NORD
National Organization for Rare Disorders

A Patient Advocate's View of : Drug Development in Rare Diseases

Diane E. Dorman
Vice President
National Organization for Rare Disorders

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Overview of NORD

- 501(c)3 nonprofit organization, 455 members, 35 FTE's, 3 offices
- Founded in 1983 and responsible for the Orphan Drug Act
- This year we are celebrating 30th anniversaries of ODA and NORD

NORD Today

- NORD is the Voice and Advocate for Patients with Rare Diseases in the US
  - Policy/legislative advocacy, state and federal
  - Medical assistance program support
  - Research support
  - Mentoring of patient organizations “RareLaunch”
  - Connecting patients globally “RareConnect”
  - International coordination with EURORDIS
  - Databases for medical community and patients

Clinical Development is One of Many Challenges Today

- Other challenges for Americans with rare diseases
  - Assuring prompt and early diagnosis
  - Reimbursement for needed treatments
  - Access to treatments and clinical trials
  - Expediting connections with other patients
  - Assuring investment in research/development
  - Expediting disability claims
  - Educating the medical community on rare diseases

Key Issues in Clinical Development – The Patient Perspective

- Flexibility by research community
  - Recognition of
    - Small patient populations
    - Recruitment challenges
    - Lack of natural histories
    - Need for biomarkers
    - Research where there are no therapies

Key Issue

- Flexibility by regulatory system

  NORD-sponsored study found evidence that FDA showed flexibility in 135 non-oncology orphan drugs approvals, 1983-2010

  - ✓ Study published DIA Journal, March 2012. Vol. 46, Number 2, pp. 238-262
  - Need for collaboration across regulatory agencies
  - Need for knowledge of rare diseases within regulatory agencies

Key Issue

- Involvement of patients in risk-benefit decisions
  - NORD initiated project in 2010 to involve patients more aggressively in product specific risk-benefit decisions, from IND to NDA
  - FDA is developing programs to involve patients, we need to enlarge its scope and systematize process

Key Issue

- Continued commitment to rare disease patients in government programs
  - FDASIA included reference to patients with rare diseases
  - But budget cutbacks at FDA and NIH have potential to threaten commitments and focus
  - NORD supports a well-funded FDA and NIH

Key Issue

- Funding by private sector for orphan product development
  - Business experience has demonstrated that orphan product development can be a sound financial investment
  - We must maintain an environment conducive to R&D for orphan products
  - It is critical to enhance ongoing communication among government, industry, investors, payers and patients

Conclusions

- We are on the right track
  - ODA has been successful in providing incentives for orphan product development
  - Industry has shown business models work
  - Involvement of patients has been increasing
  - Medical advances hold great promise for patients with rare diseases
  - We recognize the challenges of clinical development and will collaborate with all audiences to help address them

Thank you