

# National Organization for Rare Disorders, Inc.

NORD • 100 Rt. 37, P.O. Box 8023 • New Fairfield, CT 06812-1783 • (203) 746-6518



Telephone: 202-778-2306

May 19, 1993

- President:**  
Jess Thoene, M.D.
- Executive Director:**  
Abbey G. Meyers
- Member Organizations:**  
 Anorexia Nervosa Association  
 Alliance of Genealogical Support Groups  
 Alpha, Antisepsin Deficiency National Association  
 American Brain Tumor Association  
 American Cystic Fibrosis Foundation  
 American Neurology Association, Inc.  
 American Porphyria Foundation  
 American Society of Adults with Pseudo-  
 Croup, Inc. (ASAP)  
 American Syringomyelia Association Project  
 Aplastic Anemia Foundation of America  
 Association for Spinaean  
 Storage Disease  
 Autism Disease Support &  
 Research Association  
 Berlin Essential Spheroanemia  
 Research Foundation, Inc.  
 Charcot-Marie-Tooth Association  
 Chromosome 16 Registry and  
 Research Society  
 Cornelia de Lange Syndrome  
 Foundation, Inc.  
 Cystinosis Foundation, Inc.  
 Direct Link for the Disabled, Inc.  
 Dyslexia Foundation, Inc.  
 Dyslexia Medical Research Foundation  
 Dyslexic Spellers' Society  
 Research Assoc. (D.E.S.R.A.)  
 Ehlers-Danlos National Foundation  
 Epilepsy Foundation of America  
 Families of Spinae (FAS) Society  
 French Anemia Research Fund, Inc.  
 Foundation for Ichthyosis &  
 Related Skin Types (F.I.R.T.)  
 Guillain-Barre Syndrome  
 Foundation International  
 Hemochromatosis Foundation, Inc.  
 Hereditary Disease Foundation  
 Histiocytosis Association of America  
 Huntington's Disease Society  
 of America, Inc.  
 Immune Deficiency Foundation  
 International Fibrosarcoma Scleroma  
 Progressive (IFSP)  
 International Joseph Disease  
 Foundation, Inc.  
 International Pain Syndrome  
 Association  
 Interstitial Cystitis Association  
 of America, Inc.  
 Loua's Syndrome Association  
 Malignant Hypertension Association  
 of the United States  
 Myeloproliferative Disease Center  
 Neurology Network, Inc.  
 National Adrenal Disease Foundation  
 National Ataxia Association  
 National Association for  
 Sickle Cell Disease, Inc.  
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 Society, Inc.  
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 National Neuromuscular Foundation  
 National PKU Home  
 National Rasmussen Epilepsy  
 Foundation, Inc.  
 National Sjogren's Syndrome Assoc.  
 National Spasmodic Torticollis  
 Assoc. (NSTA)  
 National Tay-Sachs & Allied Disease  
 Association, Inc.  
 National Tuberculous Sclerosis  
 Association, Inc.  
 National Urea Cycle Disorders Fdn.  
 National Vitigo Research, Inc.  
 Neuroimmunology, Inc.  
 Obesive Compulsive Foundation  
 Oculopharyngeal Imperforate Foundation  
 Oculosis & Hypertonia Foundation  
 Paget's Disease Foundation, Inc.  
 Parents of Galactosemia Children  
 Parents' Disease Foundation, Inc.  
 PKU Foundation  
 Prader-Willi Syndrome Association  
 Reflex Sympathic Dystrophy  
 Syndrome Association  
 Scleroderma Foundation, Inc.  
 Scleroderma Into Change, Inc.  
 Sjogren's Syndrome Foundation, Inc.  
 The A.L.S. Association  
 The B.A.R. Foundation  
 The Neurologic Ovary Foundation  
 Tourette Syndrome Association  
 Tripharmal Hypertonia Association  
 United Leukodystrophy Foundation, Inc.  
 United Parkinson Foundation  
 United Patients' Association for  
 Pulmonary Hypertension, Inc.  
 Vestibular Disorders Association  
 Wegener's Granulomatosis Support  
 Group  
 Williams Syndrome Association  
 Wilson's Disease Association

TO: Carol Rasco  
 Assistant to the President for Domestic Policy

FROM: Glenda Booth *Glenda Booth*  
 Director of Policy

SUBJ: Thursday meeting on health care reform

Thank you for your willingness to meet with NORD on Thursday at 11:00 a.m.

Attending will be --

Dr. Jess Thoene, President  
 Abbey Meyers, Executive Director  
 Glenda Booth, Director of Policy

I am faxing a short briefing paper that outlines some of our concerns that we understand under consideration as the White House Task Force develops the President's health care reform proposal. We look forward to discussing these with you.

*Call Glenda - Tax credit for orphan drugs - why not included?*

*8 of 10 tax credit research info*

- Associate Members**
- |  |  |  |  |   |  |
|--|--|--|--|---|--|
| Alcald Syndrome Newsletter<br>Alcald's Society of Sleep Disorder<br>A.L.S. Association Greater<br>Philadelphia Chapter<br>American Scleroderma Association, Inc.<br>American Scleroderma Clearinghouse/<br>N.J.<br>ARC of Ohio<br>Association for Children with<br>Rett's/Silver Syndrome, Inc.<br>Center for Research in Sleep<br>Disorders | Charcot-Marie-Tooth International<br>Children's Leukemia Foundation/NIH<br>Christie Lazzari Foundation for<br>Juvenile Laryngeal Papillomatosis<br>Chronic Granulomatous Disease<br>Association<br>Congenital Adrenal Hyperplasia<br>Assoc. Inc. (CAHA)<br>Devereaux Foundation<br>Fogarty Institute<br>Foundation for Nerve & Mitochondria<br>Syndromes | Freeman-Sheldon Parent Support<br>Group<br>Help Hospitalized Children's Fund<br>Kappa-Triumph Support<br>Group<br>Lathrop Society for Rare<br>Disorders/Grants<br>Lyme Borne Disease Foundation<br>National Association For<br>Pseudo-Tetanus Epidemic | National Coalition for Research in<br>Neurological & Communicative<br>Disorders<br>National Cushing's Association<br>National Ear-Hop Clearinghouse<br>(NHAC)<br>North American Pediatric<br>Pseudo-Obstruction Society<br>Organic Acidemia Association, Inc.<br>Parent to Parent of GA, Inc.<br>Parent to Parent of New Zealand | Port Agemi Association for Children<br>with Developmental Disabilities<br>Research Trust for Metabolic<br>Diseases in Children/England<br>Rocky Mountain Resource & Training<br>Institute<br>Shy-Drager Syndrome Support<br>Group<br>Sickle Cell Association of the<br>Texas Gulf Coast<br>Sickle Syndrome Support Group<br>Sturge-Weber Foundation | Tourette Syndrome Assoc. of MD<br>Tourette Syndrome Assoc. of<br>New York<br>Tourette Syndrome Assoc. of OH<br>Tourette/Compulsions Foundation<br>Tourette Syndrome Assoc. of IL<br>Valencia les Malades<br>Lysocholine France |
|--|--|--|--|---|--|

Dedicated to Helping People with Orphan Diseases

President:  
Jess Thoene, M.D.

Executive Director:  
Abbey S. Meyers

Member Organizations:  
Acoustic Neuroma Association  
Alliance of Genetic Support Groups  
Alpha-1 Antitrypsin Deficiency National Association  
American Brain Tumor Association  
American Carpal Tunnel Syndrome Association  
American Narcolepsy Association, Inc.  
American Porphyria Foundation  
American Society of Adults with Pseudo-Obstruction, Inc. (ASAP)  
American Syringomyelia Alliance Project  
Aplastic Anemia Foundation of America  
Association for Glycogen Storage Diseases  
Batten Disease Support & Research Association  
Benign Essential Blepharospasm Research Foundation, Inc.  
Charcot-Marie-Tooth Association  
Chromosome 18 Registry and Research Society  
Cornelia de Lange Syndrome Foundation, Inc.  
Cystinosis Foundation, Inc.  
Direct Link for the Disabled, Inc.  
Dysautonomia Foundation, Inc.  
Dystonia Medical Research Foundation  
Dystrophic Epidermolysis Bullosa Research Assoc. (D.E.B.R.A.)  
Ehlers-Danlos National Foundation  
Epilepsy Foundation of America  
Families of Spinal Muscular Atrophy  
Fanconi Anemia Research Fund, Inc.  
Foundation for Ichthyosis & Related Skin Types (F.I.R.S.T.)  
Guillain-Barré Syndrome Foundation  
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)  
International Joseph Diseases Foundation, Inc.  
International Rett Syndrome Association  
Interstitial Cystitis Association of America, Inc.  
Lowe's Syndrome Association  
Malignant Hyperthermia Association of the United States  
Myeloproliferative Disease Center  
Narcolepsy Network, Inc.  
National Adrenal Diseases Foundation  
National Alopecia Areata Foundation  
National Association for Sickle Cell Disease, Inc.  
National Ataxia Foundation  
National Chronic Fatigue Syndrome Association, Inc.  
National Foundation for Ectodermal Dysplasias  
National Fragile X Foundation  
National Gaucher Foundation  
National Leishmaniasis Foundation  
National Marfan Foundation  
National Mucopolysaccharidoses Society, Inc.  
National Multiple Sclerosis Society  
National Neurofibromatosis Foundation  
National PKU News  
National Retinitis Pigmentosa Foundation, Inc.  
National Sjogren's Syndrome Assoc.  
National Spasmodic Torticollis Assoc. (NSTA)  
National Tay-Sachs & Allied Diseases Association, Inc.  
National Tuberous Sclerosis Association, Inc.  
National Urea Cycle Disorders Fdn.  
National Vitiligo Foundation, Inc.  
Neurofibromatosis, Inc.  
Obsessive Compulsive Foundation  
Osteogenesis Imperfecta Foundation  
Oxalosis & Hypoxaluria Foundation  
Paget's Disease Foundation, Inc.  
Parents of Galactosemic Children  
Parkinson's Disease Foundation, Inc.  
PKR Foundation  
Prader-Willi Syndrome Association  
Reflex Sympathetic Dystrophy Syndrome Association  
Scleroderma Federation, Inc.  
Scleroderma Info Exchange, Inc.  
Sjogren's Syndrome Foundation, Inc.  
The A.L.S. Association  
The EAR Foundation  
The Myasthenia Gravis Foundation  
Tourette Syndrome Association  
Trigeminal Neuralgia Association  
United Leukodystrophy Foundation, Inc.  
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Vestibular Disorders Association  
Wegener's Granulomatosis Support Group  
Williams Syndrome Association  
Wilson's Disease Association

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out of the darkness,  
into the light.

May 27, 1993

Ms. Carol Rasco  
Assistant to the President for Domestic Policy  
The White House  
Washington, DC 20500

Dear Ms. Rasco:

I want to sincerely thank you for meeting with me, Dr. Jess Thoene and Glenda Booth last week. Our discussion about the concerns of people with rare "orphan diseases" was truly encouraging because we know that you can personally identify with the fear and pain that families face every time they have to deal with the inequities of our health care system. The members of NORD (127 national voluntary health agencies and 50,000 individuals with rare disorders and their families) are very hopeful that President Clinton can restructure our health care system and guarantee every American access to affordable medical care.

We applaud the President for his willingness to address many long-neglected health care needs, and we look forward to working closely with you to enact a proposal that is truly comprehensive and universal. You and I as parents of children with disabilities know that our legacy to future generations must be a health care system that truly provides access by the ill and disabled, and restores people like Cathy McClanahan to a productive life.

The unique problems of people with rare disorders are only different because they are complicated by medical ignorance, isolation and the absence of adequate treatments. Every person with a disability relies on hope - hope that a treatment or cure will be discovered, or that a technological breakthrough will enable them to lead a more normal life (e.g., talking computers, or portable respirators instead of iron lungs). But people with orphan diseases fear that scientists are not studying their disorder, and that drug companies will not develop treatments for them. Ultimately they fear loss of their health insurance, and even when a treatment is developed, families can only hope that they can afford it.

#### Associate Members

Aicardi Syndrome Newsletter  
Alabama Society for Sleep Disorders  
A.L.S. Association Greater Philadelphia Chapter  
American Behçet's Association, Inc.  
American Self-Help Clearinghouse/  
N.J.  
ARC of Ohio  
Association for Children with  
Russell-Silver Syndrome, Inc.  
Center for Research in Sleep  
Disorders

Charcot-Marie-Tooth International  
Children's Leukemia Foundation/Al  
Christina Lazar Foundation for  
Juvenile Laryngeal Papillomatosis  
Chronic Granulomatous Disease  
Association  
Congenital Adrenal Hyperplasia  
Assoc., Inc. (CAHSA)  
Devereux Foundation  
Footstep Institute  
Foundation for Nager & Miller  
Syndromes

Freeman-Sheldon Parent Support  
Group  
Help Hospitalized Children's Fund  
Klippel-Trenaunay Support  
Group  
Lethbridge Society for Rare  
Disorders/Canada  
Lyme Borreliosis Foundation  
National Association For  
Pseudoanthraxoma Elasticum

National Coalition for Research in  
Neurological & Communicative  
Disorders  
National Cushings Association  
National Self-Help Clearinghouse  
(Israel)  
North American Pediatric  
Pseudo-Obstruction Society  
Organic Acidemia Association, Inc.  
Parent to Parent of GA, Inc.  
Parent to Parent of New Zealand

Port Albeni Association for Children  
with Development Disabilities  
Research Trust for Metabolic  
Diseases in Children/England  
Rocky Mountain Resource & Training  
Institute  
Shy-Drager Syndrome Support  
Group  
Sickle Cell Association of the  
Texas Gulf Coast  
Soto's Syndrome Support Group  
Sturge-Weber Foundation

Tourette Syndrome Assoc. of MD  
Tourette Syndrome Assoc. of  
Nova Scotia  
Tourette Syndrome Assoc. of OH  
Treacher-Collins Foundation  
Tuberous Sclerosis Assoc. of IL  
Valence les Maladies  
Lysosomales/France

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

Dedicated to Helping People with Orphan Diseases

Ms. Carol Rasco  
May 27, 1993  
Page Two

As Mrs. Clinton said so eloquently, we cannot stand alone among all industrialized nations of the world and rationalize why we cannot guarantee health care to all of our citizens. We at NORD want to help you end this injustice.

Thank you again for your precious time. We hope you will stay in close touch with us during this time of historic changes. We pray that President Clinton will have the courage and stamina to face the massive health care struggle that lies ahead.

Very truly yours,



Abbey S. Meyers  
Executive Director

ASM:aa

cc: Jess Thoene, M.D.  
Glenda Booth  
Cathy McClanahan

## NORD MEMBER ORGANIZATIONS

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Jess Thoene, M.D.

### Executive Director:

Abbey S. Meyers

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Paget's Disease Foundation, Inc.

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Parkinson's Disease Foundation, Inc.  
PKR Foundation  
Prader-Willi Syndrome Association  
Reflex Sympathetic Dystrophy Syndrome Association  
Scleroderma Federation, Inc.  
Scleroderma Info Exchange, Inc.  
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The A.L.S. Association  
The American Brain Tumor Association  
The EAR Foundation  
The Myasthenia Gravis Foundation  
Tourette Syndrome Association  
Trigeminal Neuralgia Association  
T.S.A. of Illinois  
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Tourette Syndrome Assoc. of OH  
Treacher-Collins Foundation  
Vaincre les Maladies Lysosomales/France

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

## NORD'S RESEARCH PROGRAM

NORD's Research Program is governed by its **Research Advisory Council (NRAC)** on the recommendations of **NORD's Medical Advisory Board (MAB)**. The scientific grant program was initiated in 1988 through a request for proposals. These grants provide funding to scientists performing research on new **treatments** for rare disorders. NORD will not fund grants for basic research unless it is directly related to a new **treatment** for a rare disease or condition.

All of NORD's programs must develop and flourish simultaneously because one cannot be effective without the other. For example, NORD may fund a research study on a disease that affects only a handful of people in the United States. The scientist may require the participation of 25 people with that disease for the research project. Through NORD's **education** and **networking** programs, NORD must attempt to locate patients needed for the study and encourage them to contact the researcher. Each individual can then make a decision as to whether they wish to participate in the research project. Additionally, NORD attempts to relay information about important scientific studies to the medical community so that practicing physicians can refer their rare disease patients to the research scientist. Thus, adequate resources are required to carry out NORD's mission through all of its vital programs.

Your donation to NORD will provide help by expanding medical research, fostering education and ensuring that the voices of all people with rare disorders are heard in unison before all levels of the government, health related industries, and the scientific community.

# NORD



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

# NATIONAL ORGANIZATION FOR RARE DISORDERS

NORD

100 Rt. 37, P.O. Box 8923,  
New Fairfield, CT 06812-1783  
(203) 746-6518

**Not so rare. . .**

"Our son was misdiagnosed for more than two years. You cannot imagine the nightmare we lived through. The professionals we saw could not identify his illness. We spent huge sums of money searching for an answer. When his disorder was finally diagnosed, we learned that it was so rare, physicians knew little about it. There seems to be no treatment, and very little research giving us hope for his future."

Although this parent thought her story was unique, millions of Americans with rare disorders have suffered similar agonies. Names of their illnesses may be different, but most have experienced the indignity of searching for help in vain. Rare disorders are "back of the textbook" illnesses, unfamiliar to the general public and professionals alike. They can strike people of all ages, all races, and all ethnic backgrounds. Many are genetic; others are acquired through environmental causes; but for most, the cause is still unknown. Thus, even after a family obtains a proper diagnosis, they are too often left with more unanswerable questions.

**NORD created to help**

The National Organization for Rare Disorders (NORD) has been created by a group of voluntary agencies, medical researchers and individuals concerned about Orphan Diseases and Orphan Drugs. Orphan Diseases are rare, debilitating illnesses which strike small numbers of people. Orphan Drugs are therapies which alleviate symptoms of some rare diseases, but which have not been developed by the pharmaceutical industry because they are unprofitable.

Any disorder affecting fewer than 200,000 people is an "Orphan Disease" because products developed for these illnesses are considered by the pharmaceutical industry as "drugs of little commercial value." The cost of developing a drug in the U.S. today ranges between \$50 million and \$80 million. To provide incentives for commercial development of Orphan Drugs, Congress enacted the "Orphan Drug Act," which became a law on January 4, 1983.

**It's just the beginning. . .**

This legislation has substantially impacted upon the Orphan Drug problem by offering tax incentives to drug manufacturers who develop Orphan Drugs. In addition, the Act gives seven years exclusive marketing rights to developers of unpatentable therapies. A small pool of money is authorized by the legislation for grants to scientific investigators for research on new treatments for rare disorders.

Passage of the Orphan Drug Act, however, does not signify the end of the struggle for people with rare disorders; rather, it represents only the end of the beginning. Recognizing that more than 5,000 rare disorders affect more than 20 million Americans, NORD addresses their common concerns; people with Orphan Diseases do not suffer less pain and their families do not endure less agony simply because small numbers are affected by these illnesses.

**NORD's objectives are:**

- To encourage, promote and fund scientific research on the cause, control and ultimate cure of rare disorders.
- To educate the general public and medical profession about the existence, diagnosis and treatment of rare disorders.
- To act as a clearinghouse for information about rare disorders and to network families with similar disorders together for mutual support.
- To foster communication among rare disease voluntary agencies, government bodies, industry, scientific researchers, academic institutions and concerned individuals.
- To accumulate and disseminate information about Orphan Drugs and Devices, making known their availability to patients, physicians and other concerned parties.

- To assist in harmonizing and making more efficient the work of voluntary agencies and to offer technical assistance to newly organized support groups.
- To focus the attention of government, industry and the scientific community on the needs of people with rare disorders.

**Your help is needed. . .**

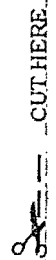
NORD's newsletter, *ORPHAN DISEASE UPDATE*, reports about progress in research on rare disorders; recent activities by government, health related industries and the scientific community; and relates personal accounts of courageous struggles by people with orphan diseases throughout the world.

NORD is dependent upon your support to carry on its vital activities, which have not been addressed by any other agency. Even if you do not suffer with a rare disorder, chances are a relative, friend or neighbor does. **NO ONE IS IMMUNE FROM BEING STRICKEN BY AN ORPHAN DISEASE.**

Your donation provides medical **research**, fostering **education** of the public and medical professionals so that people with rare diseases will be more readily recognized and helped, and expanding NORD's "**Networking**" program so that families with orphan diseases can be linked together with others having the same health condition.

Please help as your donation does make a difference in helping us overcome these little known painful, debilitating and, in some cases, life threatening disorders. Your support of NORD today may make a difference to someone you love tomorrow.

**National Organization for Rare Disorders**  
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(203) 746-6518



CUT HERE

**Associate Membership**

- Basic Individual or Family (\$ 25)
- Friend (\$ 50)
- Contributing (\$100)
- Donor (\$ )
- I cannot afford dues at this time but please accept my contribution of (\$ )

**Check appropriate membership category**

Check or Money Order Enclosed

Charge my  MasterCard  Visa (Minimum charge is \$10.00, please.)

Card Number \_\_\_\_\_ Exp. Date \_\_\_\_\_

Name on Card \_\_\_\_\_

Signature \_\_\_\_\_ Today's Date \_\_\_\_\_

Daytime Phone No. \_\_\_\_\_

Please enclose membership dues and donation and return to: National Organization for Rare Disorders  
NORD • 100 Rt. 37, P.O. Box 8923, • New Fairfield, CT 06812-1783 • (203) 746-6518

Have you contacted NORD before?  Yes  No

Name \_\_\_\_\_

Address \_\_\_\_\_

City \_\_\_\_\_

State \_\_\_\_\_ Zip \_\_\_\_\_ Phone ( \_\_\_\_\_ ) \_\_\_\_\_

My relationship to the person with the rare disorder is:

(i.e., patient, parent, etc.)

# **Genetic Information and Health Insurance**

**Report of the Task Force on Genetic Information and Insurance**

**NIH/DOE Working Group on Ethical, Legal, and Social Implications  
of Human Genome Research**

**Pre-Publication Copy**

**May 10, 1993**

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May 19, 1993

Concerns of the National Organization for Rare Disorders  
Health Care Access and Cost Control Reform

Prepared by Dr. Jess Thoene, President  
Abbey Meyers, Executive Director  
Glenda Booth, Director of Policy

Telephone: 202-778-2306

NORD

The National Organization for Rare Disorders is composed of 127 organizations representing over 5,000 known rare or "orphan" diseases which affect an estimated 20 million Americans. A voluntary, nonprofit organization, NORD is dedicated to the prevention, treatment and cure of rare diseases and the welfare of people afflicted by these illnesses. Since its inception in 1983, NORD has served as the primary non-governmental source of information and referral on rare diseases for those seeking help.

1. ACCESS TO APPROPRIATE CARE

■ Practice Guidelines or Parameters: The growing use of practice guidelines or parameters has serious consequences for people with rare disorders.

Practice guidelines and normative practice standards have not been established for most rare diseases due to the few patients available and the difficulty in providing a patient base adequate for a meaningful clinical trial. Many patients are treated according to the clinical experience of specialist physicians in academic medical centers who have personally had contact with relatively large numbers of persons with specific rare disorders. In addition, patients with rare disorders frequently require highly individualized care, including, for example, specialized diets, medications and appliances.

Recommendation:

Coverage plans must recognize that practice guidelines may be nonexistent or inappropriate for persons with rare disorders and must include easily implemented exceptions for reimbursement.

■ Reimbursement for Use of Specialists: Because symptoms of rare diseases are often vague and confusing and because most physicians are unfamiliar with rare diseases, accurate diagnosis can take years. The National Commission on Orphan Diseases found that 31 percent of rare disease patients took one to five years



to receive a correct diagnosis and 15 percent took more than six years. Once diagnosed, a patient faces many obstacles to getting appropriate treatment. Generally, there are few physicians knowledgeable about rare diseases. These physicians, who are usually dispersed all over the country in university medical centers, are often the only source of appropriate care.

### Recommendation

Benefit and reimbursement policies must recognize the need for recognized specialists to provide care for persons with rare diseases. Coverage plans should be able to negotiate reasonable prices or develop reciprocal agreements with distant providers when qualified specialists are not locally available.

Failing to provide coverage or reimbursement for treatment by an appropriate provider will result in inappropriate care and deterioration of health, which creates unnecessary costs.

### ■ Reimbursement for "Off-label" Drug Therapies

Adequate prescription drug benefits are critical for people with rare disorders. Current problems take three forms.

First, many rare diseases are chronic; thus, drugs to treat them must often be taken for a lifetime. Current commercial and public coverage plans usually provide limited coverage and have high deductibles and copayments.

Second, physicians treating persons with rare disorders often use "off-label" drug therapies, therapies considered by insurers to be experimental and thus, not medically appropriate or medically reasonable, the traditional standards for coverage.

In addition, drug companies are not willing to invest time and money in getting Food and Drug Administration approval for "off-label" uses.

"Off-label" drug therapies are frequently the only effective treatment for many rare disorder patients, enabling them to lead productive lives. This is often their only source of hope.

Third, drug prices, particularly for persons with rare disorders are often exorbitant. For example, Ceredase, used to treat Gaucher's Disease, costs \$350,000 per year for adults and must be taken for a lifetime. Human Growth Hormone, for treating Pituitary Dwarfism, costs \$30,000 per year. Drug prices overall have been rising more than twice as fast as the Consumer Price Index for a decade.

Recommendation:

Coverage plans should recognize and reimburse for "off-label" treatments. Many recognized experts are available to help develop standards. All normative standards should be carefully developed to recognize the broad diversity of diseases and conditions which can affect all Americans.

Drug benefits must be comprehensive enough to provide persons with rare disorders enough care to help them live functional lives. Modest deductibles and copayments are reasonable.

Pharmaceutical prices must be controlled.

2. MEDICAL PRIVACY

As advances in human genetic research accelerate, there will be a vast increase in the amount and kind of genetic information available for and about individuals. Much of this information will be useful in predicting, preventing and treating rare diseases.

Traditional commercial health insurance policies have increasingly relied on the selection and rating of risk (underwriting) and have used information from an individual's medical history to selectively insure individuals and price policies. With potentially more genetic information available, insurers, thus, may have access to more predictive information on individuals and use it in rating the risk for insuring individuals and their families.

Recommendation:

We welcome press reports that the Administration's health care reform plan will prohibit experience rating by coverage plans and will prohibit plans from conditioning insurance on "pre-existing conditions."

Health care reform must guarantee that medical records are kept confidential, that patient privacy is protected. Providers must be required to protect patients' privacy and patients must not be compelled to provide medical histories for purposes of health insurance. The privacy of medical records has broad social implications beyond health care.

3. ORPHAN DRUG ACT REAUTHORIZATION

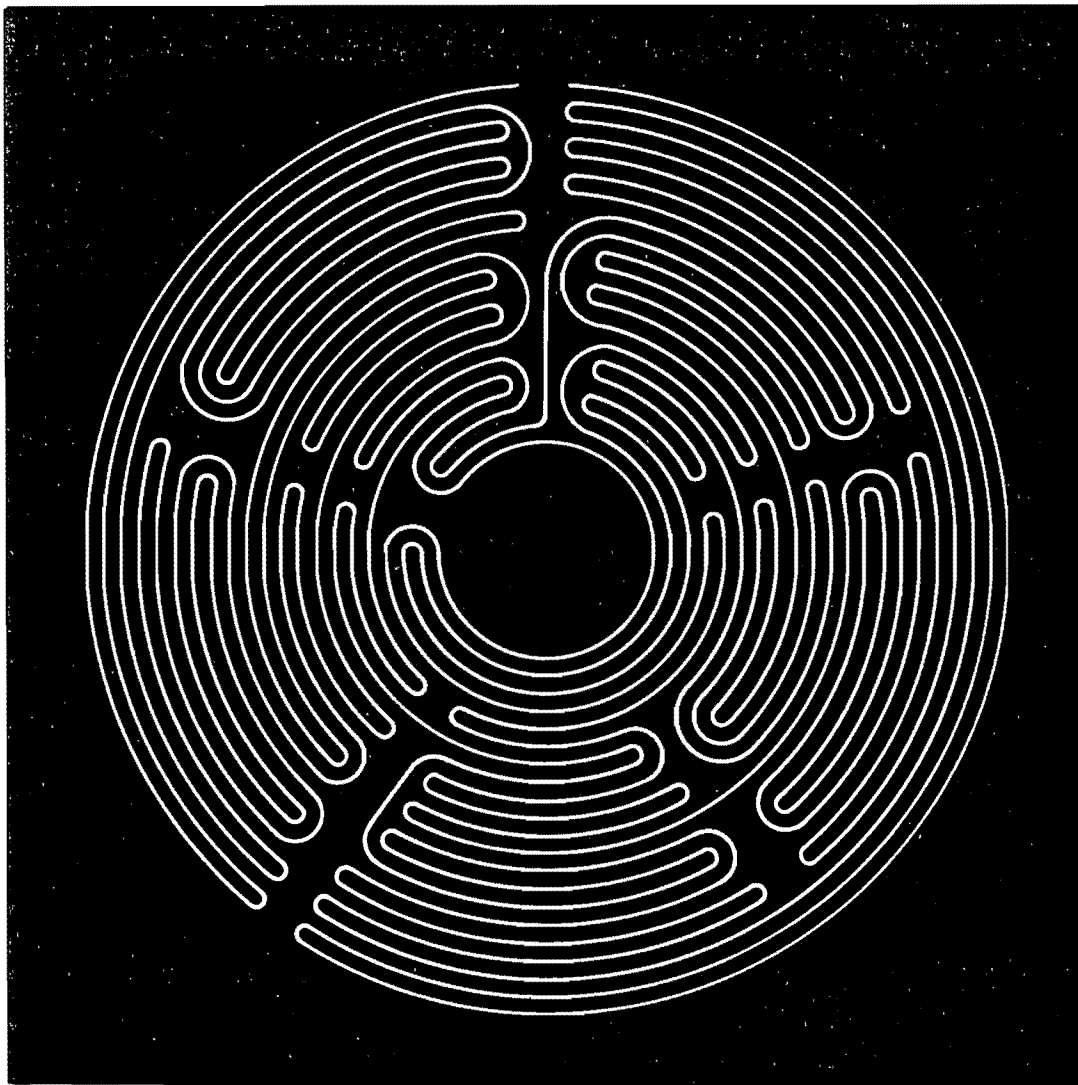
Pharmaceutical companies have hesitated to make drugs for treating rare diseases because markets for these drugs are limited. The Orphan Drug Act of 1983 (P. L. 97-414) includes three major approaches designed to encourage manufacturers to

develop orphan drugs: market exclusivity for 7 years; a research and development tax credit; and a grant program administered by the Food and Drug Administration. The tax credit expired in June 1992 and the grant program's funding authorization terminated in 1990. Former President Bush vetoed amendments in 1990 and bills introduced in the 102nd Congress did not become law.

Recommendation: The Orphan Drug Act should be amended to address excessive profits never intended by its originators along the lines of the legislation introduced in the last Congress. Under these bills, if drug sales exceed \$200 million in cumulative sales before expiration of the seven-year period of exclusivity, the exclusivity would end. The tax credit and the grant program should be extended.

# REPORT OF THE NATIONAL COMMISSION ON ORPHAN DISEASES

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U.S. DEPARTMENT OF HEALTH AND HUMAN SERVICES  
Public Health Service  
Office of the Assistant Secretary for Health  
February 1989

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MEMORANDUM OF CALL

2/13/44  
Glenda Booth  
Dir - Policy  
Previous editions usable

TO: CR 4/11/44 E.A. DITZ  
A. Dea Meyers

YOU WERE CALLED BY: 8/4/44 J. W. WELLS  
Kathryn H. Clavahan (Nana)

OF (Organization): Nat'l. Org. for Rare Diseases

PLEASE PHONE: 501-345-6909  
 FTS: 601-400-1000

WILL CALL AGAIN  IS WAITING TO SEE YOU

RETURNED YOUR CALL  WISHES AN APPOINTMENT

MESSAGE

(4) [Redacted] P6/b(6)

Glenda Booth  
Christene  
Dr. Ruzlaster

[Redacted] P6/b(6)

RECEIVED BY: PR DATE: 5/14 TIME: 10:10

THE WHITE HOUSE  
WASHINGTON

P6/b(6)

P6/b(6)

Glenda Booth, Director of Policy  
Abbey Meyers, Exec. Dir  
Dr. Jess Thoene (Taney), President