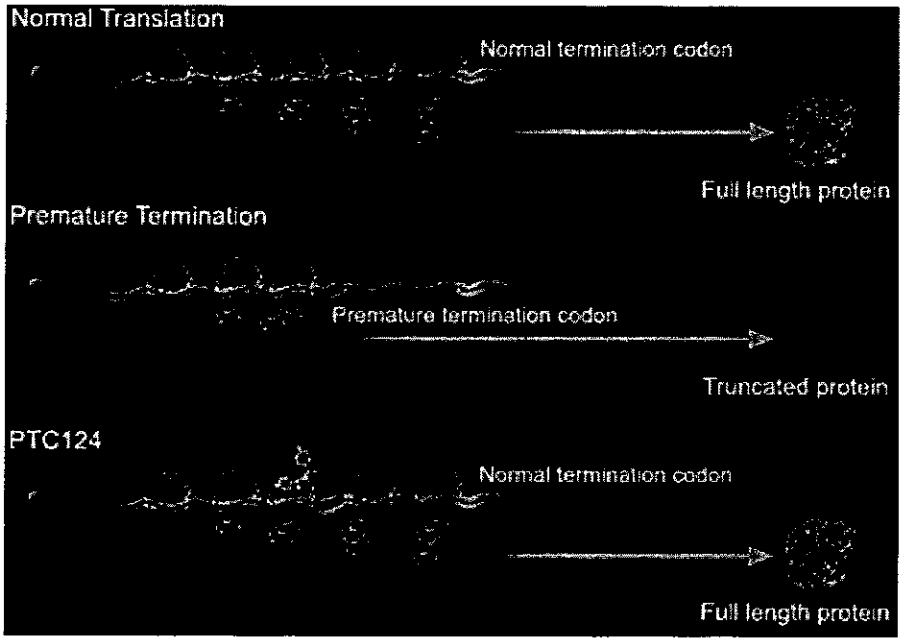
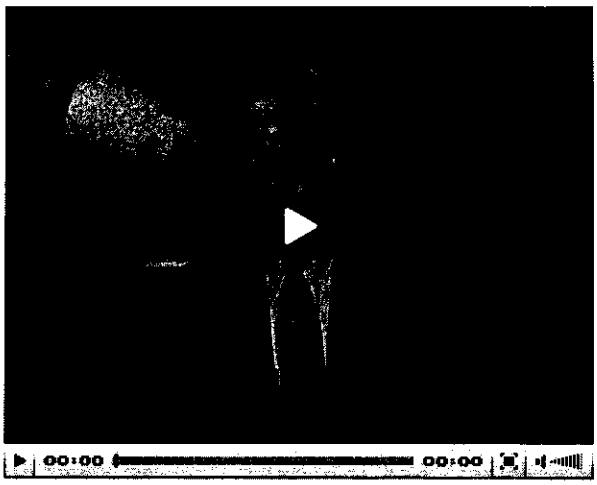


Figure 1. Translation of an mRNA into protein: comparison of normal translation, premature translation termination, and treatment with PTC124 restoring synthesis of full-length protein.

The National Institutes of Health (NIH) Office of Rare Diseases estimates that genetic disorders are responsible for the majority of rare diseases that afflict 25 million people in the US. It is estimated that, on average, 5-15% of patients with any of at least 1,800 distinct genetic disorders have a nonsense mutation as the underlying cause of the disease, including cystic fibrosis, Duchenne muscular dystrophy, spinal muscular atrophy, hemophilia, lysosomal storage disorders, retinitis pigmentosa, familial hypercholesterolemia and some forms of cancer.

PTC124 allows the cellular machinery to read through premature stop codons in mRNA, and thereby enables the translation process to produce full-length, functional proteins.



PTC124: PRECLINICAL STUDIES

PTC has conducted multiple *in vitro* and *in vivo* preclinical studies of PTC124. Key findings include:

- Exposure to PTC124 resulted in the production of full-length and functionally active dystrophin in both the *in vitro* and mouse models of nonsense mutation mediated Duchenne muscular dystrophy; these data were published in the May 3, 2007 edition of *Nature (click on text below to download a PDF of the Nature article).*

  PTC124 Targets Genetic Disorders Caused by Nonsense Mutations

- Similarly, the administration of PTC124 resulted in the production of full-length and functionally active CFTR in mouse models of nonsense mutation mediated cystic fibrosis.

- PTC124 demonstrated greater potency and activity then gentamicin in the read through of premature stop codons in *in vitro* studies.

PTC124: CLINICAL STUDIES

Phase 1 Clinical Trials — Results from Phase 1 clinical studies in healthy volunteers show that PTC124 is generally well tolerated, achieves target plasma concentrations that have been associated with activity in preclinical models, and does not induce ribosomal readthrough of normal stop codons. Data from these studies were published in the April 2007 edition of the *Journal of Clinical Pharmacology.*

Phase 2 Clinical Trials for Duchenne muscular dystrophy (DMD) - In May 2007, interim data from a Phase 2 clinical trial in DMD were presented. This Phase 2 multi-site, open-label, dose-ranging clinical trial is evaluating muscle dystrophin expression and also blood levels of muscle-derived creatine kinase as a measure of muscle integrity. PTC124 safety, compliance, and PK are also being evaluated. The preliminary results show that treatment with PTC124 was associated with increases in muscle dystrophin expression in the Day 28 biopsy specimen in approximately 50 percent of the subjects and statistically significant reductions in serum creatine kinase values were also noted. Exposure to PTC124 of patient muscle cells cultured in vitro from pre-treatment biopsy showed evidence of a dose-dependent increase in dystrophin expression in all of the evaluable patients. Several parents and teachers reported that boys participating in the study had improvements in activity level and increased endurance during treatment. Individual patients at the first two dose levels tested demonstrated some improvement in upper and lower body muscle strength, however, in the overall analysis the magnitude of change was not statistically significant. Potentially drug-related adverse events have been infrequent, mild to moderate in severity, have not resulted in therapy interruptions or discontinuations, and have been reversible. There are no safety concerns based on physical examinations, vital sign measurements, electrocardiograms, or laboratory parameters. Compliance has been excellent. The PK data demonstrated that plasma levels of PTC124 were lower at both doses tested as compared to those observed in the healthy volunteer studies and in the adult CF studies. Therefore, a higher dose level has been administered to an additional 12 boys and the data are currently being evaluated.

Phase 2 Clinical Trials for Cystic Fibrosis (CF) - In November 2006, PTC investigators presented data from two Phase 2 CF clinical trials. Studies were open-label, dose-ranging clinical trials evaluating change in chloride conductance using

a standardized nasal transepithelial potential difference (TEPD) procedure in patients with nonsense-mutation-mediated CF. PTC124 safety, compliance, and pharmacokinetics (PK) were also evaluated. Across the two studies, at the two PTC124 dose levels tested, TEPD assessments showed statistically significant improvements of average CFTR-dependent chloride secretion in the airways. By the end of the first cycle of treatment, 18 out of 42 patients from the two studies had responded with a change of at least -5 mV in chloride secretion TEPD and 15 out of 42 patients had chloride secretion TEPD values in the generally accepted normal range (more electrically negative than -5 mV). Blood neutrophil counts were also monitored before and during PTC124 treatment because CF is a neutrophil-mediated disease, and statistically significant reductions in blood neutrophil counts were observed in both studies. Furthermore, improvements in circulating levels of liver enzymes in the blood were seen in both trials, supporting the hypotheses that PTC124 could offer systemic benefits to patients with multiorgan compromise due to CF. Trends toward improved pulmonary function and body weight were also observed in patients participating in the Phase 2 program. Although a formal symptom assessment was not a component of the Phase 2 program, a number of patients described decreased sputum volume and thickness, decreased frequency and severity of coughing and a better sense of well-being during PTC124 therapy. PTC124 was well tolerated, resulting in excellent compliance with the treatment regimen.

Collective results from the Phase 2 studies will be reviewed with regulatory authorities with the intent of initiating a program of longer-term clinical studies by the end of 2007.

To receive status updates on PTC124, please visit the Contact Us page of the website and join our mailing list.

Patients, families and advocacy groups may also contact Ms. Diane Goetz, Director, Patient and Professional Relations, 908-222-7000 x9256 or patientinfo@ptcbio.com.

Click here to read Frequently Asked Questions about PTC124.

EXHIBIT B

John R. Parkin M.D.
1705 Anne St NW
Bemidji, MN 56601
[218] 333-4710
[218] 333-4728 FAX

Langdon Miller M.D.
Chief Medical Officer
PTC Therapeutics
100 Corporate Court
South Plainfield, NJ 07080

Dear Dr. Miller,

I am writing as the initial step of a compassionate use application of PTC 124 as a single patient IND for a patient of mine. As the first step, we would need agreement from PTC to provide the medication

Jacob Gunvalson [DOB 10/05/91] has been a patient of mine since birth. In early childhood he was diagnosed with Muscular Dystrophy [MD] secondary to a nonsense stop codon mutation of his dystrophin gene. Fortunately he has not had any major medical complications from his MD but his motor functions are deteriorating in spite of aggressive therapy. He does show stunted growth and Cushingoid features from the long term steroids.

His major medications at this time include Deflazacort, Fosamax, Enalopril and Prevacid. He also has been treated with an experimental protocol of IV gentamycin 3 times weekly. This has slowed the progression of his disease but appears to be losing effectiveness.

His general health remains good thus far. Specifically his most recent echocardiogram [11/03/05] was normal with an ejection fraction of 79%. Pulmonary functions were last tested [12/30/04] and were also normal. He has not had pneumonias. Dexascan [5/19/04] did show decreased but improving bone density and he has not had any fractures. His most recent creatinine clearance was normal at 86 ml/minute.

I am aware of the cystic fibrosis trials and the current MD trial in progress. If the MD trial shows any promise, I would like to see Jacob start PCT 124 as soon as possible before any further deterioration occurs.

Jacob is a pleasant, bright adolescent who is motivated to succeed. He has been cooperative in his therapy and treatments. He can still walk short distances with a pronounced abnormal gait and uses an electric scooter for longer walks.

His parents are very knowledgeable about MD and understand the potential risks of a phase 2 drug such as PTC 124. They have also been very active politically in getting research funds for MD.

I feel that Jacob would be an ideal candidate for a single patient IND under compassionate use. It is important that this be started as soon as possible before further deterioration occurs.

I would be glad to answer any questions or discuss this further by any means you wish. I appreciate your careful consideration of my request. Thank you.

Sincerely

John R. Parkin M.D.
johnparkin@meritcare.com

EXHIBIT C

April 14, 2006

John R. Parkin, MD
MeritCare Bemidji
1705 Anne Street NW
Bemidji, Minnesota 56601

Dear Dr. Parkin:

Thank you for your inquiry regarding a single-patient protocol for Jacob Gunvalson. I would like to provide you with some details about the current clinical development of PTC124.

At this point we have Phase 2 studies ongoing in cystic fibrosis (CF) and in Duchenne muscular dystrophy (DMD). In the CF Phase 2 program, we have performed an interim analysis of data from the first 15 patients who have received two 14-day courses of PTC124.

The findings show evidence that PTC124 can increase chloride channel activity in some patients. In the DMD Phase 2 study, we are not yet in the position to perform an analysis and therefore have no indication of PTC124's activity in DMD patients. The safety data for CF and DMD are also limited and are awaiting data monitoring committee review. Therapy in the Phase 2 clinical trial program is currently limited to a total of 28 days, based on the duration of preclinical toxicology data available to date. A long-term preclinical toxicology program is in progress as a prelude to conducting longer-term clinical trials in CF and DMD.

Given this situation, implementation of an expanded access program at this time would be premature. While we are encouraged that the available data may suggest proof of concept in CF, these data were obtained in only a small number of adult patients receiving PTC124 for a short period of time. We cannot be sure that the preliminary results in CF will indicate long-term clinical benefit in CF, nor can we surmise that the preliminary CF findings will predict clinical benefit in patients with DMD.

We want to avoid unacceptable risks for patients and be certain that we do not jeopardize the development of PTC124. Our clinical development plan is designed to ensure that PTC124 becomes available for all patients who might benefit if its efficacy and safety are eventually proven. Thus, we must finish the additional toxicology studies and complete accrual to the current Phase 2 clinical trials in CF and DMD. We expect to complete our further toxicology studies and Phase 2 clinical trials in CF and DMD by the end of 2006, with data available following completion.

I would like to suggest that we plan to be in touch in early 2007 once more information is available. In the meantime, should you have any questions, please do not hesitate to contact us. In my role I am often traveling and I did not have an opportunity to see your letter until late last week. The best point of contact here at PTC Therapeutics is Ms. Kerri Donnelly (kdonnelly@ptcbio.com; 908-222-7000, x112). Ms. Donnelly will ensure you receive a prompt reply and she is always able to reach me and anyone else within PTC Therapeutics who could be of assistance.

Best regards,

Langdon L. Miller, MD

Langdon L. Miller, M.D.
Chief Medical Officer
PTC Therapeutics

cc: Ms. Kerri Donnelly
Ms. Cheri Gunvalson

EXHIBIT D

PTC124 Phase 2b Study in DMD/BMD: Trial Sites

The PTC124 Phase 2b trial in Duchenne muscular dystrophy and Becker muscular dystrophy will involve more than 35 sites on 4 continents. Use the map below to locate the study site closest to you. As you click on each region, you will be able to see names and locations of specific sites. As each study site opens (indicated by a green dot), information will be added so that you can contact the site of your choice directly.

Australia

- **Royal Children's Hospital**
  Parkville, Victoria

- **Institute For Neuromuscular Research, The Children's Hospital at Westmead**
  Westmead, NSW

Belgium

- **University Hospital KU Leuven**
  Leuvan, Belgium
  Contact: Greet Leemputten
  +32 16 34 38 45
  greet.vanleemputten@uz.kuleuven.ac.be

  Prinicpal Investigator: Nathalie Goemans, M.D.

Canada

- **Alberta Children's Hospital**
  Calgary, Alberta
- **British Columbia Children's Hospital**
  Vancouver, British Columbia
- **London Health Sciences Center**
  London, Ontario

France

- **Service de Neuropediatrie, hôpital La Timone**
  Marseille, France
- **Groupe Hospitalier La Pitie-Salpetriere**
  Paris, France
- **Neuromuscular center of Nantes**
  Nantes, France

Germany

- **University Clinic for Children, University of Essen**
  Essen, Germany
- **University Hospital**
  Freiburg, Germany

Israel

- **Hadassah Medical Center, Hebrew University Hospital**
  Jerusalem, Israel

Italy

- **Ospedale Maggiore Policlinico in Milan**
  Milan, Italy
- **U.O. Complessa di Neuropsichiatria Infantile**
  Rome, Italy
- **Ospedale Pediatrico Bambino Gesu**
  Rome, Italy

Spain

- **Hospital Sant Joan de déu**
  Barcelona, Spain
- **Hospital Universitari La Fe**
  Valencia, Spain

Sweden

- **Queen Silvia Children's Hospital**
  Goteburg, Sweden
- **Astrid Lindgren Pediatric Hospital**
  Stockholm, Sweden

United Kingdom

- **Imperial College London, Hammersmith Hospital**
  London, UK
- **Robert Jones and Agnes Hunt Orthopaedic NHS Trust**
  Shropshire, UK
- **Univ of Newcastle, Institute of Human Genetics**
  New Castle, UK

USA

- **University of California-Davis**
  Sacramento, CA
- **Child Neurology Center of Pensacola**
  Pensacola, FL

  Contact: Holley Moseley
  850-484-6060
  holley.moseley@childneurologycenter.com

  Principal Investigator: Ben Renfroe, M.D.
- **University of Kansas Medical Centre**
  Kansas City, KS
- **University of Minnesota**
  Minneapolis, MN
- **Duke University Medical Center**
  Durham, NC
- **University of Rochester**
  Rochester, NY

  Contact: Alexis Smirnow
  585-275-4715
  alexis_smirnow@urmc.rochester.edu

  Principal Investigator: Emma Ciafaloni, M.D.
- **The Children's Hospital**
  Aurora, CO
- **University of Iowa Healthcare**
  Iowa City, IA

  Contact: Carrie Stephan
  319-356-2673
  carrie-stephan@uiowa.edu
  Prinicpal Investigator: Kathy Mathews, M.D.
- **Children's Hospital of Boston/Harvard Medical School**
  Boston, MA
- **Washington University School of Medicine**
  Saint Louis, MO

  Contact: Betsy Malkus
  314-362-1624
  malkusb@neuro.wustl.edu

  Principal Investigator: Anne Connolly
- **Columbia University Medical School**
  New York City, NY
- **Cincinnati Children's Hospital**
  Cincinnati, OH

  Contact: Paula Morehart
  513-636-8967
  Paula.Morehart@cchmc.org

  Principal Investigator: Brenda Wong, M.D.

- **Shriners Hospital for Children**
  Portland, OR

- **Children's Hospital of Philadelphia**
  Philadelphia, PA

  Contact: Lindsay Dorsey
  267-426-7623
  dorsey@email.chop.edu

  Principal Investigator: Richard Finkel, M.D.

- **Southwestern University**
  Dallas, TX

- **University of Utah**
  Salt Lake City, UT

  Contact: Kristi Newingham
  801-585-1299
  knewing@genetics.utah.edu

  Principal Investigator: Kevin Flanigan, M.D.