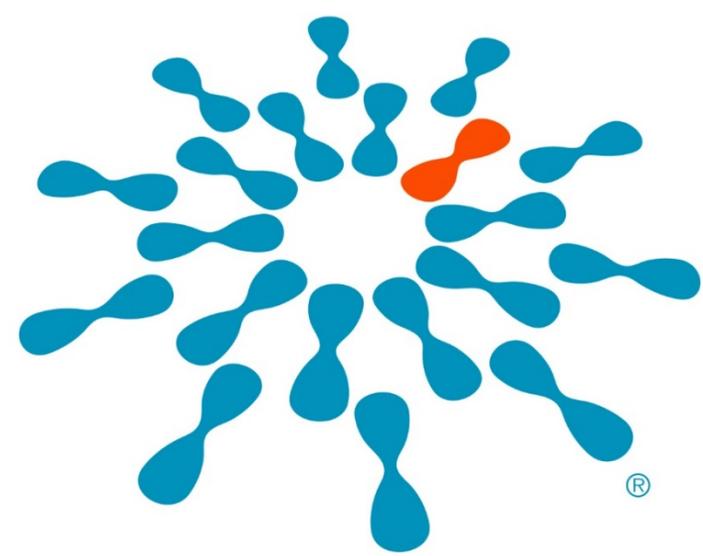


Patient Recruitment, Enrollment, and Retention: The Rare Disease Patient Perspective

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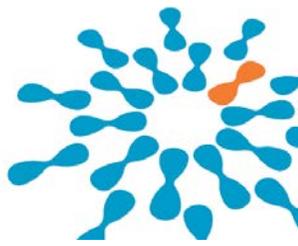
Facts About Rare Diseases



- There are an estimated 7,000 rare diseases
- 1 in 10 or nearly 30 million Americans have one or more rare diseases
- A rare or “orphan” disease in the US affects less than 200,000 people in a year
- Two-thirds of people with rare diseases are children
- 80% of rare diseases have a genetic component
- Orphan drugs are drugs used to treat rare diseases
- Only about 350 diseases have an FDA approved treatment

Experience of Patients

- It can take years for patients to obtain an accurate diagnosis
- There are limited treatment options for most rare diseases
- Lack of knowledge among many medical professionals
- Treatments are generally more expensive than for regular diseases
- Reimbursement issues related to private insurance, Medicare and Medicaid



Our History



In the decade before 1983, only 10 new treatments were brought to market by industry for diseases that today would be defined as rare.

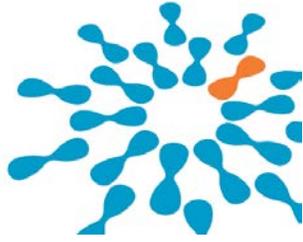
Leaders of rare-disease patient organizations began to realize that there were certain problems their patients and families shared...problems that were common to all people with rare diseases.

A small story in the LA Times led to an episode on a popular TV show, Quincy ME. Then letters began to arrive from people all over the nation who had rare diseases and thought they were alone in their struggles.

The Orphan Drug Act passed in 1983 and the patient leaders who had worked to bring national recognition to the problem founded NORD as an umbrella organization to represent the rare disease community.

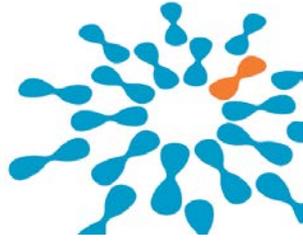
Today, NORD provides information, advocacy, research, and patient services to help all patients and families affected by rare diseases.

Our Mission



The National Organization for Rare Disorders (NORD), a 501(c)(3) organization, is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and service.

Our Vision



NORD's vision and guiding principles on which our advocacy initiatives are based:

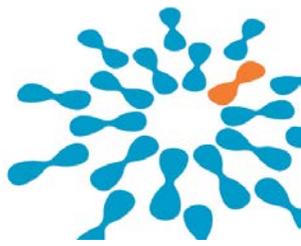
A national awareness and recognition of the challenges faced by people living with rare diseases and the associated costs to society.

A nation where people with rare diseases can secure access to diagnostics and therapies that extend and improve their lives.

A social, political, and financial culture of innovation that supports both the basic and translational research necessary to create diagnostic tests and therapies for all rare disorders.

A regulatory environment that encourages development and timely approval of safe and effective diagnostics and treatments for patients with rare diseases.

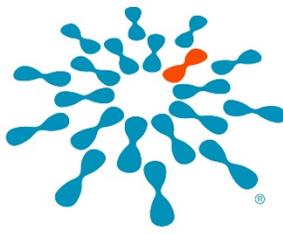
Major NORD Programs and Initiatives



- Policy and regulatory advocacy
- State advocacy and alliance partnerships
- Patient representation (FDA, NIH, SSA)
- Education (patients, professionals, public)
- Mentoring (patient organizations)
- Patient assistance programs
- Patient Networking (disease specific meetings, online communities, creation of new patient organizations)
- Increase disease understanding (Research grants, patient registries)
- International Partnerships
- US Sponsor of International Rare Disease Day



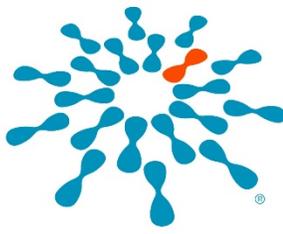
Patient Recruitment, Enrollment, and Retention in Clinical Trials



Unique Challenges in Rare Diseases Clinical Trials

- Small patient populations
 - Sometimes only a few handful of patients can participate
- Dispersed patient populations
 - Genetic conditions unlikely to group geographically
- 2/3rds of rare diseases affect children
- Many diseases manifest heterogeneously
- Many co-morbidities
- Additional financial constraints





Recruitment, Enrollment, and Retention:

- Partnership with Patient Orgs
- Creation and support of patient registries
- Use of social media
- Careful consideration of:
 - Inclusion/exclusion criteria
 - Trial location sites
 - Trial structure
 - Trial duration and time of year
 - Full ecosystem of patient's circumstances



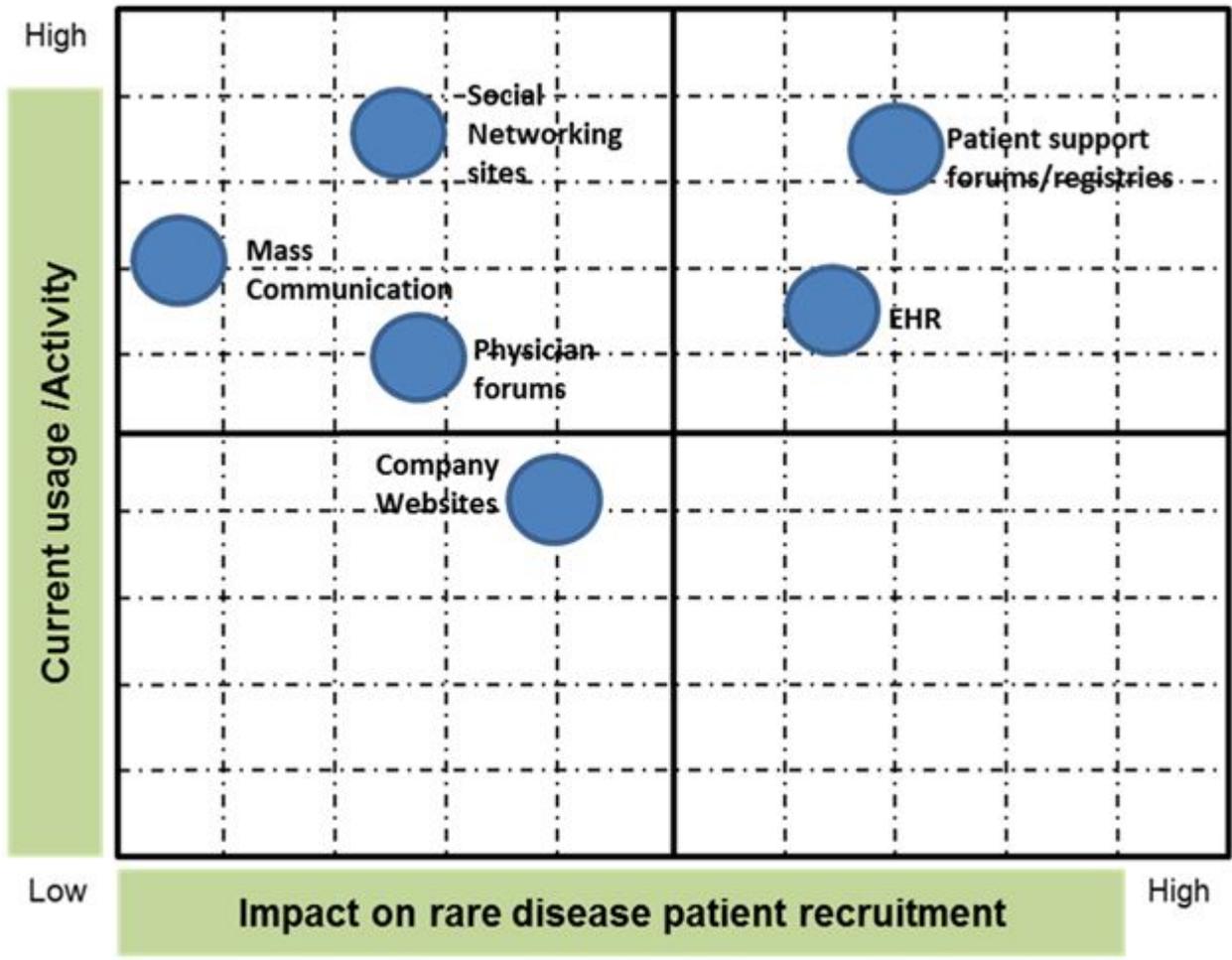
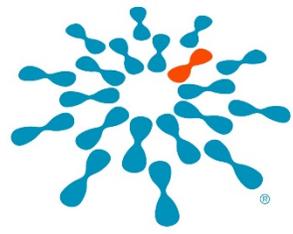


Figure 6; Source: Beroe Analysis



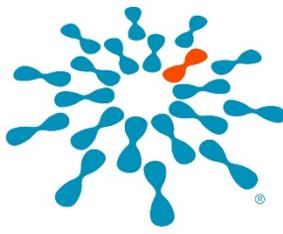


Recruitment, Enrollment, and Retention:



- Support for diagnostics
- Consideration of individuals outside the inclusion criteria
 - Expanded access
- In-home and local clinical trial support
- Creation of travel and lodging assistance
- Provision of psychological and emotional support
- Return of information





Questions?

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