

# National Organization for Rare Disorders, Inc.®



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

## MEMBER ORGANIZATIONS

Alpha One Antitrypsin Deficiency National Association  
Alpha One Foundation  
ALS Association  
American Brain Tumor Association  
American Laryngeal Papilloma Foundation  
American Porphyria Foundation  
American Syringomyelia Alliance Project  
Aplastic Anemia Foundation of America  
Association for Glycogen Storage Disease  
Association of Gastrointestinal Motility Disorder, Inc.  
Batten Disease Support & Research Association  
Benign Essential Blepharospasm Research Foundation, Inc.  
Charcot-Marie-Tooth Association  
Chromosome 18 Registry and Research Society  
Cleft Palate Foundation  
Cornelia de Lange Syndrome Foundation, Inc.  
Cystinosis Foundation, Inc.  
Dysautonomia Foundation, Inc.  
Dystonia Medical Research Foundation  
Dystrophic Epidermolysis Bullosa Research Association (D.E.B.R.A.)  
Ehlers-Danlos National Foundation  
Epilepsy Foundation of America  
Families of Spinal Muscular Atrophy Foundation Fighting Blindness  
Foundation for Ichthyosis & Related Skin Types (F.I.R.S.T.)  
Genetic Alliance  
Guillain-Barre Syndrome Foundation International  
HHT Foundation International, Inc.  
Hemochromatosis Foundation, Inc.  
Hereditary Disease Foundation  
Histiocytosis Association of America  
Huntington's Disease Society of America, Inc.  
Immune Deficiency Foundation  
International Fibrodysplasia Ossificans Progressiva (FOP) Association, Inc.  
International Joseph Diseases Foundation, Inc.  
International Rett Syndrome Association  
Interstitial Cystitis Association of America, Inc.  
Lowe Syndrome Association  
Malignant Hyperthermia Association of the United States  
Mastocytosis Society  
Myasthenia Gravis Foundation  
Myeloproliferative Disease Research Center  
Myositis Association of America  
Mucopolidiosis Type IV Foundation (ML4)  
Narcolepsy Network, Inc.  
National Adrenal Diseases Foundation  
National Alopecia Areata Foundation  
National Ataxia Foundation  
National Foundation for Ectodermal Dysplasias  
National Hemophilia Foundation  
National Incontinentia Pigmenti Foundation  
National Marfan Foundation  
National Mucopolysaccharidoses Society, Inc.  
National Multiple Sclerosis Society  
National Neurofibromatosis Foundation  
National PKU News  
National Sjogren's Syndrome Association  
National Spasmodic Torticollis Association  
National Tay-Sachs & Allied Diseases Association, Inc.  
National Urea Cycle Disorders Foundation  
Neurofibromatosis, Inc.  
Osteogenesis Imperfecta Foundation  
Parkinson's Disease Foundation, Inc.  
Prader-Willi Syndrome Association  
Pulmonary Hypertension Association  
PXE International, Inc.  
Reflex Sympathetic Dystrophy Syndrome Association  
Scleroderma Foundation, Inc.  
Sickle Cell Disease Association of America, Inc.  
Sturge Weber Foundation  
The Paget Foundation  
The Steven Johnson Foundation  
Tourette Syndrome Association, Inc.  
Trigeminal Neuralgia Association  
United Leukodystrophy Foundation, Inc.  
United Mitochondrial Disease Foundation  
VHL Family Alliance  
Wegener's Granulomatosis Support Group, Inc.  
Williams Syndrome Association  
Wilson's Disease Association

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December 6, 2000

0491 '00 DEC 11 A9:59

Dockets Management Branch (HFA-305)  
Food & Drug Administration  
5630 Fishers Lane  
Room 1061  
Rockville, MD 20852

Re: Applications for FDA Approval to Market a New Drug; Proposed Revision of Postmarketing Reporting Requirements, 21 CFR Part 314, Docket No. 00N-1545

Dear Sirs:

In response to your *Federal Register* Notice of Proposed Rules, *Applications for FDA Approval to Market a New Drug; Proposed Revision of Postmarketing Reporting Requirements*, we submit the following comments about the Notice of Discontinuance:

## Background

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. In this regard, NORD advocates for the interests of the rare disease community to promote the development of new orphan drugs, and to assure continued access to existing therapies.

NORD advocated for the inclusion of the **Notice of Discontinuance** provision in the *FDA Modernization Act of 1997* because the orphan disease community felt very strongly that pharmaceutical companies should be obliged to notify the public when they are the only manufacturer of a drug, and they wish to discontinue the product. Prior to FDAMA, there was usually no notice given until a patient went to their drug store to pick up their prescription, and the pharmacist would inform the patient that the drug was no longer available. For example, too often old products with sales so small that there was no generic competition were discontinued, leaving patients with no other adequate treatment options for their disease. In several cases, these were old epilepsy drugs, and withdrawal from these medicines caused irreparable harm to patients.

## Associate Members

Acid Maltase Deficiency Association  
ALS Association/Greater Philadelphia Chapter  
American Autoimmune Related Diseases Association  
American Behcet's Disease Association, Inc.  
American Pseudo-obstruction & Hirschsprung's Disease Society, Inc.  
American Self-Help Clearinghouse  
Angel view Crippled Children's Foundation  
A-T Project

Ataxia Telangiectasia Children's Project  
CDGS Family Network  
Canadian Organization for Rare Disorders  
Children's Leukemia Foundation/Michigan Children's Living with Inherited Metabolic Diseases  
Children's Medical Library  
Children's PKU Network  
Chromosome Deletion Outreach, Inc.  
Chronic Granulomatous Disease Association, Inc.  
Consortium of Multiple Sclerosis Centers  
Contact A Family

Cooley's Anemia Foundation  
Cushing Support & Research Foundation  
Ear Goldberg Aplastic Anemia Foundation  
Family Caregiver Alliance  
Family Support System for North Carolina  
Freeman-Sheldon Parent Support Group  
Hydrocephalus Association  
International Foundation for Alternating Hemiplegia of Childhood  
Klippel-Trenaunay Support Group  
Late Onset Tay-Sachs Foundation  
Les Turner ALS Foundation, Inc.

National Association for Pseudoxanthoma Elasticum  
National Gaucher Foundation  
National Lymphedema Network  
National Niemann-Pick Disease Foundation  
National Patient Air Transport Helpline  
National Spasmodic Dysphonia Association  
Organic Acidemia Association  
Osteoporosis and Related Bone Diseases National Resource Center  
Parents Available to Help (PATH)  
Parent to Parent of Georgia, Inc.

Parent to Parent of New Zealand  
Rare and Expensive Disease Management Program  
Recurrent Respiratory Papillomatosis Foundation  
Restless Legs Syndrome Foundation  
Sarcoid Networking Association  
Shwachman Syndrome Support Group  
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  
Treacher Collins Foundation

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

00N-1545

Dedicated to Helping People with Orphan Diseases

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*NORD was convinced that this problem should not be allowed to continue.*

During the debate over *FDAMA of 1997*, the pharmaceutical industry strongly opposed the one-year notice of discontinuation provision, and only after protracted negotiations did we finally agree to the six-month notification even though we feared it would not allow sufficient time for physicians to wean their patients off one drug and start them on another (when another treatment existed).

We provide the above background material to you only because we would appreciate the agency's recognition that the **Notice of Discontinuance** was written for the benefit of patients, not pharmaceutical firms, yet we sense that the proposed regulatory revision may be slanted toward the concerns of the pharmaceutical industry, rather than patients.

## **II. Section 506C**

The FDA proposes many reasons why a manufacturer may seek a reduction in the six-month notification period, including a claim that continuation of manufacturing may cause, "substantial economic hardship for the manufacturer."

We urge you to put the onus on the manufacturer to prove that reduction of the six-month requirement **will not cause substantial physical and emotional harm to the patients** who rely on the drug for treatment of their serious and sometimes life-threatening condition. If the drug is not being withdrawn from the market for safety reasons, the agency should ask the manufacturer if any effort was made to find another company that may be willing to adopt the drug, and if not, why. In this way, the FDA may be able to work with a new manufacturer to transfer the product without any interruption of supply.

## **Section III B**

This section clearly says the agency will grant a reduction in the six-month notification for "good cause". We assert there should be very few instances of "good cause" that can outweigh the FDA's responsibility to patients who need a drug, but will not be able to get it after manufacturing ceases.

For example, in the case of a biomaterials shortage, we would urge the FDA to require companies to ration the remaining supply of a drug so that the three-month supply could be stretched to six months. Sometimes off-label uses of the drug could be stopped, or healthier patients might do without the drug while the sickest will continue to get it. In other words, the onus should always be on the manufacturer, and the agency should create the highest hurdles for waivers if the health and welfare of patients is at stake.

In fact, if a company has not filed for bankruptcy it is difficult to envision when "substantial economic hardship" could possibly be a reasonable excuse to reduce the six-month notice requirement. Physicians will need every minute of this time to find another treatment alternative. Indeed, the FDA should urge companies to voluntarily give one year's notice even though six months is all that is statutorily required. This is the humane and decent way of dealing with this issue, and many companies will be pleased to do the ethical thing if they understand the historical reasoning behind the discontinuation notice requirement.

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### Conclusion

We urge any pharmaceutical company intending to discontinue manufacture of a drug for a serious health condition to voluntarily provide more than six months notice to patients and physicians. We also encourage FDA, when it receives a notice of discontinuation, to urge companies to license these products to other pharmaceutical firms that would be satisfied with the small but loyal market.

We appreciate the opportunity to express our views about these important regulations.

Very truly yours,



Abbey S. Meyers  
President

ASM:aa

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



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into the light ...

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**CFC# 0551**



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