# National Organization for Rare Disorders, Inc.®

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February 11, 2003

Dr. Mark B. McClellan, Commissioner
Food and Drug Administration
Bldg. PKLN, RM 1471, Mail Stop HF-1
5600 Fishers Lane
Rockville, MD 20857

Re: Waiver of Application Fees for Orphan Products: Orphan Medical Citizen Petition, January 28, 2003

Dear Dr. McClellan:

On behalf of the National Organization for Rare Disorders (NORD), its member organizations and the over 25 million Americans affected by rare "orphan" diseases, we are writing in support of the Citizen Petition filed by Orphan Medical on January 28, 2003.

Specifically, the Citizen Petition requests the following actions:

- Revoke the Center for Drug Evaluation and Research (CDER) policy of determining that a company is "in arrears" with respect to payment of a user fee during the period that a waiver is under consideration
- Establish a clear and fair waiver policy from the establishment and product fees for orphan drugs that have modest sales.

By definition, "orphan" products are treatments for rare conditions that have small potential markets and thus are not attractive to the commercial sector. Such treatments were not being developed for "orphan" diseases by the private sector until the *Orphan Drug Act* was enacted in 1983 because these treatments were understood to have limited commercial value. Therefore, NORD is very concerned that the Food and Drug Administration (FDA) is requiring small companies that develop orphan drugs to pay user fees on very low profit, low volume products prior to a waiver being granted. Congress clearly grants the agency the authority to grant waivers for orphan drug user fees (H.R. Rep. No. 102-895, at 17: 1992).

Firstly, it is our concern that the current policy will deter companies from developing and bringing to market lifesaving therapies for small patient populations. We ask that fees be paid only **after** a waiver has been denied, and not before.



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Secondly, Orphan Medical's petition clearly illustrates that the annual gross revenue for Elliots B Solution is $30,000 to $40,000, but the product fee alone is $32,400. For another orphan product, Sucraid, the user fee amounts to 40 percent of the gross annual revenue of $500,000. Based on this information, it is clear that waivers should be based on individual U.S. sales of each orphan drug or biologic, not on total worldwide sales of the company.

Thirdly, the draft interim guidance for waivers or reductions of user fees was adopted without notice-and-comment rulemaking. The public should have had an opportunity to object to this policy especially since small companies will have to pass these costs on to consumers through higher prices.

Congress has clearly stated that orphan drugs should not pay user fees unless the FDA finds that they are as profitable as other drugs for prevalent diseases. We quite agree that if in five years Elliots B solution, Sucraid, or any other orphan therapies, are being used for non-orphan indications, then by all means institute user fees. Until then, however, we ask that the agency consider the health and welfare of babies and children who would die without access to these extraordinarily important orphan drugs.

Finally, the federal government must have a consistent over-all policy that fosters orphan drug development from the laboratory to the patient's bedside. Current policy can only discourage the growth of many small companies like Orphan Medical that devote their limited resources and talents to the orphan drugs that larger companies refuse to make.

The National Organization for Rare Disorders (NORD) is a non-profit voluntary health agency dedicated to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and services to patients and families. We would welcome the opportunity to meet with you regarding this issue as well to discuss the unique needs of the rare disease community

Again, thank you for your consideration of this request. I look forward to your reply.

Very truly yours,

Abbey S. Meyers
President

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cc: Marlene Haffner, M.D., Director, FDA Office for Orphan Products