UNITED STATES COURT OF APPEALS
FOR THE THIRD CIRCUIT

No. 08-3575

JACOB GUNVALSON; CHERI and JOHN GUNVALSON, as Guardians for Jacob Gunvalson; and CHERI and JOHN GUNVALSON, Individually,

Plaintiffs-Appellees,

v.

PTC THERAPEUTICS, INC.,

Defendant-Appellant.

On Appeal from the United States District Court
for the District of New Jersey

BRIEF OF *AMICI CURIAE* PARENT PROJECT FOR MUSCULAR DYSTROPHY RESEARCH, INC. AND UNITED PARENT PROJECTS MUSCULAR DYSTROPHY SUPPORTING APPELLANT AND URGING REVERSAL

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RULE 26.1 CORPORATE DISCLOSURE STATEMENT

Pursuant to Rules 26.1 and 29 of the Federal Rules of Appellate Procedure and Third Circuit Local Appellate Rule 26.1.1, *Amici Curiae* Parent Project for Muscular Dystrophy Research, Inc. and United Parent Projects Muscular Dystrophy state that they have no corporate parents or affiliates, and no publicly held corporation stockholders.

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STATEMENT OF INTEREST

Parent Project for Muscular Dystrophy Research, Inc. ("PPMD") is an organization focused on Duchenne and Becker muscular dystrophy ("DMD/BMD"). PPMD invests in high risk/high impact research in academia and with industry. PPMD has developed effective collaborations with healthcare professionals to improve clinical care and develop models to increase awareness of DMD/BMD, improve diagnosis, and to provide access to genetic testing. One of the most significant efforts of PPMD is its broad advocacy agenda. PPMD works with Members of Congress to recognize DMD/BMD, the impact of the disorder on families, the relevance of muscle research, the importance of developing clinical and research centers of excellence, and programs designed to accelerate translation into clinical studies. With its professional consultants in Washington, DC (Cornerstone Group), PPMD mobilizes its families to advocate on behalf of DMD/BMD, contact their local representatives in Congress, and advocate for increased federal investment in muscular dystrophy across the National Institutes of Health and in other federal agencies.

United Parent Projects Muscular Dystrophy ("UPPMD") is an international organization dedicated to finding a cure and viable treatments for DMD, to promoting good standards of care, and to informing parents around the world. UPPMD serves as the international umbrella organization for DMD parent project

organizations in many countries, to include PPMD. The objective of UPPMD is to coalesce the collective experiences and resources of national parent projects in an effort to improve care and research for DMD around the world. Because UPPMD, like its national parent project members, is led by parents, UPPMD is a responsive organization that is able to understand the concerns of parents in the DMD community.

Both PPMD and UPPMD maintain an interest in ensuring that the clinical trial process for developing drugs to treat DMD/BMD remains effective and fair. PPMD's and UPPMD's experience in research, collaboration, and advocacy offers this Court a distinct perspective on the serious ramifications of the district court's preliminary injunction order in this case. Because both organizations are led by parents in the DMD/BMD community, PPMD and UPPMD hold a significant stake in the outcome of this case.

ARGUMENT

DMD is a catastrophic illness. Efforts to combat the disease must be aggressive and unimpeded. Unfortunately, the district court's preliminary injunction order in this case, while perhaps well-intentioned, does a disservice to the many individuals who suffer from DMD. Not only does the order circumvent the clinical trial process, the order is patently unfair and will harm ongoing efforts to develop a safe and effective treatment for DMD. For these reasons, PPMD and UPPMD urge this Court to reverse the district court's preliminary injunction order.

I. THE DISTRICT COURT'S ORDER IS UNFAIR, WILL RESULT IN INCREASED LITIGATION OVER ACCESS TO EXPERIMENTAL DRUGS, AND WILL IMPEDE THE DEVELOPMENT OF SAFE AND EFFECTIVE TREATMENTS FOR DMD

The district court's order allows one individual to receive PTC124 while numerous other individuals are forced to wait until the clinical trial process is complete. Such a result is patently unfair and will undoubtedly lead to increased litigation over access to drugs that have yet to complete the clinical trial process. There are many individuals with premature stop mutations that might benefit from access to PTC124. The district court's order accords preferential treatment to one individual and, as a consequence, raises serious issues of fairness. The mean age of death for those afflicted with DMD is approximately 25 years of age. Even if literal death is not imminent, sufferers of DMD are subject to many "little" deaths—heartbreaking milestones involving debilitating conditions where function

is lost and quality of life is diminished. *See, e.g.*, Parent Project for Muscular Dystrophy: Understanding DMD - Progression, http://www.parentprojectmd.org/site/PageServer?pagename=und_progression (last visited Sept. 23, 2008). Simply stated, there are many individuals who could potentially benefit from immediate access to PTC124. It is unfair to allow one individual access while so many others must wait for the clinical trial process to be completed.

Concomitant to the issue of fairness is the concern that the district court's order will lead to an increase in litigation over access to unapproved drugs like PTC124. In its letter opinion, the district court found unpersuasive PTC's argument that an injunction would "open[] the floodgates" to litigation. Dist. Ct. Letter Op. at 8. The court concluded that the injunction "will not have implications beyond this case," citing the "unusually close relationship" between PTC and the Plaintiffs. *See id.* But the district court's view of its order is unrealistic. What the court's order does, in reality, is allow for immediate access to a treatment for an illness (1) that results in significant losses in function by ages 6-12 and death by ages 25-30, and (2) for which there is currently no effective treatment. Given the nature of DMD, any order compelling PTC to distribute PTC124 to one individual will necessarily encourage litigation by other individuals. Indeed, the case has already garnered significant public attention. *See, e.g.*, Michael M. Grynbaum, *Judge Orders Drug Maker to Provide*

Experimental Treatment to Terminally Ill Teenager, N.Y. Times, Aug. 21, 2008, at C3, *available at* http://www.nytimes.com/2008/08/21/business/21dystrophy.html?emc=eta1. In the wake of the district court's ruling, three parents contacted PPMD founder and President Patricia Furlong to inquire whether litigation was an effective means of access to PTC124. *See* Declaration of Patricia Furlong at 2 (Sept. 3, 2008) (attached as Exhibit A to PPMD's Brief in Support of Appellant's Motion for Stay). Importantly, even if a large majority of such claims ultimately prove unsuccessful, there is nothing to stop individuals from bringing law suits, and manufacturers will have to expend resources defending those suits.

Second, in distinguishing the instant case based on the "close relationship" between Plaintiffs and PTC, the district court failed to recognize that close relationships between individuals/family members and industry executives are a common—and, indeed, a vital—occurrence in the research and testing of treatments for rare diseases. *See* Anne-Laure Winkler & David Finegold, *Giving Patients a Say: How to Work with Patient Advocacy Groups*, 26 Nature Biotechnology 1, 2 (January 2008), at JA 90 ("Working with patient groups in the drug discovery stage enables a company to gain a much deeper understanding of the disease and can have a major impact on how it markets its drugs."). Executives develop relationships with family members and individuals in order to understand the progress, burden, and potential impact of therapy. *See* Declaration of Patricia

Furlong, *supra*, at 2. Because conditions like DMD are rare and affect only small subsets of families, the actual number of families able to be engaged is limited. *Id.* Thus, industry executives and their employees repeatedly come into contact with the same individuals and often develop close relationships throughout the drug development process. *Id.* In failing to acknowledge the commonality of close relationships between industry executives and individuals afflicted with DMD and their families, the district court's letter opinion understates the possibility of increased litigation based on theories like promissory estoppel. This case is simply not the narrow case that the district court believes it to be.

Finally, and perhaps ironically, the district court's order will chill the very relationships that it relied upon to grant relief, thereby impeding the timely development of a safe and effective treatment for DMD. As discussed, close relationships between individuals/family members and industry executives are vital to the research and testing of treatments for rare diseases. If the district court's order is allowed to stand, the prospect of increased litigation over access to experimental drugs will almost certainly discourage the very cooperation between families and industry that is so important to the process of developing effective treatments for rare diseases like DMD. Going forward, industry executives will be reluctant to interact with patients and families for fear of exposure to costly promissory estoppel claims like the one in this case. This effect would be

detrimental to all individuals afflicted with DMD. *See, e.g.*, Drug and Device Law, Manufacturer Ordered to Provide Experimental Drug, http://druganddevicelaw.blogspot.com/2008/08/manufacturer-ordered-to-provide.html (last visited Sept. 23, 2008) ("If direct company-patient communications can be the basis for expensive and disruptive litigation, those of us representing companies will advise our clients either not to engage in such communication at all, or to do so only through formal, recorded procedures. As a matter of medical practice and more than that, simple human compassion, we don't think it's a good idea to inhibit such communication between desperate, terminally ill people, and companies that might just be in a position to help. But as lawyers, our first duty is to our clients.").

In sum, the order at issue here has implications that go far beyond this case. If allowed to stand, the order will encourage additional costly litigation and chill interaction between industry executives and families and individuals. Both results will impede the effective development of drugs to treat DMD to the detriment of all individuals afflicted with the illness. For these reasons, the district court's order should be reversed.

II. THE DISTRICT COURT'S ORDER CIRCUMVENTS THE CLINICAL TRIAL PROCESS AND UNDERMINES THE PUBLIC INTEREST IN ACHIEVING WIDESPREAD AVAILABILITY OF EXPERIMENTAL DRUGS

Congress and the FDA established the controlled clinical trial process to ensure that drugs are safe and effective before they become available for

widespread public use. *See* 21 U.S.C. § 355 (a), (b), and (d); *see also Abigail Alliance for Better Access to Developmental Drugs v. von Eschenbach*, 495 F.3d 695, 697-98 (D.C. Cir. 2007). Because completion of the clinical trial process is necessary to achieve the broad availability of any drug, there is a significant public policy interest in ensuring that the process remains viable. The district court's order in this case circumvents the clinical trial process, thereby undermining the informed decisions of Congress and the FDA and the significant public interest in the availability of safe and effective drugs.

The district court's order in this case directly undermines the clinical trial process. That process reflects the considered policy judgments of Congress and the FDA with respect to whether and when a drug may become available for public use. In its letter opinion, the district court dismissed PTC's concerns that granting the Plaintiffs relief would effectively circumvent the clinical trial process, noting that "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." Dist. Ct. Letter Op. at 8. But contrary to the district court's conclusion, the compassionate use exception cannot be relied upon to support the extraordinary preliminary injunction order issued in this case.

While it is true that certain FDA expanded access programs allow individuals early access to experimental drugs under limited circumstances, *see* 21 C.F.R. §§ 312.34, 312.35, the FDA explicitly accounted for the continuing viability of the clinical trial process when it established the regulations governing those programs. *See* 71 Fed. Reg. 75,147, 75,151 (Dec. 14, 2006) ("[I]t is important to ensure that expanded access use does not compromise enrollment in the trials needed to demonstrate the safety and effectiveness of the drug."); *see also* Judy Vale, Note, *Expanding Expanded Access: How the Food and Drug Administration Can Achieve Better Access to Experimental Drugs for Seriously Ill Patients*, 96 Geo. L.J. 2143, 2160 (2008) ("By protecting enrollment in clinical trials, the FDA has made a necessary sacrifice in access to ensure that adequate information on safety and effectiveness is gained, drugs are marketed widely, and poor patients are not forced to bear the burden of producing clinical data."). Thus, unlike the district court's order, expanded access programs were carefully crafted by the FDA to ensure that the clinical trial process remains viable. And because these programs involve the *voluntary* participation of drug sponsors, it is unclear how they can be used to justify the judicial compulsion extant in this case.

Moreover, unlike the expanded access programs, the district court's order will directly undermine the clinical trial process, threatening to impede the widespread availability of lifesaving drugs. Clinical trials are vital to the

development and approval of new drugs. *See* Larry Thompson, *Experimental Treatments? Unapproved but Not Always Unavailable*, FDA Consumer Magazine (Jan.-Feb. 2000), at JA 143. It is not always the case, however, that a patient participating in a clinical trial will receive the experimental drug. Some patients will receive the drug, while others might receive a placebo. As a consequence, any ability to receive the experimental drug outside of the clinical trial process could greatly diminish the incentive for a patient to participate in a clinical trial. Indeed, if a patient can obtain the drug notwithstanding participation in the process, there is little to encourage the patient to participate. *See* 71 Fed. Reg. at 75,150 ("[A] system of blindly permitting uncontrolled access to investigational drugs could make it difficult or impossible to enroll adequate numbers of patients in clinical trials"); *see also* Vale, *supra*, at 2158 (noting that "granting easier access to unapproved drugs could create a real risk to the viability of clinical trials necessary for market approval."); Gina Kolata, *Innovative AIDS Drug Plan May Be Undermining Testing*, N.Y. Times, Nov. 21, 1989, at A1 (discussing government initiative to provide drugs to AIDS patients outside of the clinical testing process and noting that "75 patients had volunteered for the clinical trial, which requires 1,900 people, but 1,300 had applied to receive the drug outside the trial."). The district court's order, in allowing a single individual to demand and obtain access to an experimental drug outside of the clinical trial process, will encourage patients

to opt for litigation rather than risk receiving a placebo during the clinical process. The order therefore undercuts the public interest in ensuring adequate participation in the clinical process.

In sum, the district court's order circumvents the considered policy judgments of Congress and the FDA. The general public has a significant interest in achieving the widespread availability of lifesaving drugs. This availability depends upon successful completion of the clinical trial process. Because the district court's order threatens the viability that process, this Court should reverse the order.

CONCLUSION

For the foregoing reasons, PPMD and UPPMD urge this Court to reverse the preliminary injunction order of the district court.

Respectfully submitted,

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CERTIFICATE OF SERVICE

I certify that on this 30th day of September, 2008, I served a copy of the Brief of *Amici Curiae* Parent Project for Muscular Dystrophy Research, Inc. and United Parent Projects Muscular Dystrophy Supporting Appellant and Urging Reversal on the following via Federal Express mail:

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CERTIFICATION OF BAR MEMBERSHIP

JULIA B. MEISTER certifies as follows:

1. I am a member in good standing of the Bar of the United States Court of Appeals for the Third Circuit.

2. Pursuant to 28 U.S.C. § 1746, I certify under penalty of perjury that the foregoing is true and correct.

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Dated: September 30, 2008

CERTIFICATION OF COMPLIANCE PURSUANT TO FEDERAL RULE OF APPELLATE PROCEDURE 32(a)(7)(C)

The attached Brief of *Amici Curiae* Parent Project for Muscular Dystrophy Research, Inc. and United Parent Projects Muscular Dystrophy complies with the type-volume limitation of Federal Rule of Appellate Procedure 32(a)(7)(B) because the brief contains 2,354 words, excluding the parts of the brief exempted by Federal Rule of Appellate Procedure 32(a)(7)(B)(iii).

The attached brief also complies with the typeface requirements of Federal Rule of Appellate Procedure 32(a)(5) and the type style requirements of Federal Rule of Appellate Procedure 32(a)(6) because this brief has been prepared in a proportionately-spaced typeface, using Microsoft Office Word 2002 in 14 point Times New Roman font.

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CERTIFICATE OF COMPLIANCE WITH L.A.R. 31.1

Certificate of Compliance With Electronic Filing Requirements:

1. This brief complies with the electronic filing requirements of L.A.R. 31.1(c) because :

a. the text of this electronic brief is identical to the text of the paper copies.

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