National Organization for Rare Disorders, Inc.®

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

September 18, 2003

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

Dear Commissioner McClellan:

Re: June 11, 2003 Citizen Petition of the Abigail Alliance and Washington Legal Foundation

We are writing to urge you to reject the relief sought in the June 11, 2003 citizen petition from the Abigail Alliance and Washington Legal Foundation. The citizen petition proposes a new "Tier 1" drug approval program that would make experimental drugs in Phase I clinical trials available to terminally ill patients outside of clinical trials.

As you know, the National Organization for Rare Disorders (NORD) is a non-profit voluntary health organization dedicated to the identification, treatment and cure of rare "orphan" diseases. We have always been concerned about access to investigational treatments for people who do not qualify for clinical trials, and those who live too far away from clinical sites. Therefore, the *Orphan Drug Act of 1983* contains provisions for pre-approval access to promote availability of experimental therapeutics. A few years later, at the height of the AIDS epidemic, FDA Commissioner Frank Young issued "Treatment IND" regulations that formalized the pre-approval access process for all serious and life-threatening diseases without satisfactory therapeutic alternatives. Thus experimental treatments in Phase III trials are allowed to be given to desperately ill patients.

More recently FDA has allowed distribution of experimental drugs and biologics through a variety of other expanded access programs, even though the agency has not published regulations to structure the process. NORD has been privileged to administer the computerized random selection component of several of these programs so we are sensitive to the intense emotional issues that these families are dealing with. Ordinarily there will be a limited supply of the investigational drug with an unlimited number of patients who want it. The computerized random selection process (known commonly as a "lottery") is a structured incorruptible system to ensure that names will be selected fairly and all applicants will have an equal chance of being selected.

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
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.
Histiocytosis 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.
Mucolipidosis 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

Associate Member Organizations

Acid Maltase Deficiency Association (AMDA)
American Autoimmune Related Diseases Association
American Behcet's Disease Association
American Self-Help Group Clearinghouse
Amyotrophic Lateral Sclerosis (ALS) of Greater Philadelphia Chapter
Association for People with the Van Lebruzen Syndrome (CMTC)
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 (CGMB)
Consortium of Multiple Sclerosis Centers
Contact A Family
Cushing Support & Research Foundation, Inc.
CUTANEOUS
Family Caregiver Alliance
Family Support Network of North Carolina
Freemon Sheldon Parent Support Group
Hydrocephalus Association
Ingwiniorrha Pigment International Foundation
K-T Support Group
Late Onset Tay-Sachs Foundation
Lou Tumer ALS Foundation, Ltd
Mercy Medical Airlift
National Lymphedema Network, Inc
National Meniere-Pick Disease Foundation
National Spasmodic Dysphonia Association
Organic Acidaemia Association
Osteogenesis and Related Bone Diseases National Resource Center
Parent to Parent New Zealand, Inc.
Rare & Expensive Disease Management Program (REDM)
Reclaimed Respiratory Papillomatosis Foundation
Restless Legs Syndrome Foundation
Sarcoid Networking Association
Shwachman - Diamond Syndrome International
Society for Progressive Supranuclear Palsy, Inc.
Sotos Syndrome Support Association
Tuberous Sclerosis Association

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

Rev 6/03

Dedicated to Helping People with Orphan Diseases

We believe the proposed Tier 1 system from the Abigail Alliance is bad medicine. It proposes to give people with life threatening conditions access to experimental drugs that have no evidence of safety or efficacy. Phase I trials are aimed at toxicity, not efficacy, and their results do not prove that they have a clinical effect. It is estimated that 90 percent of treatments in Phase I clinical trials do not go on to further development because they are either unsafe or clearly ineffective. Thus every patient entering a "Tier I" program would have only a 10 percent chance of either not being harmed, or possibly benefiting. Moreover, it would greatly diminish the chances of fully enrolling Phase II or III trials because patients would likely opt for the chance of receiving active drug under Tier 1, over possible placebo in Phase II or III.

Additionally, we are gravely concerned that concepts such as "Tier 1" are adding to the problem of "therapeutic misconception." Some patients feel that experimental therapies are their "last chance", whereas there is actually no realistic evidence that most Phase I experimental drugs represent a reasonable treatment. History shows that virtually every major amendment to the *Food, Drug & Cosmetic Act* was precipitated by a major public health catastrophe that killed or maimed innocent people. If FDA adopts the proposed Tier 1 concept, it will virtually wipe out the *Kefauver Harris Amendments* that were precipitated by the Thalidomide tragedy. Pharmaceutical companies would be allowed to profit from sales of drugs that have not been proven safe or effective, thus reverting public health protections to the level of the 19th century.

We are concerned that some people feel they have a "right" to medical care of their choice, and this includes access to unapproved and unregulated therapies. However, in the United States health care is not a right, it is a privilege, and access to treatments that are unsafe or ineffective represents a clear threat to public health. This is why the FDA was created, and why Congress periodically strengthens the agency's regulatory powers.

We feel strongly that expanded access to investigational Phase III drugs (only after Phase II studies have shown convincing evidence of safety and efficacy) for patients with life-threatening and serious diseases that have no adequate alternatives should be conducted in a structured and equitable manner. Unapproved experimental drugs should not be sold, primarily because it would remove the incentive for manufacturers to seek FDA marketing approval. Additionally, only the richest patients would have access because insurance will not pay for experimental therapies. It would create a two-class system of medicine that would tear at the fabric of society, and it is morally unacceptable. We urge you to reject the Tier 1 concept and pledge to uphold the regulations that have made FDA's gold standard the marvel of modern medicine.

Very truly yours,

Abbey S. Meyers
President

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cc: Diane E. Dorman, NORD Vice President for Public Policy