

National Organization for Rare Disorders, Inc.®

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

January 12, 2007

Division of Dockets Management (HFA-305)
Food and Drug Administration
5630 Fishers Lane, Room 1061
Rockville, MD 20852

RE: Federal Register
Proposed Rule: Food and Drug Administration
Docket No. 2006N-0061
Promotion and Charging of Investigational Drugs
21 CFR Part 312, RIN 0910-AF13

Dear Sir or Madam:

The National Organization for Rare Disorders (NORD) is a non-profit voluntary health agency dedicated to the identification, treatment, and cure of rare "orphan" diseases through programs of education, research, advocacy, and services. There are an estimated 25 million Americans with rare disorders that are defined under the Orphan Drug Act of 1983 as diseases or conditions that each affects fewer than 200,000 Americans. There are approximately 6,000 known rare disorders.

We are concerned that FDA's Proposed Regulation, "Charging for Investigational Drugs," may have serious consequences for patients. The rule suggests that reimbursement may often be requested when the cost of manufacturing is extraordinarily high, when an approved drug must be obtained from another manufacturer, and when sponsor-investigators conduct small trials at single sites and are not conducting the research for commercial purposes.

We agree that academic-based clinical research on approved drugs for off-label uses that are commercially less viable is very important and should be encouraged. Academic investigators tend to have limited funding, and will likely benefit from recovering some of their costs. However, we are concerned that the proposed rule will enable for-profit companies to charge for the most expensive investigational drugs that are unaffordable for most patients.

2006N-0061

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NATIONAL MEMBER ORGANIZATIONS

Alagille Syndrome Alliance
Alpha 1 Association
Alpha 1 Foundation
American Brain Tumor Association
American Porphyria Foundation
American Stryngomyelia Alliance Project
Aplastic Anemia & MDS International Foundation, Inc.
Association for Glycogen Storage Disease
Association of Gastrointestinal Motility Disorders, Inc. (AGMD)
Association for Frontotemporal Dementias (AFTD)
Batten Disease Support & Research Association
Benign Essential Blepharospasm Research Foundation
Charcot-Marie Tooth Association
Children's Tumor Foundation
Chromosome 18 Registry Research Society
Cicatricial Alopecia Research Foundation (CARF)
Cleft Palate Foundation
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Cystinosis Foundation, Inc.
Dystrophic Epidermolysis Bullosa Research Association of America-DEBRA
Dysautonomia Foundation, Inc.
Dystonia Medical Research Foundation
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GBS/CIDP Foundation International
Genetic Alliance
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Hereditary Disease Foundation
HHT Foundation International, Inc.
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Immune Deficiency Foundation
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National PKU News
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Neurofibromatosis, Inc.
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Paget Foundation for Page's Disease of Bone & Related Disorders
Parkinson's Disease Foundation, Inc.
Platelet Disorder Support Association
Prader Willi Syndrome Association, USA
Pulmonary Hypertension Association
Reflex Sympathetic Dystrophy Syndrome Association
Sarcoma Foundation of America
Scleroderma Foundation
Stevens Johnson Syndrome Foundation
Sturge-Weber Foundation
The Oxalosis and Hyperoxaluria Foundation
Tourette Syndrome Association
Trigeminal Neuralgia Association
United Leukodystrophy Foundation
United Mitochondrial Disease Foundation
Vasculitis Foundation
Vestibular Disorders Association, VEDA
VHL Family Alliance
Williams Syndrome Association
Wilson's Disease Association

Associate Member Organizations

Acid Maltase Deficiency Association (AMDA)	Children's PKU Network	Family Support Network of North Carolina	Mercy Medical Airlift	Sarcoid Networking Association
ALS Association- Greater Philadelphia Chapter	Chromosome Deletion Outreach Inc.	Fibromuscular Dysplasia Society of America	National Lymphedema Network, Inc.	Shwachman - Diamond Syndrome Foundation
American Autoimmune Related Disease Association	Chronic Granulomatous Disease Association	Freeman-Sheldon Parent Support Group	National Niemann-Pick Disease Foundation	Society for Progressive Supranuclear Palsy, Inc.
American Behcet's Disease Association	CLUMB	GOLD, Global Organization for Lysoosomal Diseases	National Organization for Albinism and Hypopigmentation, NOAH	Sotos Syndrome Support Association
American Self Help Group Clearinghouse	Coalition for Pulmonary Fibrosis	Hereditary Neoropathy Foundation	National Spasmodic Dysphonia Association	Syncope Trust & Reflex Anoxic Seizures Assn. (STARS)
American Child Health Clearinghouse	Consortium of Multiple Sclerosis Centers	Hydrocephalus Association	Organic Acidemia Association	Taiwan Foundation for Rare Disorders
A-T Children's Project	Contact A Family	Incontinentia Pigmenti International Foundation	Osteoporosis and Related Bone Diseases National Resource Center	Wisconsin Chronic Fatigue Syndrome Association
Canadian Organization for Rare Disorders (CORD)	Cushing Support & Research Foundation, Inc.	Klippel-Trenaunay Support Group	Parent to Parent New Zealand, Inc.	Associations are joining continuously. For newest listing, please contact the NORD office.
(The) CDG Family Network Foundation	Cutus Laxa Internationale	Les Turner ALS Foundation, Ltd.	Recurrent Respiratory Papillomatosis Foundation	
Children's Cardiomyopathy Foundation	EURORDIS	Locks of Love		
Children's Craniofacial Association	Family Caregiver Alliance	Melosteosis Association		

Dedicated to Helping People with Orphan Diseases

In the real world, health insurance policies will not pay for investigational therapies. As written, the Proposed Rule will make investigational drugs available to wealthy people, and those who cannot pay will be omitted. Additionally, by definition investigational drugs are not yet proven safe or effective, but under the rule FDA will enable companies to charge for drugs that may have absolutely no benefit to patients, and may every pose safety problems.

We suggest that the agency separate the two types of sponsors: Academic investigators without commercial sponsors, and companies that are developing drugs or diagnostics for-profit. We agree that the cost of drug development is the cost of doing business in the commercial pharmaceutical world. We suggest that permission to charge for an experimental treatment should be tied to a requirement that a percentage of drugs will be provided at no cost to uninsured patients and patients whose insurer refuses to pay. Academic investigators should also be required to put aside a percentage of drugs that will be provided to patients who cannot afford to pay. If FDA does not assure access to an investigational treatment based on medical criteria – not financial ability to pay – only the wealthy will have access, and the poor will be barred from the system. For humane and scientific purposes, it would be a grave mistake to allow access to a limited class of patients (while others who are just a desperate are denied access).

Unfortunately, the FDA does not regulate the health insurance industry. Nevertheless, it is critically important for the agency to understand the reality of the insurance world, and the obstacles it presents to patients who wish to participate in clinical trials. Companies currently complain that they cannot recruit enough patients in clinical trials; but if they are allowed to charge patients, there will be even fewer volunteers. If only the wealthy can afford to participate in research, it could trigger a class struggle with enormous political repercussions.

The patient community wants fairness and equity in all expanded access programs. They don't want to know that an aunt of the company's CEO got the investigational drug, but their neighbor didn't. Allowing cost recovery for experimental treatments sets the stage for serious financial barriers that can be insurmountable to ordinary people, which will be added to other existing barriers such as distance from the research site, cost of travel, protocol inclusion and exclusion criteria, other medical and living costs, day care, and other responsibilities.

We hope FDA will adjust the proposed rule to require sponsors to provide free investigational drugs to at least 25 percent and as much as 50 percent of applicant patients who wish to participate in clinical trials, but cannot afford to do so. The more expensive a drug is, the less likely that patients will afford a drug.

Very truly yours,



Abbey S. Meyers
President

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