

National Organization for Rare Disorders, Inc.[®]

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MEMBER ORGANIZATIONS

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

January 22, 2001

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Dockets Management Branch (HFA-305)

FDA

5630 Fishers Lane

Room 1061

Rockville, MD 20852

Re: Availability of Public Disclosure and Submission to FDA for Public Disclosure of Certain Data and Information Related to Human Gene Therapy or Xenotransplantation. Proposed Rule, January 18, 2001, Federal Register, Vol.66, Number 12, pgs 4688-4706.

Dear Sirs:

The proposed rules for public disclosure of certain information regarding human gene therapy and xenotransplantation experiments are excellent and long overdue. We congratulate the FDA on recognizing the importance of the public's access to information about controversial new technologies. In fact, if the same disclosure requirements would be extended to genetically-modified foods and other controversial areas of science, these technologies would probably not be controversial. The public fears what it does not understand, and transparency of FDA's regulatory process gives the public confidence that the agency is protecting the public's health.

Gene therapy and xenotransplantation are the two most controversial areas of science because they have the potential for unique public health risks. Modification of the human genome could affect future generations, and infectious viruses jumping from animals to humans represent the possibility of future catastrophic epidemics. Removing the shroud of secrecy from these experiments will restore public confidence in the FDA's role as the guardian of public health.

The proposed rules call for submission of information to the FDA that is ordinarily submitted to the agency and/or the NIH, but with confidential information removed (including all personally identifiable patient information). By making this information available to the public, HHS will reduce fear, promote understanding, and encourage research subjects to volunteer for clinical trials.

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Acid Maltase Deficiency Association
ALS Association/Greater Philadelphia Chapter
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Society For Progressive Supranuclear Palsy, Inc.
Sotos Syndrome Support Association

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

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

Dedicated to Helping People with Orphan Diseases

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We understand the rules will govern all experiments under an active IND, and we assume that they will continue to govern the entire process of research from Phase I to Phase IV. We suggest that the final rules should clearly stipulate that all phases of clinical research will be covered by the rules.

Although consumers have long urged the FDA to make this information public, the decision to publish the regulations may have been precipitated by the controversial death of a young man in a gene therapy trial during 1999. In this case, the FDA approved an informed consent document that indicated the experiment was not without risk, and monkeys had died in the pre-clinical trials. Apparently, the informed consent document was later changed without informing the FDA or NIH, and mention of the monkey deaths was deleted in the document that the young man signed. If this new rule had been in effect, the original FDA-approved informed consent document would have been available on the Internet, and deletion of critically important sentences would not have occurred without the knowledge and permission of the FDA.

As long as trade secret and commercial or financial information, which is privileged or confidential, is omitted from the required information, companies should not object to these submissions. The public wants and needs transparency of the FDA's oversight system for new technologies, and these proposed rules will do much to restore the public's trust in clinical research.

We suggest that the most useful information for the public will be the non-technical abstracts and the informed consent documents. To this end, we question what will happen when a multi-site protocol results in several different informed consent documents because each institution requires different wording. We suggest that in such cases all of the informed consent documents should be posted so that a patient volunteering at one site would be able to read the documents being used for the same experiment at other sites. We also reiterate that original informed consent documents should remain on-line while updated versions are added. This would provide information to the public about changes that are made as the experiment proceeds.

We find that parts of the regulations are confusing with respect to reporting of safety data. The rules will require an annual report for each experiment, but will the FDA make adverse event reports available to the public as they occur? As the *Federal Register* proposal noted, one of the most important events of gene therapy history was the occurrence of a severe adverse event in a cystic fibrosis patient, which was quickly shared with the scientific community. That adverse event advanced the scientific understanding of the immune system's reactions to vectors, and enhanced the safety of patients in other gene therapy trials. Thus the immediate dissemination of information about adverse events is critically important, and the proposed regulations should make it clear that ongoing safety information will be added throughout the year for the public's perusal.

We wish to reaffirm the importance of the proposed FDA regulations as an essential supplement to the public information responsibilities of the NIH Recombinant DNA Advisory Committee (RAC). The NIH is not a regulatory agency. Thus RAC can only make public information about clinical trials that involve federal funding. The FDA, however, proposes to release information about publicly and privately-funded research involving gene transfers or xenotransplantation. This will enable the public to understand these new technologies, and thus will reduce fear and promote public trust at a time when trust in the research enterprise has eroded. The regulations also represent a major step forward for biomedical research because it will enable scientists to learn from each other so they may avoid duplication, preserve resources, and reduce risks to patients.

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We heartily endorse the proposed FDA disclosure regulations for gene therapy and xenotransplantation research.

Very truly yours,

A handwritten signature in cursive script, appearing to read "Abbey S. Meyers".

Abbey S. Meyers
President

ASM:aa

cc: Kathy Zoon, M.D.
Marlene Haffner, M.D.
Amy Patterson, M.D.



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 into the light ... ®

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