



NGO COMMITTEE FOR
RARE DISEASES

Tackling Rare Diseases in the United States

Paul Melmeyer

Associate Director of Public Policy

National Organization for Rare Disorders (NORD)

A Committee of **CoNGO**

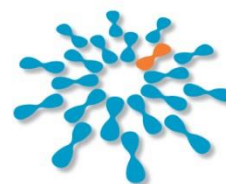


CoNGO
The Conference of NGOs
in Consultative Relationship
with the United Nations



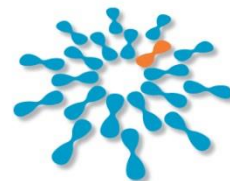
Presentation Outline

- * Where We Were
- * What We Have Accomplished
- * Where We Are Going



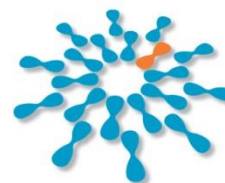
Where We Were

- * Very little research into rare diseases
- * Minimal drug development
- * Many uninsured or underinsured



What We Have Accomplished

- * Expanded Rare Disease Research at the NIH
 - * Human Genome Project
 - * NIH FY 2017 funding for rare diseases: \$3.8 billion



What We Have Accomplished

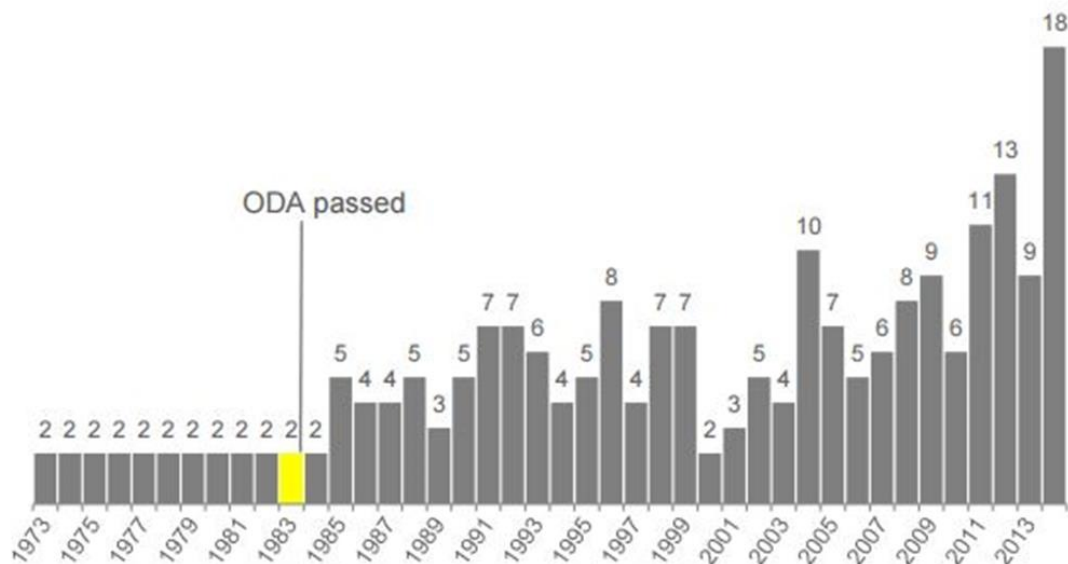
* Incentivized Drug Development in Rare Diseases:

- * Over 550 orphan therapies for about 375 rare diseases
- * 2015 - Almost half of FDA approvals were for rare diseases



What We Have Accomplished

Figure 1. New orphan drugs before and after the ODA



Note: New orphan drugs are defined as NMEs or BLAs. Prior to the ODA, the graph shows the average annual number of approved drugs that would have been considered orphan drugs.

Source: Drug Approval Reports, Food and Drug Administration, various years; EY analysis.



What We Have Accomplished

* Incentivized Development in Rare Diseases:

- * Over 550 orphan therapies for about 375 rare diseases
- * 2015 - Almost half of FDA approvals were for rare diseases
- * Continued FDA flexible review



What We Have Accomplished

Application of Flexible Clinical Development Programs

CDER NME approvals 1/1/2008 – 9/25/2015



Flexible Development Programs	Rare Approvals	Non-Rare Approvals
Use of ≥ 1 flexible development approaches*	81% N=73	36% N=64
Traditional development program**	19% N=17	64% N=113

*Flexible Development approaches are defined as approval supported by other than 2 AWC Studies and/or use of a novel end point

**Traditional Development defined as ≥ 2 AWC studies using endpoints with prior precedents



What We Have Accomplished

* Expanded Insurance Coverage*

* Affordable Care Act:

- * Prohibited discrimination on pre-existing conditions
- * No annual or life-time limits
- * Expanded Medicaid
- * Requires private insurance coverage
- * Many other important provisions



Where We Are Going*



- * Continued expansion of genetic and genomic research
 - * Precision Medicine Initiative
 - * Cancer Moonshot
 - * Gene editing technologies and gene therapies
 - * Undiagnosed Diseases
 - * Translational Research



Where We Are Going*

- * Greater Patient Involvement in Drug Development
 - * Patient-Focused Drug Development
 - * Inclusion of patient-preference data
- * Natural-history data registries
- * Genetically-targeted therapies
- * Targeted Incentives for Rare Disease Drug Development



Where We Are Going*

- * Defense of Insurance Coverage Expansion and Protections
 - * Defend the Affordable Care Act





NGO COMMITTEE FOR
RARE DISEASES

Thank You!

Paul Melmeyer
Associate Director of Public Policy
National Organization for Rare Disorders (NORD)

pmelmeyer@rarediseases.org

A Committee of **CoNGO**



CoNGO
The Conference of NGOs
in Consultative Relationship
with the United Nations

