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August 18, 2008

VIA ELECTRONIC FILING

Honorable William J. Martini
United States District Judge
M.L. King Jr. Federal Building & Courthouse
Room 4076
50 Walnut Street
Newark, NJ 07102

Re: *Gunvalson v. PTC Therapeutics, Inc.*
Case No. 2:08-cv-03559-WJM-MF

Dear Judge Martini:

Enclosed is a Supplemental Declaration of Langdon L. Miller, M.D. in further support of PTC Therapeutics, Inc.'s ("PTC") opposition to plaintiffs' motion for preliminary injunction. This supplemental declaration is being filed with the Court's permission. Thank you for your consideration of this matter.

Respectfully submitted,

s/Kenneth R. Meyer (7523)

KRM:kmh
cc: John G. Hutchinson, Esq. (Via Electronic Mail)
Marc E. Wolin, Esq. (Via Electronic Filing)
Michael A. Hatch, Esq. (Via Electronic Filing)

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.	:	

SUPPLEMENTAL DECLARATION OF LANGDON L. MILLER, M.D.

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

I, LANGDON L. MILLER, M.D., pursuant to 28 U.S.C. § 1746, declare as follows:

1. I am the Chief Medical Officer of defendant PTC Therapeutics, Inc. ("PTC"). I submit this supplemental affidavit on behalf of PTC in further 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. I make this affidavit on the basis of my own personal knowledge.

Clinical Testing of PTC124 in Cystic Fibrosis Does Not Support a Conclusion of Drug Safety

2. I understand that the Gunvalsons have claimed in their reply papers that, because PTC has conducted a three-month clinical trial for PTC124 in Cystic Fibrosis ("CF"), there is sufficient safety data available for the drug to justify giving it to patients with Duchenne muscular dystrophy ("DMD") or Becker muscular dystrophy ("BMD") on a long-term basis. This is untrue.

3. As a threshold matter, the three-month CF clinical trial conducted in Israel to which the Gunvalsons have referred involved a very small patient population – only 19 patients at the outset, and, only 17 patients at the end. Moreover, the highest PTC124 dose level that was tested in this trial was lower than the highest dose level that is currently being tested in the Phase 2b clinical trial for patients with DMD/BMD. Testing of PTC124 in CF patients participating in the three-month study involved the administration of PTC124 three times per day at the following dose levels: 4 mg/kg (of body weight) (at breakfast), 4 mg/kg (at lunch) and 8 mg/kg (at dinner) in 11 patients; and 10 mg/kg (at breakfast), 10 mg/kg (at lunch) and 20 mg (at dinner) in 8 patients. All of the patients in the three-month CF study were adults. In PTC's Phase 2b trial and Phase 2a extension trial, twice the amount of the highest dose that has ever been tested in adults with CF will be tested in approximately 90 children with DMD/BMD; these children will receive a dose of 20 mg/kg (at breakfast), 20 mg (at lunch), and 40 mg (at dinner). Obviously, testing of lower dose levels in eight adults with CF for three months does not adequately address the potential risks that may be incurred with testing of a higher dose level in 90 children with DMD/BMD for 12 to 24 months. Only through the consistent monitoring of safety in the Phase 2b study and the Phase 2a extension study in larger numbers of patients with

the relevant disease will the long-term safety profile of PTC124 in the DMD/BMD population be characterized.

4. Given the modest number of subjects in the CF trials described above and the lower dose of PTC124 that they received, the limited safety data obtained through these trials is not sufficient to justify any conclusion that PTC124 is sufficiently safe for more chronic therapy in either CF or DMD/BMD. Just as we must presume that PTC124 is not safe or effective as a DMD/BMD treatment prior to the conclusion of our Phase 2b controlled clinical trial, we currently must assume that PTC124 is neither safe nor effective as a CF treatment until a long-term controlled trial is conducted. In fact, to obtain regulatory approval for PTC124 in nonsense-mutation-mediated CF, we are planning to conduct a separate, placebo-controlled long-term clinical trial to evaluate the safety and efficacy of PTC124 in that specific disease.

The Safety Data from 150 Patients Available in 2007 Does Not Support a Finding of Drug Safety

5. I understand the Gunvalsons are also relying on a statement that I made in a press release PTC released on October 11, 2007, indicating that data from our Phase 2a trials in DMD added to the growing body of safety data for PTC124, which, at that time, had been administered to more than 150 subjects. The figure of more than 150 subjects to which I referred in the press release included subjects who received a single dose of PTC124, subjects who received the drug for 7 days, subjects who received the drug for 14 days, subjects who received the drug for 28 days, and the 19 participants in the three-month CF trial in Israel I have just described. For the reasons set forth above and in my initial affidavit, the safety data to which I referred in this press release were wholly insufficient to draw any conclusions about PTC124's long-term safety or efficacy. It is important to note that the press release made reference to the growing body of safety data, referring to the type of data being described. This is very distinct from stating a

conclusion that the drug is "safe", which I and PTC were careful not to do in the press release. The only conclusion that may scientifically be drawn at this stage is that the prior short-term trials demonstrated a level of safety justifying the conduct of longer-term clinical trials, including controlled clinical trials. As PTC has consistently maintained, until these trials are completed, the drug cannot be considered to have demonstrated safety and efficacy for long-term use.

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

Langdon L. Miller, MD

Executed this 17th day of August, 2008.