

The Orphan Drug Act

National Organization for Rare Disorders (NORD®)



THE SPARK THAT CAUGHT FIRE

A movement that changed the lives of countless Americans was ignited by a mother on a mission.

In the 1970s, Abbey Meyers was fighting for a treatment for her son's Tourette's syndrome. Meyers found an effective experimental drug but was told it wouldn't come to market – the drug maker had deemed the disease “too rare” to make production worth it.

Undaunted, Meyers turned her crusade into a coalition of rare disease trailblazers. Together, they led the national movement that resulted in the Orphan Drug Act (ODA) and the founding of the National Organization for Rare Disorders (NORD®).

Enacted into law in 1983, the ODA incentivizes drug makers to develop “orphan” drugs for rare disorders, allowing patients and families to, for the first time, hope for a treatment or cure. That hope persists today as Meyers' legacy, carried forward by NORD and a strong community of rare disease patients, caregivers, advocates, health care professionals and researchers.

Alone we are rare. Together we are strong.®

OUR IMPACT

40 years ago, NORD's founders won the fight to pass the Orphan Drug Act – and we haven't stopped fighting. Here are some highlights from the past few decades:

 **880+**

orphan drugs have been FDA-approved as a result of the Orphan Drug Act.*

* As of 2023

Source: Orphanet Journal of Rare Diseases



NORD launched the nation's **first rare disease patient assistance program** in 1987, providing urgently needed financial support to thousands of patients and caregivers.



NORD lobbied and helped launch the **first NIH Office of Rare Diseases Research**, which serves as the federal focal point for rare disease biomedical research.



NORD developed the **first natural history patient registry platform** to serve the rare disease patient community. Today the IAMRARE® patient registry platform serves more than 35 patient registries.



The NORD Rare Disease Centers of Excellence Network is the **first and only world-class network of U.S. hospitals and medical institutions** dedicated to diagnosing, treating and researching all rare diseases.

THE LOS ANGELES TIMES

PART 1

FRIDAY, DECEMBER 31, 1982

25 CENTS

MR. PRESIDENT, PLEASE SIGN THE ORPHAN DRUG ACT

Dear Mr. President,

While you are back home for the holiday, we hope you will see this letter. We represent millions of Americans who suffer from over 2,000 rare diseases. Our only hope is the ORPHAN DRUG ACT. This bill authored by Congressman Henry A. Waxman, is now sitting on your desk. It would give tax credits to drug companies that develop treatments for diseases that occur so infrequently that no company can expect to profit from the new drugs.

Just two weeks ago we rejoiced at the news that the ORPHAN DRUG ACT had passed both the Senate and the House of Representatives by unanimous vote.

Shortly before Christmas we were shocked to learn that you are considering vetoing the ORPHAN DRUG ACT. This news turned our holidays from a time of joy to one of deep despair.

Without the ORPHAN DRUG ACT some of us are doomed to an early death. Some of us will be forced to face painful and disabling sicknesses with no hope of recovery or even relief.

Your signature before January 4th will bring America's great pharmaceutical industry into partnership with the Federal government on our behalf. It is incomprehensible to us and to our families that you would reject this opportunity to alleviate so much human suffering.

PLEASE SIGN THE ORPHAN DRUG ACT TODAY!

Sharon Dobkin
Myeloid Families United
Dennis Smur
Paralyzed Veterans of America
Charlotte Drake
Parkinson's Ed. Program
John Chung
Wilson's Disease Assoc.
Ruth Horvitz
Nat'l Huntington's Disease Assoc.
George Brewer, M.D.
Jes Thoen, M.D.
Univ. of Michigan
Marjorie Guthrie
Comm. to Combat Huntington's Disease

Judy Roser
United Parkinson Disease Found.
Barbara Landwehr
Nat'l Ichthyosis Found.
Dick Vodre
Cystic Fibrosis Found.
Burt Diamond
Nat'l Myeloid Found.
Eames Bishop
Amyotrophic Lateral Sclerosis Society
Rose Marie Silva
Internat'l Joseph Diseases Assoc.
Ther Hanson
Nat'l Multiple Sclerosis

Anne Kone
Paget's Disease Found.
Abbey Meyers
Tourette Syndrome Assoc.
Melvin Van Woert, M.D.
Mt. Sinai Sch. of Medicine
William Baird
American Narcolepsy Assoc.
Arlene Pessar
Dystrophic Epidermolysis
Research Foundation
Rita Kasky
Nat'l Neurofibromatosis Foundation
Rubin Bakin
Gaucher's Disease Internat'l Registry

**PLEASE CALL THE
WHITE HOUSE IMMEDIATELY!
(202) 456-1414**

There is no time for letters. Without the President's signature the Orphan Drug Act will die January 4, 1983.

Committee for Orphan Drug Act
8425 W. Third St., Los Angeles, CA. 90048

When the coalition of rare disease advocates heard that President Reagan might not enact the Orphan Drug Act, they bought ads in The Washington Post and The Los Angeles Times to appeal to the President during his holiday vacation.

The ads were made possible by a \$10,000 anonymous gift and resulted in a flood of calls to the White House in support of the ODA.

* This is a rendition of the original ad that ran in the Los Angeles Times

Alone we are rare. Together we are strong.®



NORD®
National Organization
for Rare Disorders

WHAT IS THE ORPHAN DRUG ACT?

1983

The Orphan Drug Act was enacted in 1983 and became the world's first law to incentivize biopharmaceutical companies to invest in the development of drugs and biologics, known as "orphan drugs," to treat individuals with rare diseases.

A RARE DISEASE IS ANY
CONDITION AFFECTING
FEWER THAN
200,000
AMERICANS

HOW DOES THE ORPHAN DRUG ACT WORK?

THERE ARE

4

INCENTIVES

in the ODA to encourage
drug development

7

YEARS

of market exclusivity

25%

TAX CREDIT

for clinical testing expenses¹

EXEMPTION

from FDA marketing
application fees²

FEDERAL
RESEARCH
GRANTS

for clinical testing of new
therapies to diagnose
and treat rare diseases

THE FIGHT CONTINUES



Prior to 1983, there were fewer than 40 FDA-approved therapies to treat rare diseases, leaving millions of patients without treatments.³



Today, there are 880 FDA-approved treatments.



More than 95% of rare diseases are still WITHOUT any FDA-approved treatment.

Today, the approximately 30 million Americans living with a rare disease have new options for treatment and hope of more innovations in the years to come. However, less than 5% of 7,000 known rare diseases have treatments and so we fight on.

References: 1. Orphan Drugs in the United States: An Examination of Patents and Orphan Drug Exclusivity (2021). 2. Tax Cuts and Jobs Act, Pub L No. 115-97, 131 Stat 2054 (2017). 3. Food and Drug Administration Modernization Act, Pub L No. 105-115, 111 Stat 2297 (1997).

We cannot fight alone – we need your help. Learn how to join us in the fight:
rarediseases.org/get-involved



Alone we are rare.
Together we are strong.®

Learn more: rarediseases.org

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