

National Organization for Rare Disorders, Inc.®



out of the darkness
into the light

NATIONAL MEMBER ORGANIZATIONS

Alagille Syndrome Alliance
Alpha 1 Association
Alpha 1 Foundation
American Brain Tumor Association
American Laryngeal Papilloma Foundation
American Porphyria Foundation
American Syringomyelia Alliance Project
Amyotrophic Lateral Sclerosis Association (ALS)
Aplastic Anemia & MDS International Foundation, Inc.
Association for Glycogen Storage Disease
Association of Gastrointestinal Motility Disorders, Inc. (AGMD)
Batten Disease Support & Research Association
Benign Essential Blepharospasm Research Foundation
Charcot-Marie Tooth Association
Chromosome 18 Registry Research Society
Cleft Palate Foundation
Cornelia De Lange Syndrome Foundation
Cystinosis Foundation, Inc.
DEBRA of America
Dysautonomia Foundation, Inc.
Dystonia Medical Research Foundation
Ehlers Danlos National Foundation
Epilepsy Foundation
Families of Spinal Muscular Atrophy
Foundation for Ichthyosis and Related Skin Types
Genetic Alliance
Guillain Barre Syndrome Foundation International
Hemochromatosis Foundation
Hereditary Colon Cancer Association
Hereditary Disease Foundation
HHT Foundation International, Inc.
Histocytosis Association of America
Huntington's Disease Society of America
Immune Deficiency Foundation
International FOP Association, Inc.
International Joseph Diseases Foundation, Inc.
International Rett Syndrome Association
Interstitial Cystitis Association
Lowe Syndrome Association, Inc.
Mastocytosis Society, Inc.
Mucopolidiosis Type IV Foundation, Inc.
Myasthenia Gravis Foundation of America, Inc.
Myeloproliferative Disease Research Center
Myositis Association of America, Inc.
Narcolepsy Network, Inc.
National Adrenal Disease Foundation
National Alopecia Areata Foundation
National Ataxia Foundation
National Foundation for Ectodermal Dysplasias
National Hemophilia Foundation
National Marfan Foundation
National MPS Society, Inc.
National Multiple Sclerosis Society
National Neurofibromatosis Foundation
National PKU News
National Spasmodic Torticollis Association
National Tay Sachs & Allied Diseases Association
National Urea Cycle Disorders Foundation
Neurofibromatosis, Inc.
Osteogenesis Imperfecta Foundation
Parkinson's Disease Foundation, Inc.
Platelet Disorder Support Association
Prader Willi Syndrome Association, USA
Pulmonary Hypertension Association
PXE International, Inc.
Reflex Sympathetic Dystrophy Syndrome Association
Scleroderma Foundation
Sickle Cell Disease Association of America
Stevens Johnson Syndrome Foundation
Sturge-Weber Foundation
The Erythromelalgia Association
The Oxalosis and Hyperoxaluria Foundation
The Paget Foundation
Tourette Syndrome Association
Trigeminal Neuralgia Association
United Leukodystrophy Foundation
United Mitochondrial Disease Foundation
VHL Family Alliance
Wegener's Granulomatosis Association
Williams Syndrome Association
Wilson's Disease Association

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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.

Associate Member Organizations

Acid Maltase Deficiency Association (AMDA)
Alternating Hemiplegia of Children Foundation
American Autoimmune Related Disease Association
American Behcet's Disease Association
Amyotrophic Lateral Sclerosis (ALS) of Greater Philadelphia Chapter
A-T Children's Project
(The) CDG Family Network Foundation
Canadian Organization for Rare Disorders (CORD)
Children's PKU Network

Chromosome Deletion Outreach Inc.
Chronic Granulomatous Disease Association
CLIMB
Consortium of Multiple Sclerosis Centers
Contact A Family
Cooley's Anemia Foundation, Inc.
Cushing Support & Research Foundation, Inc.
Family Caregiver Alliance
Family Support Network of North Carolina
Freeman-Sheldon Parent Support Group

Hydrocephalus Association
Incontinentia Pigmenti International Foundation
K T Support Group
Late Onset Tay Sachs Foundation
Les Turner ALS Foundation Ltd
Mercy Medical Airlift
National Lymphedema Network, Inc.
National Niemann-Pick Disease Foundation
National Spasmodic Dysphonia Association
Organic Acidemia Association

Osteoporosis and Related Bone Diseases National Resource Center
Parent to Parent New Zealand, Inc.
Rare & Expensive Disease Management Program (REM)
Recurrent Respiratory Papillomatosis Foundation
Restless Legs Syndrome Foundation
Saroid Networking Association
Shwachman - Diamond Syndrome International
Society for Progressive Supranuclear Palsy, Inc.

Solus Syndrome Support Association
St. Claire's Health Services
Taiwan Foundation for Rare Disorders
Takayasu's Arteritis Association

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

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Dedicated to Helping People with Orphan Diseases

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Dr. Mark B. McClellan, Commissioner
February 11, 2003
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Secondly, Orphan Medical's petition clearly illustrates that the annual gross revenue for Elliotts 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.

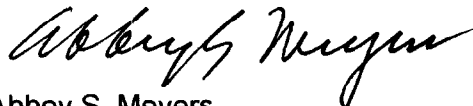
Congress has clearly stated that orphan drugs should not pay user fees unless the FDA finds that they are as profitable as other drugs for prevalent diseases. We quite agree that if in five years Elliotts B solution, Sucraid, or any other orphan therapies, are being used for non-orphan indications, then 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

ASM:aa

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