The Unmet Need: Partnership with Patient Advocate Groups

Ron DeBellis, Pharm.D., FCCP
Scientific Advisor
National Organization for Rare Disorders
Professor of Clinical Sciences
Keck Graduate Institute School of Pharmacy
Conflicts of Interest

NONE
NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.
About NORD
Four Pillars

- Founded by patients, caregivers and patient organization leaders in 1983 to “unify the voice of the rare disease community.”
- Leading advocacy organization addressing the challenges faced by patients and families at the local, state and federal levels.
- NORD, along with our 270+ disease-specific organizational members, are committed to the identification, treatment, and cure of rare disorders through programs of:

  - Policy & Advocacy
  - Research
  - Education
  - Patient Assistance
Challenges for Rare Patients

- Average of 7-9 years for diagnosis
- Only ~5% have an FDA approved treatment or therapy
- Extensive, life-long medical needs
- High cost of care and treatment
- Few medical experts
- Little research or known about diseases
- Social Isolation
- Small, scattered patient populations
Unity Through Registries and Research
NORD Finds Unmet Research Needs

- **Support for registry clients**
  - Guide clients through the development of a registry
  - Provide insight on survey design
  - Offer interdisciplinary learning opportunities to improve recruitment and retention strategies
  - Provide training on reporting and analysis

- **Foster sense of community**
  - Move from each registry functioning separately to harmonization
  - Propagate sharing of ideas
  - Foster cross-registry learning opportunities so clients can learn from each other rather than exclusively from NORD

- **Identify unmet need**
  - Pt. advocacy orgs commonly lack research training, m/b connected with academic or medical research
  - Want to do the best for their patients
  - Little or no experience in research and data analysis
  - Orgs within the same disease state do not always work with each other

- **Groups get each other excited about research possibilities!!**
Benefits of the NORD Partnership
Outcome From Working With NORD

- Advocacy on behalf of working with RD community to facilitate data collection
- Use of data to provide pt. orgs a seat at the table with industry and govt for reasons other than listening to pt. stories
- Provide governance on research structure for organizations
- Have a relationship with and provide access to Hummingbird IRB

- Enhance recruitment and retention
  - Provide landscape competitor research in order to keep front end of our system engaging and stimulating participants to return
  - Share information from other NP’s who are running successful registries
  - Provide tips, tools and platform development techniques that we learn from others
NORD’s FDA Backed Platform

• Pt. advocacy orgs can use NORD’s core survey that is backed by data standards, and can develop and/or add unique disease-specific questions in partnership with their scientific advisory committee that reflect the needs of the pt population

• Platform is backed by the FDA who provided guidance and funding support via a cooperative agreement to bring on 20 clients to expand and develop the system
  ▪ Under the terms of the cooperative agreement, each client brought on could initially consult with the FDA
Research@rarediseases.org
Thank you.

Alone we are rare. Together we are strong.