

NORD
National Organization for Rare Disorders

Public Meeting - Reauthorization of the Prescription Drug User Fee Program

July 15th, 2015

Paul Melmeyer
Associate Director of Public Policy



Introduction to NORD and Rare Diseases



Facts About Rare Diseases



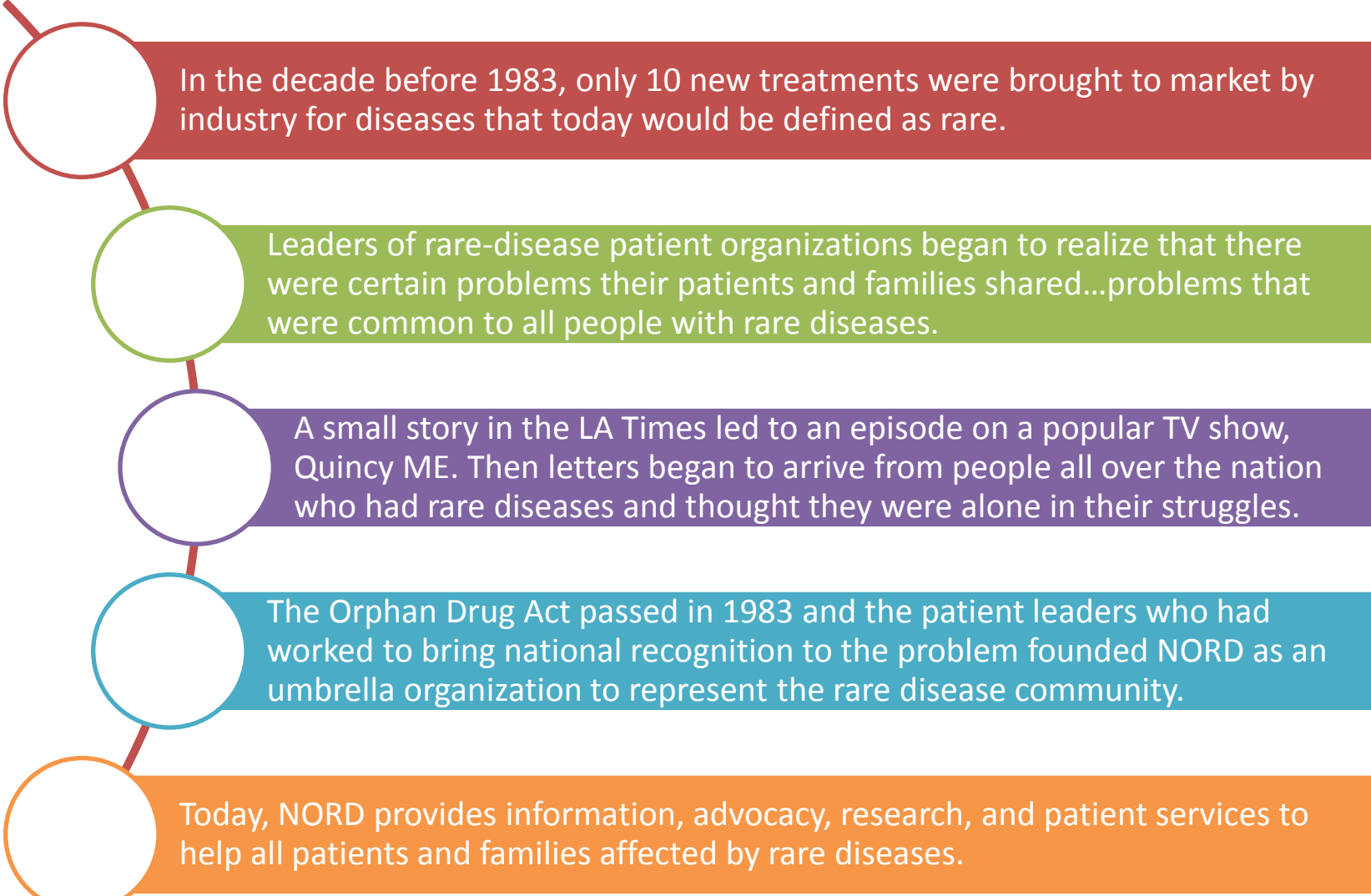
- There are an estimated 7,000 rare diseases
- 1 in 10 or nearly 30 million Americans have one or more rare diseases
- A rare or “orphan” disease in the US affects less than 200,000 people in a year
- Two-thirds of people with rare diseases are children
- 80% of rare diseases have a genetic component
- 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.

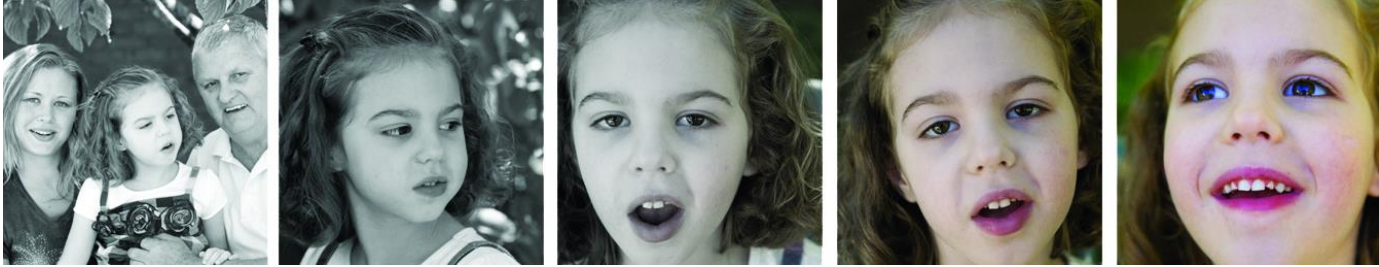
A small story in the LA Times led to an episode on a popular TV show, Quincy ME. Then letters began to arrive from people all over the nation who had rare diseases and thought they were alone in their struggles.

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.



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



Goals for PDUFA VI



Goals for PDUFA VI

- Fund the FDA appropriately in order to ensure safe and expedient review of drugs and biologics



Goals for PDUFA VI

- Fund the FDA appropriately in order to ensure safe and expedient review of drugs and biologics
- Strengthen and incorporate the patient voice throughout the drug development process



Goals for PDUFA VI

- Fund the FDA appropriately in order to ensure safe and expedient review of drugs and biologics
- Strengthen and incorporate the patient voice throughout the drug development process
 - Patient-Focused Drug Development



Goals for PDUFA VI

- Fund the FDA appropriately in order to ensure safe and expedient review of drugs and biologics
- Strengthen and incorporate the patient voice throughout the drug development process
 - Patient-Focused Drug Development
 - Participation on Advisory Committees



Goals for PDUFA VI

- Fund the FDA appropriately in order to ensure safe and expedient review of drugs and biologics
- Strengthen and incorporate the patient voice throughout the drug development process
 - Patient-Focused Drug Development
 - Participation on Advisory Committees
 - Greater coordination across centers on patient involvement



Goals for PDUFA VI

- Fund the FDA appropriately in order to ensure safe and expedient review of drugs and biologics
- Strengthen and incorporate the patient voice throughout the drug development process
 - Patient-Focused Drug Development
 - Participation on Advisory Committees
 - Greater coordination across centers on patient involvement
 - Inclusion of the patient voice throughout the development process, not just the end of the review



Goals for PDUFA VI

- Ensure orphan incentives remain strong
 - Strengthen the Rare Pediatric Disease Priority Review Voucher program



Goals for PDUFA VI

- Ensure orphan incentives remain strong
 - Strengthen the Rare Pediatric Disease Priority Review Voucher program
 - Expand the Orphan Products Grant Program



Goals for PDUFA VI

- Ensure orphan incentives remain strong
 - Strengthen the Rare Pediatric Disease Priority Review Voucher program
 - Expand the Orphan Products Grant Program
- Expand patient access to off-label therapies



Goals for PDUFA VI

- Ensure orphan incentives remain strong
 - Strengthen the Rare Pediatric Disease Priority Review Voucher program
 - Expand the Orphan Products Grant Program
- Expand patient access to off-label therapies
- Promote consistency across review divisions on the use of expedited review pathways as well as biomarkers and surrogate endpoints for rare diseases



Thank You!

pmelmeyer@rarediseases.org

