

# 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 Porphyrria 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  
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 Mucopolysaccharidose 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 Association  
Williams Syndrome Association  
Wilson's Disease Association

NORD • 100 Rt. 37, P.O. Box 8923 • New Fairfield, CT 06812-8923

Tel: (203) 746-6518 • FAX : (203) 746-6481

TDD (for hearing impaired) (203) 746-6927

<http://www.rarediseases.org> • e-mail: [orphan@rarediseases.org](mailto:orphan@rarediseases.org)

May 9, 2002

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

RE: **ANPRM Obtaining Timely Pediatric Studies of and Adequate Pediatric Labeling for Human Drugs and Biologics (Docket No. 02N-0152, 4-24-02)**

To Whom It May Concern:

The National Organization for Rare Disorders (NORD) firmly believes that FDA must continue to enforce its 1998 mandatory requirement for all new drugs that are expected to be used by children, specifying that they must be studied in the pediatric population before the drug is approved for marketing in the United States. We believe that pediatric exclusivity afforded under BPCA is not enough to ensure that all drugs used by children will be properly tested in children, and therefore the mandatory pediatric testing requirement for all new drugs is needed.

NORD is a non-profit voluntary health organization dedicated to the identification, treatment and cure of rare "orphan" diseases. Under the *Orphan Drug Act of 1983*, a rare disorder is defined as a health condition affecting fewer than 200,000 Americans. The National Institutes of Health (NIH) estimates there are approximately 6,000 of these "orphan diseases" cumulatively affecting more than 25 million Americans. Most rare diseases are genetic, and many affect children.

Except for a few prevalent health conditions such as common infections, asthma, allergies, and Attention Deficit Disorder, most other serious health conditions are rare in children. For example, cystic fibrosis affects 25,000 people in the United States (most of whom are children), Duchenne Muscular Dystrophy affects 10,000 boys, there are 200 different types of cancer and some of them affect children, Hemophilia affects only 15,000 males, etc.

Many serious health conditions in children are treated with drugs that were studied and approved for adults, but not for children. If sales of a drug are modest, companies do not want to invest in pediatric clinical trials because six months of added exclusivity for that drug will not earn enough profit. Thus financial incentives such as pediatric exclusivity, do not work unless there is reason to believe a drug is extraordinarily profitable. Since most serious diseases of childhood are rare, expanding the market of an existing drug to a small pediatric population is not a significant lone incentive.

**02N-0152**

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## Associate Members

Acid Maltase Deficiency Association  
ALS Association/Greater Philadelphia Chapter  
American Autoimmune Related Diseases Association  
American Behcet's Disease Association, Inc.  
American Self-Help Clearinghouse  
Ataxia Telangiectasia Children's Project  
CDGS Family Network  
Canadian Organization for Rare Disorders  
Children's Living with Inherited Metabolic Diseases

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  
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 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  
Parent to Parent of New Zealand Rare and Expensive Disease Management Program  
Recurrent Respiratory Papillomatosis Foundation  
Restless Legs Syndrome Foundation  
Saroid Networking Association  
Shwachman Syndrome Support Group  
Society For Progressive Supranuclear Palsy, Inc.  
Sotos Syndrome Support Association

Takayasu's Arteritis Association  
Taiwan Foundation for Rare Disorders

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

Dockets Management Branch (HFA-305)  
Food and Drug Administration  
Docket No. 02N-0152, 4-24-02  
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The *Best Pharmaceuticals for Children Act* (BPCA) offers six months of exclusivity to companies that study the use of a drug in children, but it does not require manufacturers to add pediatric information to the drug's label. This means in too many cases pediatricians do not have appropriate data for the treatment of children, even though a manufacturer is awarded the extra exclusivity. Additionally, companies tend to wait as long as possible before they perform pediatric studies, and such studies tend to be done only for the most profitable drugs rather than the medicines most needed by seriously ill children (e.g., lifesaving cancer drugs). If FDA requires all new drugs to be studied in children, and if the information is added to a drug's label before it is launched, the information that pediatricians need will reduce adverse events and save lives. If such testing is not mandatory for all new drugs, manufacturers will often wait until a patent is about to expire before they test the drug in children, and they may not agree to re-label the drug for pediatrics since they will gain exclusivity anyway.

For the purposes of BPCA, we believe FDA should consider biologics and antibiotics to be "drugs" because they have the same effect on the human body by controlling, reducing or curing disease symptoms. The critical question is whether FDA is empowered to add pediatric information to pharmaceutical labeling, even if the manufacturer objects. As far as we are concerned, BPCA is totally ineffective if it does not require pediatric labeling because children cannot benefit if pediatricians are unaware of appropriate dosing or side effects. We firmly believe FDA should mandate pediatric labeling when clinical trials in children are done. Pediatric exclusivity should not be awarded until the drug is properly labeled for use in children.

FDA's responsibilities are to enhance and protect the public health. Children are a critical part of the public. Their special medical needs must be met, including requiring necessary formulation changes for pediatric medicines. This will greatly reduce adverse events especially when children are treated with a drug or biologic that was developed for adults with a different diagnosis. Special formulations should be required when a specified drug is known to cause serious adverse events in children if the drug is prescribed in the wrong dosage.

Very truly yours,



Abbey S. Meyers  
President

ASM:aa

cc: Diane Dorman, Vice President for Public Policy (Via Fax)  
Senator Christopher Dodd, Att: Deb Barrett (Via Fax)



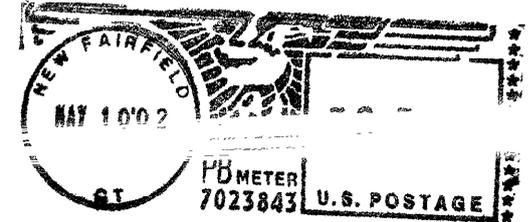
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**CFC# 0551**



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