

Fostering Collaboration to Accelerate Better Patient Outcomes

NORD Initiatives and Projects

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What Is NORD?

- **Our Mission**
- NORD, a 501(c)(3) organization, is a patient advocacy organization dedicated to individuals with rare diseases and the organizations that serve them. NORD, along with its more than 230 patient organization members (over 1000 conditions), is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.



The Issues around Rare Disease

Some rare disease factoids

- 30% of rare disease patients will die before they are 5 years old (["Rare Diseases"](#). SiopE.eu. 2009-06-09)
- Rare diseases affect 8-10% of the U.S. population (about 30,000,000) (NIH)
- If all of the people with rare diseases lived in one country, it would be the world's 3rd most populous country
- The NIH currently lists over 7,300 genetic rare diseases and growing.
- Only about 400 of these have some form of approved treatment
- 2/3 of the patients are children
- A 2004 study on the impact of genetic disease on pediatric patients found
 - An underlying disorder with a significant genetic component was found in 71% of admitted children.
 - 34% of Children's Hospital had a primary rare disease diagnosis and accounted for 50% (>\$31 million) of the total hospital charges.
 - The mean length of stay was 40% longer for individuals with an underlying disease with a genetic/rare basis than for those with no underlying disease.

Where Do Patients and NORD Fit In

- Treatment approval process (FDA)
 - What are “relevant” outcomes.
 - Patient participation on panels
 - Patient driven acceptable risk models
- Patient Centered Outcomes (know what you know and don't know)
 - NORD Patient registry program
 - FDA-NIH-NORD Collaboration
 - Economic collection of natural history data suitable for refining treatments and testing new ones.
 - Data entered primarily by patients and families

- Rare Disease Study Accelerators
 - Informed Consent Reform (reliance networks)
 - Barriers to studies in small populations across multiple sites.
 - Rare Disease study coordination
 - Match making (Rare Disease Summit)
 - Patients with organizations
 - Researchers with patients and organizations
 - Industry with all of the above
- Informatics
 - Patient and practitioner friendly guidelines
 - Consensus treatment meetings with patients, clinicians, and investigators.
 - Training for patient organizations.