

National Organization for Rare Disorders, Inc.®

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

NATIONAL MEMBER ORGANIZATIONS

Alagille Syndrome Alliance
Alpha 1 Association
Alpha 1 Foundation
American Brain Tumor Association
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
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.
Moebius Syndrome Foundation
Mucopolipidosis Type IV Foundation, Inc.
Myasthenia Gravis Foundation of America, Inc.
Myositis Association
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
Paget Foundation for Paget'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
Scleroderma Foundation
Stevens Johnson Syndrome Foundation
Sturge-Weber Foundation
The Erythromelalgia Association
The Oxalosis and Hyperoxaluria 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

June 13, 2005

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

RE: Drug Safety Oversight Board
Docket No. 2005D-0062

Dear Sir or Madam:

On May 4, 2005 the Director of the Center for Drug Evaluation and Research (CDER) issued a proposal regarding the Drug Safety Oversight Board (DSB) for the Manual of Policies and Procedures. Simultaneously a Draft Guidance was issued for FDA's Drug Watch for "Emerging Drug Safety Information." That document also contained questions and answers about the DSB.

We are submitting these comments about the proposed structure and operations of the DSB as outlined in the above documents.

NORD

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." A "rare disease" as defined in the *Orphan Drug Act of 1983*, affects fewer than 200,000 Americans. There are an estimated 6,000 rare disorders that collectively affect 25 million Americans.

NORD's programs include education, advocacy, research, and services to patients, families, and healthcare professionals. Safety of pharmaceuticals and biologics has always been an important issue to the orphan disease community, particularly because so many rare disorders are treated with off-label uses of drugs that were developed for prevalent diseases.

2005D-0062

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Associate Member Organizations

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

Children's Craniofacial Association
Children's PKU Network
Chromosome Deletion Outreach Inc.
Chronic Granulomatous Disease Association
CLIMB
Coalition for Pulmonary Fibrosis
Consortium of Multiple Sclerosis Centers
Contact A Family
Cushing Support & Research Foundation, Inc.
EURORDIS

Family Caregiver Alliance
Family Support Network of North Carolina
Freeman-Sheldon Parent Support Group
Gold, Global Organization for Lysosomal Diseases
Hydrocephalus Association
Incontinentia Pigmenti International Foundation
K-T Support Group
Les Turner ALS Foundation, Ltd.
Mercy Medical Airlift
National Lymphedema Network, Inc.

National Niemann-Pick Disease Foundation
National Organization for Albinism and Hypopigmentation.
NOAH
National Spasmodic Dysphonia Association
Organic Acidemia Association
Osteoporosis and Related Bone Diseases National Resource Center
Parent to Parent New Zealand, Inc.
Recurrent Respiratory Papillomatosis Foundation
Sarcoid Networking Association

Shwachman - Diamond Syndrome International
Society for Progressive Supranuclear Palsy, Inc.
Sotos Syndrome Support Association
Taiwan Foundation for Rare Disorders
Vestibular Disorders Association, VEDA

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

Rev 3/05

Dedicated to Helping People with Orphan Diseases

The Drug Safety Oversight Board

We applaud FDA for responding to the public's concern about drug safety, and for proposing to create a committee that will study safety issues about marketed drugs and post the information on the Internet. The public and healthcare providers are demanding access to this information so they can make informed decisions.

However, we are concerned about the proposed structure of the DSB as outlined in the Manual of Policies and Procedures and the Draft Guidance.

According to the announcement:

1. The DSB will be established to "provide independent oversight and advice" to the Director of CDER. However, the DSB will be composed entirely of government employees, most of whom work for the FDA. It will have no consumer representatives on the Board, no academic researchers, and no clinicians. Thus the public will have no seat and no vote on DSB decisions, and none of its' members will be practicing physicians who treat patients in their practice and know first-hand the consequences of adverse reactions.

The DSB will not be "independent" if it is structured as proposed in FDA's announcement. We suggest that an "independent" committee should not be composed entirely of government employees, and it should answer to the FDA Commissioner, not the Director of CDER.

2. As proposed, the DSB will report to the Director of CDER, whereas we believe it should report directly to the Commissioner. CDER makes marketing approval decisions. A DSB recommendation to withdraw a drug from the market, for example, may reflect negatively on CDER's policies or procedures. If FDA intends to make the DSB truly "independent," it must not be subservient to CDER, and it should be in the Commissioner's office where its' recommendations and decisions will be respected. If FDA insists DSB must report to CDER, it represents no change in current FDA policy, and there is no reason for DSB to exist.
3. The proposal allows the DSB to engage "consultants," including consumer and patient representatives. However, consultants will be non-voting members. It is beyond comprehension that FDA, which is trying to regain the public's trust, would not reserve seats on the DSB for patient and consumer representatives whose votes should be counted.
4. The proposal says that any organizational unit of CDER may refer drug safety issues to the DSB, but there is no provision for referrals from the public, healthcare professionals, NIH, CDC, academic scientists, professional societies, etc. Unfortunately this makes the DSB look like it will be an internal secretive committee that answers only to itself. The fact is, ordinary Americans are demanding more transparency at the FDA, and the DSB's proposed structure does nothing to ease the public's anxiety.

5. Decision-making authority is convoluted. The DSB will make recommendations to the CDER Director. This is exactly the way the current system is administered, and it has been a major factor in the erosion of public trust. We feel strongly that DSB's recommendations should go to the Commissioner, and the Commissioner should relay instructions to the Director of CDER.
6. DSB meetings will not be open to the public. This reinforces FDA's commitment to secrecy, which is one of the major reasons that the public is losing trust in the agency. Without transparency the public will continue to ask how decisions were made, and whether politics influenced decisions. Suspicions will be reinforced by the fact that DSB members will all be government employees, and FDA staff ordinarily does not answer questions from the public without a Freedom of Information (FOI) request. Corporations can file FOI requests, but it is unreasonable to expect ordinary people to do this.
7. The Board will meet on an "as needed" basis. Decisions about the need to meet will be made by busy government employees who may not want to take time out for meetings. We suggest that a more dependable and predictable schedule of meetings should be implemented.
8. The questions and answers document says that meetings and deliberations of the DSB will be classified as "confidential commercial information," and will not be disclosed to the public. This means that the public will not know if and when the DSB has held meetings and if they did meet, what they talked about. The public will not be able to determine whether decisions were influenced by FDA staff that may have been involved in the review or approval of the drug under discussion, or whether clinicians who are familiar with the drug participated in the discussion. Even if an FDA staff person is recused from voting on a drug they reviewed or approved, they could still influence the DSB's discussions about that drug. The only way to ensure that discussions are unbiased is to open them to the public (like Advisory Committee meetings).

In summary we feel that the DSB cannot be "independent" because of its proposed structure and composition; the public and healthcare providers will not be adequately represented; the FDA has done nothing to alleviate the public's criticism about the lack of transparency at the agency; and the CDER Director should not be the ultimate decision maker. The DSB should be in the Commissioner's office, and its' membership should be broadened so the public's and physician's interests are adequately represented on all topics that come to a vote.

The DSB proposal is a good start; but as it stands, it is not a solution to the public's concerns about drug safety. We urge you to reconsider the proposed composition of the Board, its' position at the agency, its' secrecy, its' structure, and its' independence.

Very truly yours,



Abbey S. Meyers
President

ASM:aa

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