

The Power of the Rare Disease Community...



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National Organization for Rare Disorders (NORD)

1st International Conference on Rare Diseases and
Orphan Drugs

Stockholm
February 14-16, 2005



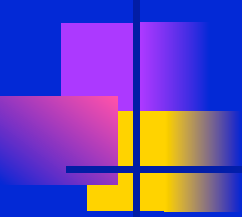
National Organization
for Rare Disorders

- Dedicated to the identification, treatment and cure of rare disorders
- Education, advocacy, research and service



\$4.5 Million in Research Grants...

- 1989 - NORD funded its first research grants totaling \$142,000
- Today - nearly \$4 million has been awarded to fund 97 grants and fellowships
- Fall 2005 – 13 additional grants totaling \$430,000 will be awarded

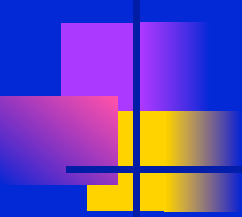
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- Small grants to academic scientists to study new treatments or diagnostics for rare diseases
 - May provide preliminary data indicating that a treatment may be safe and effective when used for a larger number of patients

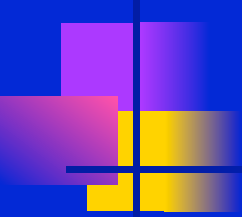
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- Use the preliminary data to apply for larger multi-year government grants, or;
 - Attract a commercial sponsor who will manufacture an orphan product and get FDA approval



Restricted Grants Criteria

- If the total donated for a specific disease is less than \$1,000 after 2 years
 - Funds are transferred to general research
- If the total donated for a specific disease is more than \$1,000 but less than \$5,000 after 4 years
 - Funds transferred to research on related diseases

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- If the total donated for a specific disease is more than \$5,000 but less than \$10,000 after 6 years
 - Funds transferred to research on related diseases
 - If the total donated for a specific disease is more than \$10,000 but less than \$15,000 after 8 years
 - Funds transferred to research on related diseases

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- If the total donated for research for a specific diseases is less than \$25,000 in 10 years
 - Funds are transferred to research on related diseases



The Power of the Many...





The Rare Disease Community in the United States

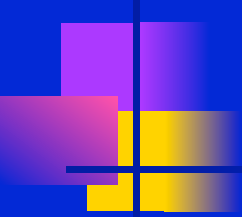
Nearly 30 Million Americans Living With One of the 6,000 Known Rare Diseases...




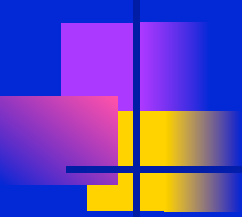
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- **August 3, 2001** -- Introduction of the *Rare Diseases Act*
 - **March 28, 2002** – Introduction of the *Rare Diseases Act*
 - **March 28, 2002** – Introduction of the *Rare Diseases Orphan Product Development Act*

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- **November 6, 2002** - President Bush signs both the the *Rare Diseases Act* and the *Rare Diseases Orphan Product Development Act*

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- **May 19, 2003** – Resolution passed in the House of Representatives commemorating the 20th Anniversary of the *Orphan Drug Act* and NORD

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- **July 20, 2003** – Introduction of the *Medicare Patient Access to Drugs for Rare Diseases Act of 2003*

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- **November 3, 2003** – NIH announces Rare Diseases Clinical Research Network
 - \$51 million in grant funding over 5 years
 - 7 Rare Diseases Clinical Research Centers
 - Data and Technology Coordinating Center
 - Trans-NIH Working Group on Rare Diseases Research

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- **December 8, 2003** – Medicare Prescription Drug Improvement and Modernization Act (MMA) signed into law
 - Ensured adequate reimbursement for orphan products administered in a hospital or outpatient setting for 2004 and 2005



- **June, 2004 – FY 2005 FDA Appropriations Language**

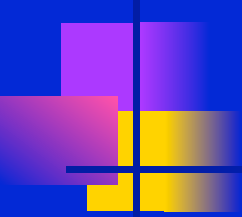
- \$1.2 million increase for the Orphan Products Research Grants Program
- Encourages FDA to speed development and approval of orphan drugs



- **June, 2004 – FY 2005 NIH Appropriations Language**

- Commends Office of Rare Diseases for rapid progress
- Encourages increased research/ interventions
- Encourages NIH to pursue exploratory grants and proof of concept studies

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- Concerned that Medicare patients with rare diseases may have difficulties accessing care that involves orphan drugs
 - Encourages CMS to carefully consider the impact on this population in proposing regulations
 - Encourages CMS to solicit the views of the OOPD, ORD, and stakeholders before determining whether an access problem exists or would be made worse by proposed regulations

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- October 27, 2004 – Fabry Disease Network Stakeholders Meeting, Toronto, Canada
 - Fabry Network of Excellence
 - Replegal® and Fabrazyme® approved by Health Canada
 - Common Drug Review would not recommend reimbursement
 - January 2005 approved for reimbursement



Federal Funding Increases

- Office of Rare Diseases, NIH
 - 2001 - \$2.1 million
 - 2003 - \$51 million for research
 - 2005 - \$16 million
- Orphan Products Research Grants Program, FDA
 - 2005 – \$1.2 million increase



Summary



The Power of Millions...

- Increased awareness of rare diseases throughout the U.S. Congress and government agencies
- Increased research funding
- Empowered to affect legislation and regulations
- Empowered to change their lives



Without the Rare Disease Community There Would Be **NO...**

- Orphan Drug Act
- Office of Orphan Product Development, FDA
- Orphan Products Research Grants
- Office of Rare Diseases, NIH
- ICORD



Thank you...



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