

InterOffice Memo

To: Carol Rasco
From: Christine Heenan
Date: November 10, 1993
Subject: National Organization for Rare Disorders letter

*orig - memo only Heenan
xc: Rasco*

In researching the appropriate response to the NORD request you received, I spoke to Chris Jennings, who was very involved in drug pricing issues when he worked with Senator Pryor and the Senate Aging Committee. Based on his input, this may be a good bill for the Administration to support.

Unless she needs packet back

Background

The group represents individuals with, (and parents of children with), rare diseases. They are a small constituency, and are the population most sensitive to the importance of continued investments in research by the drug companies.

During the 1980s, when drug pricing first came under scrutiny, this group was aligned with the drug companies in their argument that curbed pricing would cause them to cut back on research and development. However, when prices continued to go up and up with no proportional increase in research, they began to question the industry line.

Current Positioning

They now support pharmaceutical cost containment, including strong support of the Breakthrough Drug Committee in the President's Health Security Act. (see attached letter). They are very supportive of the President's proposal overall and will be on our side when the drug companies try to make the argument that cost containment will put their research efforts at risk. They will be there for us on the health care bill, and feel they need us behind them on the orphan drug legislation.

The Orphan Drug Amendment Legislation

Chris believes this is a fair, balanced piece of bi-partisan legislation, with broad-based support in the Senate as well as support on the House side from key Democrats.

It may be that we want to provide NORD with a letter of support for their press conference.

One thing we could do immediately is send back a letter from NORD indicating our interest in this issue, since pharmaceutical cost containment is so important to health reform, and saying we have taken their suggestion to meet with Bill Corr, Deputy Assistant Secretary for Health, to better understand the specifics of orphan drug policy. I could set that meeting up with you, or if you want I could meet with him and write you a memo on the bill and the issue so that you have it as background for your discussions with OMB and Howard Paster.

Please have the meeting & do memo thanks

National Organization for Rare Disorders, Inc.

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



October 14, 1993

Mrs. Hillary Rodham Clinton
The White House
Old Executive Office Bldg.
Washington, DC 20503

Dear Mrs. Clinton:

The National Organization for Rare Disorders (NORD) strongly supports the Breakthrough Drug Committee and other pharmaceutical cost control measures outlined in the President's health reform proposal.

We are certain that health reform is doomed to failure unless there are stringent measures to keep price hikes in line with inflation, particularly prescription drug prices. In addition, the nation sorely needs a mechanism for holding down the prices of new drugs that reach the market.

NORD represents approximately twenty million Americans who suffer from more than 5,000 rare "orphan diseases." Each of these ailments afflicts fewer than 200,000 Americans. Most of these disorders are serious, debilitating, chronic and some are fatal. The populations we represent often have great difficulty obtaining or retaining health insurance, and treatment very often requires expensive pharmaceuticals that must be taken throughout life.

For this reason, NORD has been an outspoken critic of the outrageous increases in pharmaceutical prices over the last decade. The following paragraph from the article, "Do We Pay Too Much for Prescriptions," in the October issue of Consumer Reports, is ample testament to the industry's single-minded and insensitive pursuit of profits:

"Last year the pharmaceutical industry had, as usual, the highest return on sales, the highest return on assets, and the highest return on stockholder equity of any industrial group in the Fortune 500. Its 11.5 percent return on sales was more than four times as high as the average of all Fortune 500 companies -- and almost twice that of the second-most profitable industry. Eight drug companies were among the 25 companies with the biggest absolute profits."

- President:
Alex Thornley, M.D.
- Executive Director:
Adele S. Meyers
- Member Organizations:
- Alzheimer's Association
 - Alliance of Genetic Support Groups
 - Alpha-1 Antitrypsin Deficiency National Association
 - American Brain Tumor Association
 - American Cardiac Tuning Syndrome Association
 - American Narcosepsy Association, Inc.
 - American Porphyria Foundation
 - American Society of Adults with Pseudo Obstruction, Inc. (ASAP)
 - American Symplocyria Alliance Project
 - Asplenic Anemia Foundation of America
 - Association for Glycogen Storage Disease
 - Batten Disease Support & Research Association
 - Bergin Essential Biopharmaceutical Research Foundation, Inc.
 - Charcot-Marie-Tooth Association
 - Chromosome 18 Registry and Research Society
 - Cornelia de Lange Syndrome Foundation, Inc.
 - Cystinosis Foundation, Inc.
 - Deaf Unit for the Deafblind, 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
 - Familial Short Stature Association
 - Fanconi Anemia Research Fund, Inc.
 - Foundation for Ichthyosis & Related Skin Types (F.I.R.S.T.)
 - Gullian-Barre Syndrome Foundation International
 - Hemochromatosis Foundation, Inc.
 - Hemophilia Disease Foundation
 - Histiocytosis Association of America
 - Huntington's Disease Society of America, Inc.
 - Immune Deficiency Foundation
 - International Porphyria Society
 - International Progressive IOP
 - International Juvenile Diseases Foundation, Inc.
 - International Ret Syndrome Association
 - Interstitial Cystitis Association of America, Inc.
 - Loew's Syndrome Association
 - Malignant Hyperthermia Association of the United States
 - Metachromic Purple Disease Center
 - Neuroblastoma Network, Inc.
 - National Adrenal Disease Foundation
 - National Adipose Anemia Foundation
 - National Association for Sickle Cell Disease, Inc.
 - National Ataxia Foundation
 - National Chronic Fatigue Syndrome Association, Inc.
 - National Foundation for Epidermolysis Bullosa
 - National Fragile X Foundation
 - National Gaucher Foundation
 - National Lipid Disease Foundation
 - National Marfan Foundation
 - National Mucopolysaccharidosis Society, Inc.
 - National Multiple Sclerosis Society
 - National Neurofibromatosis Foundation
 - National PKU News
 - National Retinal Pigmentosa Foundation, Inc.
 - National Scurvy's Syndrome Assoc.
 - National Scleroderma/Torporosis Assoc. (N.S.T.A.)
 - National Tay Sachs & Allied Diseases Association, Inc.
 - National Tuberculous Sclerosis Association, Inc.
 - National Urea Cycle Disorders Fdn.
 - National Vaginal Foundation, Inc.
 - Neurofibromatosis, Inc.
 - Oncofertility Consortium Foundation
 - Osteogenesis Imperfecta Foundation
 - Osteoarthritis & Hyperalgesia Foundation
 - Paget's Disease Foundation, Inc.
 - Parents of Galactosemic Children
 - Parkinson's Disease Foundation, Inc.
 - PKU Foundation
 - Prader-Willi Syndrome Association
 - Rett's Syndrome Association
 - Scleroderma Federation, Inc.
 - Scleroderma Int'l 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.
 - United Parkinson Foundation
 - United Parents Association for Pulmonary Hypertension, Inc.
 - Venous Disorders Association
 - Wegener's Granulomatosis Support Group
 - Williams Syndrome Association
 - Wilson's Disease Association

Associate Members

- | | | | | | |
|--|--|---|--|--|---|
| <ul style="list-style-type: none"> Academy Syndrome Newsletter Alabama Society for Sickle Disorders A.L.S. Association Greater Philadelphia Chapter American Brain's Association, Inc. American Self-Help Clearinghouse, N.J. ARC of Ohio Association for Children with Attention Deficit Syndrome, Inc. Center for Research in Sickle Disorders | <ul style="list-style-type: none"> Charcot-Marie-Tooth International Children's Leukemia Foundation Children's Leukemia Foundation for Juvenile Leukemia/Pediatric Chronic Granulomatous Disease Association Congenital Adrenal Hyperplasia Assoc. Inc. (CAHSA) Deafness Foundation Fallopian Tube Inlet Foundation for Nager & Miller Syndromes | <ul style="list-style-type: none"> Freeman Sheldon Parent Support Group Help Hospitalized Children's Fund Klepper Translucency Support Group Leimberg Society for Rare Disorders/Canada Lyme Borreliosis Foundation National Association for Pseudotumor Ectoderm | <ul style="list-style-type: none"> National Coalition for Research in Neurological & Communicative Disorders National Cushing's Association National Self-Help Clearinghouse (Israel) North American Pediatric Pseudo-Obstruction Society Organic Academic Association, Inc. Parents to Parents of GA, Inc. Parent to Parent of New Zealand | <ul style="list-style-type: none"> Port Abem Association for Children with Developmental Disabilities Research Trust for Metabolic Diseases in Children/England Rocky Mountain Resources & Training Institute Shy-Drager Syndrome Support Group Sickle Cell Association of the Texas Gulf Coast Solo's Syndrome Support Group Surge-Welder Foundation | <ul style="list-style-type: none"> Tourette Syndrome Assoc. of MD Tourette Syndrome Assoc. of Nova Scotia Tourette Syndrome Assoc. of OH Treacher-Collins Foundation Tuberous Sclerosis Assoc. of IL Venous Int. Masses Lysozymosis/France |
|--|--|---|--|--|---|

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

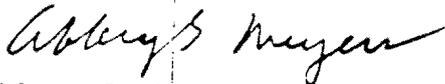
Dedicated to Helping People with Orphan Diseases

We believe in the free market system and the importance of profit as an incentive for research and development. However, we also believe there is a distinction between profit making and profiteering, particularly when these profits are being made at the expense of essentially helpless sick people.

While we have seen some evidence that the industry is beginning to rein in its wholesale price increases for drugs on the market, we continue to receive reports of increases at the retail level. Moreover, we have seen little sign of moderation in the price of new drugs, particularly "orphan drugs." Ceredase, for instance, a drug used to treat a rare genetic disorder called Gauchers Disease, is the most expensive drug in history. It costs over \$300,000 for the first year of treatment and drops off to a "mere" \$140,000 annually thereafter! Since many of the breakthrough drugs currently in the pipeline at FDA are designated as "orphan drugs", NCRD is particularly fearful of what the companies may charge for these new medications. The proposed Breakthrough Drug Committee, even though it could use more enforcement powers, is an absolute must for health care reform.

The pharmaceutical industry will not lose money under your health reform program. They will make up for price restraints through higher sales volume and a sorely needed reduction of their outrageous marketing expenses. Therefore, we urge you to retain the provisions for pharmaceutical cost containment, and especially the Breakthrough Drug Committee, which will hopefully be able to moderate the prices of new drugs.

Very truly yours,



Abbey S. Meyers
Executive Director

ASM:aa

cc: Chris Jennings ✓
Ira Magaziner

TO: Christine Heenan
FROM: Carol H. Rasco
SUBJ: National Organization for Rare Disorders
DATE: November 5, 1993

As you will remember the above referenced group is one several of us have visited with over recent months. Please review the attached letter and have other appropriate individuals review it to advise me before I go to OMB and Howard Paster. My initial reaction is that we probably have no business getting in the middle of something like this in the midst of health care reform but then again, perhaps it is a fit. I know the President is very interested in this group and its needs due to personal friends' involvement.

I need to answer this person by the close of business next week so if you can get something to me by noon Wednesday, November 10 it would be helpful. Thanks!

National Organization for Rare Disorders, Inc.

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



- President: Jake Thorne, M.D.
- Executive Director: Abbey S. Meyers
- Member Organizations:
 - Acoustic Neuroma Association
 - Alliance of Genetic Support Groups
 - American Carpal Tunnel Syndrome Association
 - American Macrodactyly Association
 - American Porphyria Foundation
 - American Stryngomyelia Adhesio Project
 - Aplastic Anemia Foundation of America
 - Association for Brain Tumor Research
 - Association for Glycogen Storage Disease
 - Batten Disease Support & Research Association
 - Benign Essential Spharoplastin Research Foundation, Inc.
 - Charcot-Marie-Tooth Association
 - Chromosome 18 Neglity and Research Society
 - Comelia de Lange Syndrome Foundation, Inc.
 - Cystinosis Foundation, Inc.
 - Direct Link for the Disabled, Inc.
 - Dysautonomia Foundation, Inc.
 - Dystonia Medical Research Project
 - 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
 - Foundation for Ichthyosis & Related Skin Types (F.I.R.S.T.)
 - Guillain-Barre Syndrome Foundation International
 - Hemochromatosis Research Foundation, Inc.
 - Hereditary Disease Foundation
 - Histiocytosis Association of America
 - Hurler's Disease Society of America, Inc.
 - Immune Deficiency Foundation
 - International Fibromyalgia Organization
 - Progressive (FDP)
 - International Juvenile Osteoporosis Foundation
 - International Rett Syndrome Association, Inc.
 - Interstitial Cystitis Association of America, Inc.
 - Leahy's Syndrome Association
 - National Hyperparathyroid Association of the United States
 - Maria's Network (CAN Foundation)
 - Mitochondrial Disease Center
 - Neurology Network
 - National Addison's Disease Foundation
 - National Alopecia Areata Foundation
 - National Association for Pseudotumor Elicthema
 - National Association for Spina Cord Disease, Inc.
 - National Alopecia Foundation
 - National Chronic Fatigue Syndrome Association, Inc.
 - National Foundation for Ectodermal Dysplasias
 - National Fragile X Foundation
 - National Gaucher Foundation, Inc.
 - National Leigh's Disease Foundation
 - National Marfan Foundation
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 - National Multiple Sclerosis Society
 - National Neurofibromatosis Foundation
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 - National Scurvy's Syndrome Assoc.
 - National Tubercous Sclerosis Association, Inc.
 - National Urea Cycle Disorders Fdn.
 - National Vitiligo Foundation, Inc.
 - Neurofibromatosis, Inc.
 - Obsessive Compulsive Foundation
 - Osteogenesis Imperfecta Foundation
 - Osteoporosis & Metabolic Disorders Foundation
 - Paget's Disease Foundation, Inc.
 - Parents of Bacteremic Children
 - Parkinson's Disease Foundation, Inc.
 - Polycystic Kidney Research Foundation
 - Prader-Willi Syndrome Association
 - Reflex Sympathetic Dystrophy Syndrome Association
 - Rodriguez Pigmentosa Foundation
 - Fighting Blindness
 - Scleroderma Federation, Inc.
 - Scleroderma Info Exchange, Inc.
 - Sjogren's Syndrome Foundation, Inc.
 - Tourette Syndrome Association, Inc.
 - Trigeminal Neuralgia Association
 - United Leukodystrophy Foundation, Inc.
 - United Postures Foundation
 - Vertebral Disorders Association
 - Wegener's Granulomatosis Support Group
 - Williams Syndrome Association
 - Wilson's Disease Association

Date: November 4

NOV 4 REC'D

FAX TRANSMITTAL

TO: Carol Rasco

ORGANIZATION: Office of Domestic Policy

FROM: Abbey Meyers

SUBJECT: Orphan Drug Act Amendments

REMARKS:

This is page 1 of 12 pages.

About Face/CANADA
Algodol Syndrome Newsletter
Albama Society for Sleep Disorders
American Balkans Association, Inc.
American Pediatric Gastroenterology
Relief Association, Inc.
American Self-Help Clearinghouse, Inc.
American Society of Adults with
Pseudo-Obstruction, Inc.
Amyotrophic Lateral Sclerosis
Association
Association for Children with
Russett-Silver Syndrome, Inc.

Brain Injured Adult Resource Center
Center for Research in Sleep
Disorders
Charcot-Marie-Tooth International
Children's Information Center
Children's Leukemia Foundation/ML
Chronic Granulomatous Disease
Association
Congenital Adrenal Hyperplasia
Assoc., Inc. (CAHSA)
Deafness Foundation
Family Survival Project for
Brain-Damaged Adults

Foundation for Nagar & Miller
Syndromes
Freeman-Sheldon Parent Support
Group
Kissel-Transmuty support
Group
Lehrer's Society for Rare
Disorders/Canada
Lynch-Bornstein Foundation
National Chronic Fatigue Syndrome
Association

Associate Members
National Coalition for Research in
Neurological & Communicative
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National Cushing's Association
National Fibromyalgia
Support Group
North American Pediatric
Pseudo-Obstruction Society
Orphan Disease Association, Inc.
Parent To Parent of GA, Inc.
Parent To Parent of NHE 228280

Pon Albans Association for Children
with Developmental Disabilities
Research Trust for Mucopolysaccharidosis
Diseases in Children/England
Shy-Drager Syndrome Support Group
Shy-Drager Association of the
Yonkers Golf Course
Society for Progressive
Supernumerary Ptery
gium Syndrome Support Group
Surge-Holzer Foundation
The MACC Foundation

Tourette Syndrome Assoc. of MD
Tourette Syndrome Assoc. of
Nova Scotia
Tourette Syndrome Assoc. of ON
Treasurer-Collins Foundation
Tuberous Sclerosis Assoc. of IL
Valley's Les Maladies
Lyonnaises/France

*Associations are joining
separately. For details please
contact the NORD office.

Dedicated to Helping People with Orphan Diseases

National Organization for Rare Disorders, Inc.

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



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

November 4, 1993

Ms. Carol Rasco
Assistant to the President
for Domestic Policy
Office of Domestic Policy
Old Executive Office Building
Washington, DC 20500

Dear Carol:

As you know from our discussion last May, Senator Howard Metzenbaum and Senator Nancy Kassebaum intend to introduce the Orphan Drug Act Amendments this year. Similar amendments were vetoed by President Bush in 1980, and in 1992 the amendments were stopped by intense pharmaceutical industry lobbying.

I am attaching a draft copy of the 1993 amendments. We really need the support of the White House on this issue. If you need details, Bill Corr, Deputy Assistant Secretary for Health, is an expert on the subject of orphan drugs and he can provide all of the political intricacies involved with the legislation.

Carol, we expect the drug industry to lobby against this bill as if it were a test run for health care reform. We hope to hold a press conference when it is introduced and it would be significant if someone from the White House were present to show the administration's support of the bill. The legislation is a first step in pharmaceutical cost containment.

If you have any questions, or if I can provide further information, please do not hesitate to contact me.

Very truly yours,

Abbey S. Meyers
Abbey S. Meyers
Executive Director

cc: Kathy McClanahan
Bill Corr

- President: Jess Troens, M.D.
- Executive Director: Abbey S. Meyers
- Member Organizations:
- Alzheimers Association
- Alzheimer's Disease Support Group
- Alpha Anterograde Amnesia National Association
- American Brain Tumor Association
- American Carpal Tunnel Syndrome Association
- American Neurology Association, Inc.
- American Paralysis Foundation
- American Society of Adult with Pseudo Obstruction, Inc. (ASAP)
- American Strykerella Alliance Project
- Asplastic Anemia Foundation of America
- Association for Glycogen Storage Disease
- Balton Disease Support & Research Association
- Benign Essential Hypertension Research Foundation, Inc.
- Chloroquine Toxicity Association
- Chromosomes to Health and Research Society
- Cornelia de Lange Syndrome Foundation, Inc.
- Cystinosis Foundation, Inc.
- Direct Link for the Disabled, Inc.
- Dysautonomia Foundation, Inc.
- Dyslexia Medical Research Foundation
- Dysphagia Epidemiology Bureau (D.E.B.)
- Ehlers-Danlos National Foundation
- Elphing Foundation of America
- Familial Amyloidosis Research Fund, Inc.
- Foundation for Rare Diseases (F.R.D.)
- Frederick Sick Types (F.I.S.T.)
- Gallbladder-Spleen 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 Coalition
- International Joseph Disease Foundation, Inc.
- International Rett Syndrome Association
- International Cystic Association of America, Inc.
- Loewy Syndrome Association
- Metabolic Hypertension Association of the United States
- Metabolic Hypertension Center
- National Adrenal Disease Foundation
- National Alzheimers Assoc. Foundation
- National Association for Sickle Cell Disease, Inc.
- National Ataxia Foundation
- National Chronic Fatigue Syndrome Association, Inc.
- National Foundation for Neurodermatitis
- National Fragile X Foundation
- National Genetic Foundation
- National Lymphoma Disease Foundation
- National Marfan Foundation
- National Mucopolysaccharidosis Society, Inc.
- National Multiple Sclerosis Society
- National Neuroendocrine Foundation
- National PIR News
- National Pediatric Fibrosarcoma Foundation, Inc.
- National Sjogren's Syndrome Assoc.
- National Spondylo Arthritis Assoc. (NSAA)
- National Tay-Sachs & Allied Diseases Association, Inc.
- National Tuberculosis Genetosis Association, Inc.
- National Urea Cycle Disorders Fdn.
- National Village Foundation, Inc.
- Neurofibromatosis, Inc.
- Obsessive Compulsive Foundation
- Osteogenesis Imperfecta Foundation
- Orbit & Hypertension Foundation
- Reagan Disease Foundation, Inc.
- Parents of Galactosem Children
- Paroxysmal Disease Foundation, Inc.
- PIR Foundation
- Piper-VA Syndrome Association
- Polar Spherulosis Dystrophy Syndrome Association
- Sideroblastic Anemia, Inc.
- Sideroblastic Anemia Assoc. (SAA)
- Sjogren's Syndrome Foundation, Inc.
- The A.L.E. Association
- The GAR Foundation
- The Myofascial Crisis Foundation
- Tourette Syndrome Association
- Tribunal Neurology Association
- United Lactobacilli Foundation, Inc.
- United Paralysis Foundation
- United Parents Association for Pathology Hypertension, Inc.
- Vasculitis Disease Association
- Wegener's Granulomatosis Support Group
- Williams Syndrome Association
- Widow's Disease Association

- Alzheimers Newsletter
- Alabama Society for Blind Disorders
- A.L.E. Association Quarterly
- Philadelphia Chapter
- American Deafness Association, Inc.
- American Self-Help Clearinghouse
- A.L.E.
- ARC of Ohio
- Association for Children with Kluver-Bucy Syndrome, Inc.
- Center for Research in Sleep Disorders

- Charlotte-Mecklenburg International Children's Leukemia Foundation
- Chloroquine Toxicity Foundation for Juvenile Liver and Kidney Disease
- Chronic Granulomatous Disease Association
- Compensated Adrenal Hypocortisolism Assoc., Inc. (CANSAA)
- Deveraux Foundation
- Footprints Institute
- Foundation for Nager & Miller Syndromes

- Freeman-Sheldon Parent Support Group
- Heic Homebased Children's Fund
- Kippax-Tansuany Support Group
- Lehigh Valley Society for Rare Disorders/Canada
- Lyme Borreliosis Foundation
- National Association for Neuroendocrine Education

- National Coalition for Research in Neurological & Communicative Disorders
- National Cystinosis Association
- National Self-Help Clearinghouse (NSHC)
- North American Pediatric Parent-Organization Society
- Organic Acidemia Association, Inc.
- Parent to Parent of SA, Inc.
- Parent to Parent of New Zealand

- Port Alford Association for Children with Development Disabilities
- Research Trust for Metabolic Diseases in Children/England
- Rocky Mountain Resource & Training Institute
- Shy-Drager Syndrome Support Group
- State 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
- Tourette Syndrome Foundation
- Tuberous Sclerosis Assoc. of IL
- Vibrona les Maladies Lymphomatiques/France

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

Dedicated to Helping People with Orphan Diseases

103D CONGRESS
1ST SESSION

S. _____

IN THE SENATE OF THE UNITED STATES

Mrs. KASPERBAUM (for herself and Mr. METZENBAUM) introduced the following bill; which was read twice and referred to the Committee on _____

A BILL

To amend the Federal Food, Drug, and Cosmetic Act, the Public Health Service Act, and the Orphan Drug Act to revise the provisions of such Acts relating to orphan drugs, and for other purposes.

1 *Be it enacted by the Senate and House of Representa-*
2 *tives of the United States of America in Congress assembled,*

3 **SECTION 1. SHORT TITLE AND REFERENCE.**

4 (a) **SHORT TITLE.**—This Act may be cited as the
5 "Orphan Drug Act Amendments of 1993".

6 (b) **REFERENCE.**—Whenever in this Act (other than
7 sections 5 and 6) an amendment or repeal is expressed
8 in terms of an amendment to, or repeal of, a section or
9 other provision, the reference shall be considered to be

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1 made to a section or other provision of the Federal Food,
2 Drug, and Cosmetic Act.

3 **SEC. 2. PERIOD OF EXCLUSIVITY.**

4 (a) **INITIAL PERIOD.**—Subsection (a) of section 527
5 (21 U.S.C. 360cc) is amended—

6 (1) by redesignating paragraphs (1), (2), and
7 (3) as subparagraphs (A), (B), and (C), respectively;

8 (2) by inserting “(1)” after “(a)”;

9 (3) by striking “seven years” and inserting “4
10 years”; and

11 (4) by striking “505(c)(2)” and inserting
12 “505(c)(1)(B)”.

13 (b) **ADDITIONAL PERIOD.**—Subsection (a) of section
14 527 (21 U.S.C. 360cc) (as amended by subsection (a)) is
15 further amended by adding at the end the following new
16 paragraphs:

17 “(2)(A) The holder of the approved application, cer-
18 tification, or license of a drug to which the 4-year period
19 of exclusivity applies under paragraph (1) may, after the
20 expiration of 3½ years of such period but not later than
21 90 days before the expiration of such period, submit an
22 application to the Secretary for a 3-year extension of such
23 period. Such an application shall contain such information
24 as the Secretary determines is necessary to evaluate such
25 application.

October 1, 1993

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1 “(B) The Secretary shall make a decision on an appli-
 2 cation submitted under subparagraph (A) within 60 days
 3 of the date of such application's submission if the appli-
 4 cant demonstrates that the net cumulative sales of the
 5 drug for which the application was submitted will not ex-
 6 ceed \$200,000,000 during such 4-year period.

7 “(3)(A) After an application submitted under para-
 8 graph (2)(A) has been approved by the Secretary for a
 9 drug, any person may petition the Secretary to terminate
 10 the additional period provided under such approval on the
 11 basis that the net cumulative sales of such drug exceed
 12 \$200,000,000. The Secretary shall provide the holder of
 13 such approved application a reasonable opportunity to re-
 14 spond to the petition within 30 days of the date of the
 15 submission of the petition. Such a petition and response
 16 shall contain such information as the Secretary determines
 17 is necessary to evaluate the petition. The Secretary shall
 18 complete action on a petition within 90 days of the date
 19 of its submission.

20 “(B) If the Secretary determines that the cumulative
 21 net sales of a drug are more than \$200,000,000 during
 22 the period beginning on the approval of such drug and
 23 ending on the date of the submission of the petition, the
 24 Secretary shall issue a notice stating that the prohibition

4 years min
 3 year est
 620 million 4yr

What about
 price rises
 before 4th yr
 7th year?

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1 on approvals in paragraph (1) does not apply to such
2 drug."

3 (c) DEFINITION.—Subsection (a) of section 527 (21
4 U.S.C. 360cc) (as amended by subsection (b)) is further
5 amended by adding at the end the following new para-
6 graph:

7 "(4) As used in this subsection, the term "cumulative
8 net sales" of a drug means total sales of the drug in the
9 United States minus discounts, allowances, and returns."

10 SEC. 3. DESIGNATIONS.

11 (a) IN GENERAL.—Section 526(a)(2) (21 U.S.C.
12 360bb(a)(2)) is amended by adding before the period at
13 the end the following: "and on the basis of projections as
14 to the number of persons who will be affected by the dis-
15 ease or condition 3 years from such date".

16 (b) EXCLUSIVITY.—Section 527(b) (21 U.S.C.
17 360cc(b)) is amended—

18 (1) by striking "or" at the end of paragraph
19 (1);

20 (2) by striking the period at the end of para-
21 graph (2) and inserting a semicolon; and

22 (3) by adding at the end the following new
23 paragraphs:

24 "(3) a drug has been designated under section
25 526 for a rare disease or condition described in sec-

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1 tion 526(a)(2)(A) and if after such designation for
2 such disease or condition does not meet such de-
3 scription;

4 "(4) the Secretary has issued a notice under
5 subsection (a)(3); or".

6 **SEC. 4. SIMULTANEOUS DEVELOPMENT.**

7 (a) **IN GENERAL.**—Section 527(b) (21 U.S.C. 860cc(b))
8 (as amended by section 2(b)) is amended—

9 (1) by redesignating paragraphs (1), (2), (3),
10 and (4) as subparagraphs (A), (B), (C), and (D), re-
11 spectively;

12 (2) by inserting "(1)" after "(b)";

13 (8) in the matter preceding paragraph (1)(A)
14 (as so redesignated) by striking "for a person who
15 is not" and by inserting "for an applicant who is
16 not"; and

17 (4) by adding at the end the following:

18 "(E) the Secretary finds, after providing the hold-
19 er, such applicant, and any other interested person
20 an opportunity to present their views, that the drugs
21 of the holder and such applicant were developed si-
22 multaneously.

23 The Secretary shall make a decision on a request for a
24 finding under subparagraph (E) not later than 60 days
25 after the filing of the request.

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1 “(2) For purposes of paragraph (1)(E), drugs of a hold-
2 er and an applicant shall be considered to be developed
3 simultaneously only if—

4 “(A) the applicant requested that its drug be des-
5 ignated under section 526 no later than 6 months
6 after publication of the designation under section
7 526(e) of the holder's drug;

8 “(B) the applicant initiated the human clinical
9 trials that the applicant relied on in its application
10 for such approval, certification, or license not more
11 than 12 months after the date the holder initiated
12 the human clinical trials that the holder relied on in
13 its application for such approval, certification, or li-
14 cense; and

15 “(C) the applicant submitted such application, in-
16 cluding the reports of the clinical and animal studies
17 necessary for approval, certification, or licensing, not
18 more than 12 months after the holder submitted its
19 application, including such reports, for such ac-
20 tion.”

21 (b) PUBLICATION.—Section 526(c) (21 U.S.C. 360bb(c))
22 is amended—

23 (1) by inserting “for a rare disease or condition”
24 after “(a)”; and

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1 (2) by striking out "shall be made available to the
2 public" and inserting in lieu thereof "shall be
3 promptly published in the Federal Register and oth-
4 erwise made available to the public in a manner de-
5 signed to notify persons who have such disease or
6 condition".

7 **SEC. 5. OFFICE FOR ORPHAN DISEASES AND CONDITIONS.**

8 Section 227 of the Public Health Service Act (42
9 U.S.C. 236) is amended—

10 (1) in subsection (a), to read as follows:

11 "(a) There is established in the Department of
12 Health and Human Services an Office for Orphan Dis-
13 eases and Conditions. The Office shall be established at
14 a level within the Department with sufficient authority to
15 assure full implementation of the functions and respon-
16 sibilities established by this section.";

17 (2) by striking "Board" each place the term ap-
18 pears and inserting "Office";

19 (3) in subsection (b), by striking "drugs and
20 devices" and inserting "drugs, devices, and medical
21 foods";

22 (4) in subsection (c)(1)(A), by inserting "of
23 chapter V" after "subchapter B";

24 (5) by adding at the end the following new sub-
25 section:

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1 “(f)(1) There is established in the Office an advisory
2 committee to advise the Office in carrying out the func-
3 tions of the Office under this section.

4 “(2) The advisory committee shall be comprised of
5 11 members appointed by the Secretary, in consultation
6 with the Office and the Commissioner of the Food and
7 Drug Administration, from persons knowledgeable about
8 rare diseases and conditions, of which—

9 “(A) 5 shall be representatives of organizations
10 of persons with rare diseases or conditions;

11 “(B) 3 shall be research scientists; and

12 “(C) 3 shall be representatives of health-related
13 companies.

14 “(3) The Secretary shall also appoint, as liaisons to
15 the advisory committee, individuals from the Food and
16 Drug Administration, the National Institutes of Health,
17 and other appropriate Federal agencies.

18 “(4) Vacancies occurring in the membership of the
19 advisory committee shall be filled in the same manner as
20 the original appointment for the position being vacated.
21 Vacancies shall not affect the power of the remaining
22 members to execute the duties of the advisory committee.

23 “(5) Members of the advisory committee, and liaisons
24 to the advisory committee, shall not be compensated, but
25 shall receive travel expenses, including per diem in lieu

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1 of subsistence, at rates authorized for employees of agen-
2 cies under subchapter 1 of chapter 57 of title 5, United
3 States Code, for each day the member or liaison is en-
4 gaged in the performance of duties away from the home
5 or regular place of business of the member or liaison.

6 "(6) Notwithstanding section 1842 of title 31, United
7 States Code, the advisory committee may accept the vol-
8 untary services provided by a member of the advisory com-
9 mittee or a liaison to the advisory committee."; and

10 (6) by amending the section heading to read as
11 follows: "OFFICE FOR ORPHAN DISEASES AND CON-
12 DITIONS".

13 **SEC. 6. AUTHORIZATION FOR ORPHAN DRUG ACT.**

14 Section 5(c) of the Orphan Drug Act (21 U.S.C.
15 360ee(c)) is amended by striking "\$10,000,000" and all
16 that follows and inserting "\$20,000,000 for fiscal year
17 1995, \$25,000,000 for fiscal year 1996, and \$30,000,000
18 for fiscal year 1997."

19 **SEC. 7. APPLICABILITY OF AMENDMENTS.**

20 The amendments made under sections 2 and 4 shall
21 apply only to an application, certification, or license for
22 a drug designated under section 526 (21 U.S.C. 360bb)
23 for a rare disease or condition that is approved or issued
24 after the date of enactment of this Act.

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- 1 SEC. 8. EFFECTIVE DATE OF DESIGNATION AMENDMENTS.
- 2 The amendments made under section 8, ^{and 8} shall take ef-
- 3 fect on the date of enactment of this Act.

October 1, 1993