

**UNITED STATES DISTRICT COURT
DISTRICT OF NEW JERSEY**



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LETTER OPINION

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RE: Gunvalson v. PTC Therapeutics, Inc.
Civ. No. 08-3559 (WJM)

Dear Counsel:

Plaintiffs, a terminally ill boy named Jacob Gunvalson and his parents, file a **MOTION FOR A PRELIMINARY INJUNCTION** requiring Defendant, a pharmaceutical company named PTC, to provide Jacob with access to one of PTC's FDA-unapproved drugs, PTC124. Plaintiffs argue that PTC is obligated to provide Jacob the drug because PTC promised Jacob access to it, causing him to forgo opportunities to receive the drug in clinical trials. Plaintiffs also argue that FDA regulations allow PTC to provide Jacob with PTC124 even though the FDA has not approved it for general use. The Court finds that these arguments have a reasonable likelihood of success on the merits, which favors preliminary relief. The Court also finds that a balancing of interests and hardships, those of both the public and the parties, also favors preliminary relief. Accordingly, Plaintiffs' motion for a preliminary injunction is **GRANTED**.

I. FACTS AND PROCEEDINGS

The parties have declined the Court's invitation to request an evidentiary hearing, so the Court decides this matter on the written submissions.

Plaintiffs are Jacob Gunvalson, a teenaged boy with Duchenne Muscular Dystrophy ("DMD"), and his parents. DMD causes muscles, including the heart, to degenerate, and its sufferers usually die by age twenty-five. There is no currently available medication for DMD that provides any meaningful treatment.

Defendant PTC, a drug company, is the creator of a drug called PTC124. PTC124 shows promise as a treatment for DMD. PTC124 is currently in a Phase 2 clinical trial.

The dispute in this case is whether PTC induced Jacob to forgo entering a clinical trial by promising him eventual access to PTC124. In early 2006, PTC was to begin a "Phase 2a" clinical trial for PTC124. The trial was to last twenty-eight days, during which time PTC would provide its participants with PTC124.¹ At this time, Jacob was taking Gentamicin, another DMD medication. Jacob's mother, Cherie Gunvalson, asked PTC whether Jacob should enroll in the clinical tests. Enrollment would have required Jacob to stop taking Gentamicin and to obtain a muscle biopsy. According to Plaintiffs, PTC—most notably its Vice President Claudia Hirawat—told Plaintiffs that Jacob should

¹PTC actually conducted two twenty-eight day Phase 2a trials, the second being necessary to give subjects a more appropriate dosage of PTC124, but the distinction between these two trials does not bear on the Court's analysis.

continue taking Gentamicin, which appeared to have some beneficial effect, and wait for later PTC124 clinical trials. According to Plaintiffs, PTC assured Plaintiffs that Jacob would be able to receive PTC124 at a later date. PTC disputes that it made such statements, as discussed below. PTC also disputes that Jacob would have been eligible for these initial clinical trials, also as discussed below. Jacob thus remained on his medication and did not enter the clinical trials.

The trial was a success, and PTC initiated efforts to conduct an expanded Phase 2a trial. This trial was to last two years, again during which PTC would provide its participants with PTC124.

Jacob, whose condition had severely deteriorated by this point, requested to participate in the clinical trials. However, these trials were limited to participants in the initial Phase 2a trial, so Jacob could not participate.²

Jacob, growing desperate, sought to convince PTC to provide him PTC124 through an FDA-regulated “compassionate use” exception to the normal rule that drug companies may not distribute FDA-unapproved drugs. PTC refused.

Plaintiffs then filed this suit against PTC. Plaintiffs generally allege that PTC induced Jacob to forgo his opportunity to participate in the Phase 2a trials with its false promises of PTC124. Plaintiffs sue for promissory estoppel, fraudulent misrepresentation, and negligent misrepresentation.

Plaintiffs now seek a preliminary injunction requiring PTC to provide Jacob with PTC124.

III. DISCUSSION

The Court must consider four factors before granting a preliminary injunction: (1) the moving party’s likelihood of ultimate success on the merits, (2) the irreparable harm to the moving party absent the injunction, (3) the hardship to the non-moving party an injunction would cause, and (4) the public interest. See, e.g., Bennington Foods LLC v. St. Croix Renaissance, Group, LLP, 528 F.3d 176, 179 (3d Cir. 2008). Here, these factors all favor granting the preliminary injunction.

²PTC also at this time began a Phase 2b clinical trial, but it is undisputed that Jacob was not eligible for this trial. The trial required its participants to be ambulatory, and Jacob was not by the time the trial started.

A. The Merit of the Plaintiffs' Claims

As just mentioned, the Court will issue a preliminary injunction only if it is reasonably likely that Plaintiffs will ultimately prevail on the merits. Id. Plaintiffs, however, need not show anything approaching a certainty of success. Highmark, Inc. v. UPMC Health Plan, Inc., 276 F.3d 160, 173 (3d Cir. 2001) (“Moreover, on an application for preliminary injunction, the plaintiff need only prove a prima facie case, not a certainty that he or she will win.”).

Analysis of Plaintiffs' claims is in two parts.³ First, Plaintiffs must show that PTC124 comports with the requirements of the FDA's “compassionate use” exception, which allows drug companies to provide experimental drugs to sick persons. Second, Plaintiffs must show that PTC had some legal obligation to provide Jacob with PTC124.

1. PTC124 Satisfies the “Compassionate Use” Requirements

Although the FDA generally prohibits drug companies from distributing FDA-unapproved drugs, FDA regulations provide for a “compassionate use” exception. Under this exception, the FDA will allow a drug company to distribute an FDA-unapproved drug if four conditions are met: (1) the drug is for a serious or life-threatening disease, (2) there is no good alternative, (3) the drug is currently under investigation in a clinical trial, and (4) the sponsor is actively pursuing marketing approval. 21 C.F.R. § 312.34. Also, even if the drug meets these criteria, the FDA may still deny the compassionate use if there is insufficient evidence of the drug's safety. Id.

Here, PTC124 meets all of these requirements. It clearly and undisputedly meets the first four conditions: (1) PTC124 is for a serious or life-threatening disease, DMD, (2) there is no good alternative to PTC124 for DMD, (3) PTC124 is currently in the midst of PTC's Phase 2 clinical trials, and (4) PTC is actively pursuing market approval. Also, all reports from the clinical trials indicate that PTC is safe to use—and there is no evidence to the contrary. See Dahl, 7 F.3d at 1404 (holding that a drug satisfied the compassionate

³The framework for this analysis comes from Dahl v. HEM Pharmaceuticals Corp., 7 F.3d 1399, 1401 (9th Cir. 1993). The facts of Dahl are quite similar to the facts here: the plaintiffs there sought to compel a drug company to provide them with experimental drugs through a “compassionate use” exception claiming that the drug company had made an enforceable promise to provide those drugs. Id. Also, the parties here appear to accept the legal framework set forth in Dahl. Accordingly, the Court applies the Dahl framework.

use exception because studies showed it was generally safe, even if the FDA expressed concern to the contrary). Accordingly, PTC124 satisfies the requirements for compassionate use.

2. It Is Reasonably Likely That PTC Has a Legal Obligation to Provide PTC124 to Jacob

PTC's contacts with Plaintiffs are reasonably likely to constitute an enforceable promise to provide Jacob with PTC124. Promises without consideration are enforceable if the promisee reasonably relied on them to his detriment. See, e.g., Lobiondo v. O'Callaghan, 815 A.2d 1013, 1020 (N.J. Super. Ct. App. Div. 2003) ("There are four elements to the doctrine of promissory estoppel: 1) a clear and definite promise, 2) made with the expectation that the promisee will rely upon it, 3) reasonable reliance upon the promise, 4) which results in definite and substantial detriment.").

Here, it is reasonably likely that PTC promised Plaintiffs to provide Jacob with PTC124, causing them to forgo enrolling him in the initial Phase 2a trials to their detriment, as Jacob is foreclosed from entering the current clinical trials as a result. Cherie Gunvalson's affidavit evidences many statements from PTC that are reasonably likely to constitute parts of such a promise. For example, Cherie states that she asked PTC Vice President Claudia Hirawat if Jacob should be enrolled in the initial Phase 2a trials, and Hirawat responded that "it was not worth taking Jacob off of Gentamicin for only a 28-day dosage of PTC124." (Aff. of Cherie Gunvalson ¶ 17.) Cherie also stated that she "asked Ms. Hirawat if there were any adverse effects on Jacob for not participating in the trial, and she told me there were none." (Cherie Aff. ¶ 17.) In another encounter shortly after, Hirawat told Cherie "that Jacob had no better or worse chance to be treated in the future based on his non-enrollment in the Phase IIa trial." (Cherie Aff. ¶ 25.) In yet another encounter shortly after, Hirawat "assured [Cherie] that Jacob would get access to PTC124." (Cherie Aff. ¶ 26.) Hirawat's affidavit confirms these assurances. For example, Hirawat states she told Cherie that "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." (Aff. of Claudia Hirawat ¶ 16.) Cherie alleges that other PTC officers made similar statements. For example, Cherie states that PTC Chief Medical Officer Dr. Langdon Miller told Cherie in July 2006 that "once positive results were back from the [Phase 2a] trial, Jacob will get PTC124." (Cherie Aff. ¶ 24.) The Court finds it reasonably likely that Plaintiffs relied on these and other similar statements made by PTC in declining to enroll Jacob in the Phase 2a clinical trials. This failure to enroll Jacob worked to Plaintiffs' detriment, as Jacob is now not eligible for the extended Phase 2a trials. In summary, the Court finds it reasonably likely that Plaintiffs

reasonably relied to their detriment on PTC's promises to provide PTC124 to Jacob, so the Court prevents PTC from denying Plaintiffs that promise.

PTC argues that it never promised Jacob such access to PTC124 or advised him not to participate in the clinical trials. It highlights affidavits from its officers, including Claudia Hirawat and Dr. Miller, denying making such statements.⁴ According to these affidavits, many communications between Plaintiffs and PTC's officers and employees suggested that PTC often may have stopped short of making any actual promises to provide PTC124 and instead may have only suggested that Plaintiffs decide on their own what is best. The evidence is somewhat contradictory on this point.

Nevertheless, the Court finds that there is a reasonable likelihood that PTC made an enforceable promise to Plaintiffs to provide PTC124 to Jacob. As an initial matter, all communications between PTC and Plaintiffs must be viewed in light of the unique relationship between Jacob's mother, Cherie, and PTC. Cherie worked at length to lobby funds from Congress for DMD research. In this capacity, she had relationships with PTC employees and officers that transcended the typical relationship that PTC had with parents of children with DMD. Of most relevance to this Court, Cherie appears to have had extensive communications on both a professional and social level with PTC Vice President Claudia Hirawat. Indeed, it is undisputed that Cherie and Jacob have even stayed overnight at Hirawat's home on at least one occasion. Because of these extended connections alone, it seems to the Court that PTC would be more likely to communicate to Plaintiffs more compassionately and less formally than with other parents of DMD children.

Upon examination of the content of these communications, the Court finds it reasonably likely that PTC promised to provide Jacob with PTC. Most persuasive to the Court, as mentioned above, is the affidavit of Claudia Hirawat, in which Hirawat describes many statements to Cherie that appear tantamount to promises to treat Jacob.

⁴Claudia Hirawat's affidavit is contradictory on this point. At points, Hirawat appears to have accurately conveyed to Plaintiffs the consequences of not enrolling Jacob in the Phase 2a trials. For example, Hirawat states she told Cherie Gunvalson that "participants in the initial 2a trials would likely have a preference over others in terms of future studies." (Hirawat Aff. ¶ 16.) But at other points, Hirawat appears to have contradicted this statement, for example, when she told Cherie 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." (Hirawat Aff. ¶ 16.)

For example, Hirawat states that “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.” This statement alone—but particularly when combined with PTC’s other statements and conduct—is reasonably likely to constitute a promise that Jacob’s non-enrollment in early Phase 2a trials would not preclude his participation in later trials.

More generally, while Plaintiffs’ and PTC’s evidence might at first blush appear inconsistent, the Court finds that on deeper inspection they may not be. While PTC may not have intended to promise PTC124 to Plaintiffs, it appears to the Court that the totality of the circumstances of PTC’s speech and conduct objectively communicated something different. Accordingly, the Court finds it reasonably likely that PTC did promise Plaintiffs to provide Jacob with PTC124, causing him to forgo participation in the initial Phase 2a trial, which ultimately prevented him from participating in the current expanded Phase 2a trial.

PTC also argues that Plaintiffs suffered no detriment from any reliance on PTC’s statements since Jacob was not eligible to participate in the initial Phase 2a trial anyway. To evidence this, PTC points to the declaration of Dr. Richard Finkel, a principal investigator in the Phase 2a clinical trial. In 2006, after the initial Phase 2a clinical trial had begun, Dr. Finkel examined Jacob’s medical files and determined that Jacob didn’t have Duchenne Muscular Dystrophy, or “DMD,” but rather had Becker Muscular Dystrophy, or “BMD.” Dr. Finkel notes that this would have disqualified Jacob from participating in the initial Phase 2a trial anyway.⁵

However, the evidence suggests that Jacob actually does have DMD, not BMD. This was the conclusion of Dr. Brenda Wong, another primary investigator in the PTC124 study. And Dr. Wong based her conclusion that Jacob has DMD on multiple physical examinations of Jacob whereas Dr. Finkel based his contrary conclusion on a single evaluation of Jacob’s medical record. In light of Dr. Wong’s superior opportunity to examine Jacob, the Court finds it reasonably likely that Jacob would have qualified for the initial Phase 2a trial.

⁵PTC also highlights emails from Cherie relaying Dr. Finkel’s findings, but the Court affords these emails no weight. Cherie is not a medical expert and has no firsthand knowledge of whether Jacob has DMD or BMD.

In summary, the Court finds it reasonably likely that Plaintiffs will succeed at trial on their promissory estoppel claim. The Court reiterates again that it does not find, nor need it find, that Plaintiffs are certain to succeed. Rather, the Court finds it reasonably likely that PTC did make such a promise. Accordingly, this factor favors preliminary relief. I will now consider the remaining factors.

B. The Remaining Factors in a Preliminary Injunction Analysis Favor Plaintiffs

The remaining three factors in a preliminary injunction analysis all favor Plaintiffs. Without an injunction mandating that PTC give Jacob PTC124—the only treatment ostensibly offering Jacob any hope for his fatal disease—he is likely to suffer irreparable harm. In contrast, PTC suffers little harm if forced to give Jacob PTC124. While PTC argues that providing Jacob PTC124 would require time-consuming and potentially expensive applications to the FDA under the compassionate use exception, this burden is trivial compared to the potential harm to Jacob without the medication. Indeed, according to PTC, the process of applying to the FDA for a compassionate use exception takes only a period of weeks and appears to be largely a matter of preparing and filing a proper application. Finally, the public has an interest in the provision of possibly life-saving experimental drugs to terminally ill persons, as evidenced by the FDA's enactment of the compassionate use exception.

PTC argues unpersuasively that an injunction would harm it and the public by opening the floodgates to all DMD sufferers who might use litigation to obtain PTC124 without participating in clinical trials. PTC reasons that the development of safe, effective drugs requires clinical testing in which not all may participate or in which some participants may receive placebos. This argument fails for two reasons. First, the FDA has provided and promoted a compassionate use exception, so clearly the FDA has determined that the public interest lies with providing unapproved drugs in situations such as this one. Second, this is a unique case in that PTC appears to have promised this drug to Plaintiffs; an injunction here will not have implications beyond this case.

The Court wishes to take a moment to focus on this final point and reiterate that Plaintiffs appear to be in a unique situation. Cherie's involvement with PTC was extensive and naturally blended between a professional and social relationship, particularly with Claudia Hirawat. The Court strongly doubts that many—if any—other parents of DMD children have this kind of relationship with PTC (or other drug companies). While the Court believes PTC's claim that it normally takes care to refrain from promising any parent access to PTC124 and that it attempted to do so in this case, the Court also finds that Plaintiffs' unusually close relationship with PTC likely muddied

this otherwise clear message. And as explained above, it is reasonably likely that Plaintiffs' reliance on this promise is the reason that Jacob is not currently enrolled in the expanded clinical trial for PTC124. Thus, the Court's ruling today should not in any way suggest that PTC has a general obligation to provide PTC124—or any experimental drug—to sick persons. Indeed, the Court appreciates that sound scientific and medicinal practices may disfavor a drug company doing so. Nevertheless, the Court finds it reasonably likely that PTC made a promise to Plaintiffs to provide Jacob with PTC124, and the Court preliminarily enjoins PTC from breaking that promise.

III. CONCLUSION

In conclusion, it appears reasonably likely that PTC is legally permitted and indeed obligated to attempt to provide Jacob with PTC124. Accordingly, the Court **GRANTS** Plaintiffs' motion for a preliminary injunction.⁶

s/ William J. Martini
William J. Martini, U.S.D.J.

⁶PTC has moved for a stay of this injunction pending its appeal. The Court denies this motion. The standards for granting a stay of an injunction are quite similar to the standards for granting the injunction itself, see Hilton v. Braunskill, 481 U.S. 770, 776 (1987), which the Court has already discussed above. Here the same considerations that favor granting Plaintiffs' motion for a preliminary injunction also disfavor granting a stay of that injunction pending appeal. Most notably, the injunction does not impose a great burden upon PTC whereas without an injunction Plaintiffs are likely to suffer irreparable harm.