

National Organization for Rare Disorders, Inc.®

NORD • 100 Rt. 37, P.O. Box 8923 • New Fairfield, CT 06812-8923

Tel: (203) 746-6518 • FAX: (203) 746-6481

TDD (for hearing impaired) (203) 746-6927

<http://www.rarediseases.org> • e-mail: orphan@rarediseases.org



... out of the darkness.
into the light ...

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Alpha One Foundation
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Wegener's Granulomatosis Support Group, Inc.
Williams Syndrome Association
Wilson's Disease Association

February 28, 2001

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Dockets Management Branch
Food & Drug Administration
5630 Fishers Lane
Room 10-61
HFA-305
Rockville, MD 20852

Re: FR18ja01-28: Public disclosure of certain information related to clinical trials involving human gene therapy and xenotransplantation.

Dear Sirs:

The National Organization for Rare Disorders (NORD) firmly supports the FDA's proposed rule that would require public disclosure of certain information related to gene therapy and xenotransplantation clinical trials. These leading edge technologies have raised the public's sensitivity about their unique public health risks, and their potential to irrevocably modify the human genome. In order to earn and maintain the public's trust, it is critically important to give the public access to relevant information about clinical trials including where they are located, explanations of adverse events, informed consent documents, etc.

The public does not want access to proprietary information, however, nor should it have access to any individually identifiable data. But, the public should have access to information about public health and safety issues that may affect research volunteers and/or the public at large. Secrecy of adverse event data makes the public suspicious about secretive human experimentation (known as "The Frankenstein Syndrome"), leading to fear and politically charged opposition to important advancements of scientific technology.

We are especially concerned that after the death of Jesse Gelsinger, reports of over a thousand adverse events in gene therapy experiments were revealed to have gone unreported to the National Institutes of Health (NIH) Recombinant DNA Advisory Committee (RAC). By keeping this information secret, people who volunteered to be in these trials were not told of serious side effects suffered by others already in the trials. Even more importantly, other scientists using the same vectors were not warned how to avoid similar adverse events in their patients because they remained unaware of their occurrence.

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Acid Maltase Deficiency Association
ALS Association/Greater Philadelphia Chapter
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Parent to Parent of New Zealand
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Recurrent Respiratory Papillomatosis Foundation
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Sickle Cell Disease Association of Texas
Gulf Coast Society For Progressive Supranuclear Palsy, Inc.
Sotos Syndrome Support Association

Takayasu's Arteritis Association
Taiwan Foundation for Rare Disorders
Teicher Collins Foundation

* Associations are joining continuously.
For newest listing, please contact the NORD office

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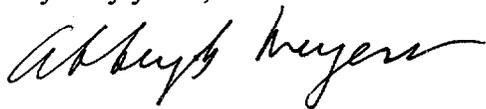
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Food & Drug Administration
February 28, 2001
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Scientific study is a long and painstaking process that builds one brick of knowledge on top of another. The progress of new cutting edge technologies such as gene therapy and xenotransplantation relies on sharing of information in order to advance the field. Because of proprietary interests, there is no reason for an investigator to reveal the construct of a vector or reagents, but it is absolutely necessary to reveal the dead ends and mistakes, in order to diminish human dangers, save precious resources, and to propel the field forward.

Public fear of new scientific technologies can be clearly seen in the current public debate about genetically modified foods, as well as xenotransplantation. On the one hand, the public wants the new technologies, yet it fears the consequences. Making clinical research on these technologies more transparent will allay public fear because people will perceive a more open and responsive clinical research system that acts in the best interest of human subjects. The FDA is the most important consumer protection agency that patients have against scientific catastrophe. Continuing to be secretive about adverse events in these clinical trials simply caters to the public's mistrust and suspicion, thus delaying real scientific advancements.

People with rare diseases are counting on the success of gene therapy, xenotransplantation, and other politically charged areas of scientific investigation. We believe that public access to more information about these clinical trials, and the emergence of adverse events, is critically important to the progress of these technologies and absolutely essential to the public's trust.

Very truly yours,



Abbey S. Meyers
President

ASM:aa

cc: Dr. Kathy Zoon
Dr. Phil Noguchi

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