

**IN THE UNITED STATES COURT OF APPEALS  
FOR THE THIRD CIRCUIT**

**PTC Therapeutics,**

*Appellant,*

v.

**C.A. No. 08-3575**

**Jacob Gunvalson, et al.,**

*Appellees.*

**MOTION FOR LEAVE TO FILE A STATEMENT IN SUPPORT OF  
APPELLANT'S MOTION FOR A STAY AS AMICUS CURIAE**

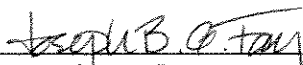
The Biotechnology Industry Organization (BIO) submits this motion in regard to the above-referenced matter, which is before the Court on appeal from the August 21, 2008 Order and Mandatory Preliminary Injunction entered by Judge William J. Martini, U.S. District Court, District of New Jersey. Appellant PTC Therapeutics has sought a stay of that Order. BIO now seeks leave pursuant to F.R.A.P. 29 to submit as *amicus curiae* the attached Statement In Support Of Appellant's Motion For Stay.

BIO represents more than 1,200 biotechnology companies, academic institutions, state biotechnology centers and related organizations across the United States and in more than 30 other nations. BIO members are involved in the research and development of innovative healthcare, agricultural, industrial and environmental

biotechnology, thereby expanding the boundaries of science to benefit humanity by providing better healthcare, enhanced agriculture, and a cleaner and safer environment. These healthcare member companies are invested in the development of new medicines to treat serious and life-threatening conditions, including cancer, heart disease, Alzheimer's, AIDS, multiple sclerosis, and many rare diseases that affect children and adults worldwide. Given this focus, BIO members have a critical interest in maintaining the strength and integrity of the clinical trial system.

As set forth in the attached Statement in Support of Appellant's Motion for Stay, it is important for the Court to hear BIO as an *amicus* because the issues raised in this case affect BIO's healthcare member companies and the drug development process upon which they rely. In addition, as set forth below, the District Court's Order and Mandatory Preliminary Injunction have a potentially harmful impact on BIO's healthcare members. Accordingly, BIO moves for leave to file as *amicus* in support of Appellant's motion for stay\*.

Respectfully submitted

  
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Joseph B. G. Fay  
Morgan, Lewis & Bockius LLP  
1701 Market Street  
Philadelphia, Pa 19103  
215.963.5000

\* BIO is filing separately herewith its Corporate Disclosure Statement and Statement of Financial Interest. BIO has no parent corporations.

Sandra J. P. Dennis  
Deputy General Counsel for Healthcare  
Biotechnology Industry Organization  
1201 Maryland Avenue, S.W.  
Suite 900  
Washington, D.C. 20024  
202.962.6673

Of Counsel:

Stephen Paul Mahinka  
Morgan, Lewis & Bockius LLP  
1111 Pennsylvania Avenue, N.W.  
Washington, D.C. 20004  
202.739.3000

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**STATEMENT OF AMICUS CURIAE BIO IN SUPPORT OF  
APPELLANT'S MOTION FOR STAY**

The District Court's Order and Mandatory Preliminary Injunction raise significant issues with regard to the drug development and clinical trials processes, as well as the nature of healthcare company relationships with patients. Given the broad policy implications of the District Court's ruling, and the potential for harm to BIO's healthcare members, it is important that the Court grant the motion for stay.

BIO's healthcare members invest significant effort in the process of drug discovery and development. Controlled clinical trials are the backbone of clinical drug development. The purpose of such clinical trials is to investigate whether an experimental drug is safe and effective. A drug must go through both the clinical trials process and the U.S. Food and Drug Administration ("FDA") approval

process before it can be made available to patients because an experimental drug is, by definition, not yet proven to be safe or beneficial to patients. Many clinical trials conducted on experimental drugs are not ultimately successful; that is the nature of the scientific process. If the trials are completed successfully, the data from these trials, demonstrating safety and effectiveness, then provides the basis for review and approval by the FDA. FDA must make that determination so there is assurance of safety and effectiveness upon which patients and their physicians can rely. Once FDA approves a medicine, it can then be made broadly available to the patient population that needs it.

Since a new drug can be proven to be safe and effective only through clinical trials, providing a drug outside of a trial could remove an incentive for patients to participate, knowing that some subjects are provided the experimental drug, and others are provided with a placebo or an alternate therapy. Without sufficient participation in a clinical trial for a particular drug, that trial will not proceed, and that experimental drug will not be developed. Thus, lack of participation in clinical trials would lead to many drugs being left on the laboratory shelf, and the opportunity to gain FDA approval and make treatments broadly available to patients would be lost. Ordering a company that is developing a drug to provide it to a particular patient could undermine the clinical trial and drug

approval processes upon which BIO's healthcare company members and millions of patients rely.

BIO and its member companies also routinely develop and maintain strong ties with patients and the patient advocacy community. These relationships create opportunities to collaborate and assist each other in policy development, advocacy, study development, patient identification and recruitment, and other efforts to achieve the mutual goal of enabling patient access to safe, effective and innovative therapies.

This case also raises significant issues regarding such relationships. If these types of routine relationships are deemed to carry special legal obligations, as the Order referenced above reflects, it could have a chilling effect on such beneficial interactions.<sup>1</sup> In turn, without such collaboration and coordination, efforts that have successfully evolved over the last few decades to meet healthcare research & development and commercialization milestones could be negatively affected. BIO's health care members could potentially suffer harm if patients pursue litigation to obtain access to experimental drugs, rather than participating in the established clinical trial process.

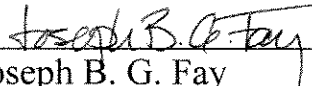
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<sup>1</sup> See "Compassionate Use: Changes You Should Make Following *Gunvalson et al v. PTC Therapeutics*", an educational audioconference to address changes in compassionate use policies and interactions with patients in light of the August 21 District Court decision.

<http://www.thompsoninteractive.com/upcoming.asp?topic=en&id=940&priority=FKYG50731>

BIO believes that the significance of the issues raised warrants a stay of the Order and Mandatory Preliminary Injunction until there is a full hearing on the merits of the case. For the reasons stated above, BIO requests that the Court grant the motion for stay.

Respectfully submitted

  
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Joseph B. G. Fay  
Morgan, Lewis & Bockius LLP  
171 Market Street  
Philadelphia, Pa 19103  
215.963.5000

Sandra J. P. Dennis  
Deputy General Counsel for Healthcare  
Biotechnology Industry Organization  
1201 Maryland Avenue, S.W.  
Suite 900  
Washington, D.C. 20024  
202.962.6673

Of Counsel:

Stephen Paul Mahinka  
Morgan, Lewis & Bockius LLP  
1111 Pennsylvania Avenue, N.W.  
Washington, D.C. 20004  
202.739.3000

**CERTIFICATION OF BAR MEMBERSHIP**

I, Joseph B. G. Fay, hereby certify that I am a member in good standing of the Bar of the United States Court of Appeals for the Third Circuit.

  
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Joseph B. G. Fay

**CERTIFICATE OF SERVICE**

I certify that on this 29th day of August, 2008, I served a copy of the Motion for Leave to File a Statement in Support of Appellant's Motion for Stay as Amicus Curiae and the attached Statement of Amicus Curiae Bio in Support of Appellant's Motion for Stay on the following via Federal Express mail:

Jakob B. Halpern, Esq.  
Marc E. Wolin, Esq.  
Saiber, Schlesinger, Satz & Goldstein  
One Gateway Center  
Suite 1300  
Newark, NJ 07102-5311


*Counsel for Plaintiffs-Appellees*

John G. Hutchinson, Esq.  
John J. Lavelle, Esq.  
Sidley Austin  
787 Seventh Avenue  
New York, NY 10019-0000

Kenneth R. Meyer, Esq.  
Porzio, Bromberg & Newman  
100 Southgate Parkway  
Morristown, NJ 07962-0000

*Counsel for Defendant-Appellant*

Dated: August 29, 2008

  
\_\_\_\_\_  
Joseph B.G. Fay  
MORGAN, LEWIS & BOCKIUS LLP  
1701 Market Street  
Philadelphia, PA 19103-2921  
(215) 963-5000