Path to Progress: Rare Disease Advocacy

Paul Melmeyer
Director of Federal Policy
NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.
NORD’s Rare Community Members

260+ patient groups
3,000+ advocates
200+ medical students
90+ corporate council

NORD’s membership spans across more than 700 diseases, all 50 states and over 30 countries.
A Brief History of Rare Disease Advocacy
Orphan Drug Act

• Enacted in 1983
• Critical incentives for developing orphan drugs
• NORD founded by coalition of advocates who supported the law
Affordable Care Act

Patient Protections:

• Guaranteed Issue
• No Benefit Exclusions
• Community Rating
• Annual out-of-pocket maximum
• No Annual and Lifetime Limits

Medicaid Expansion

Brenda and Jake Neubauer, Credit: Bismark Tribune
21st Century Cures Act

- FDA Review Innovation and Flexibility
- Reauthorization of key incentives
- Funding and Reforms for NIH
ACA Repeal and Replace

Defense of key patient protections

Maintain Medicaid Coverage

Fix the ACA Problems
What’s Next?
What’s Next

• ACA Repeal and Replace
• Orphan Drug Tax Credit
• 21\textsuperscript{st} Century Cures Act and FDARA Implementation
• OPEN Act
• And much more!
OPEN Act

- Incentive for repurposing
- 6 months of additional exclusivity in return for adding a rare disease on to the label of a drug
- Could substantially expand the number of on-label therapies for rare diseases

Credit: Everylife Foundation
How You Can Get Involved
Thank you.

Alone we are rare. Together we are strong.