We have a BEAUTIFUL. & RARE Chance

RARE. STRONG. TOGETHER. 40 YEARS

NORD®

Reimagine a Future Where Every Person With a Rare Disease, and Their Families, Live Their Best Lives.

40 Years of the Orphan Drug Act (ODA)

- Enacted into law in 1983, the ODA created incentives to encourage pharmaceutical companies to develop drugs and biologics to treat rare diseases[1]
  - — 7 years of product exclusivity
  - — 25% tax credit for qualified clinical testing expenses incurred in clinical trials[2,*]
  - — Federal research grants for clinical testing of new therapies to treat and/or diagnose rare diseases
  - — Exemption from FDA marketing application fees[3,†]

Resounding Success With More to Do

- Today, more than 600 treatments have been approved by the FDA for rare diseases, compared to fewer than 40 drugs approved prior to the passage of the ODA

We still need your help:

- 90% of rare diseases do not have an FDA-approved treatment
- The research and regulatory environment must continue to support the ODA and rare disease drug development to ensure a day when all rare disease patients have access to safe and effective treatments

What is an orphan or rare disease?

- In the United States, a rare disease is defined as a condition affecting fewer than 200,000 people
- There are an estimated 7000 known rare diseases
- There are more than 25 million Americans living with a rare disease

Rare diseases you may know:

- ALS (Lou Gehrig's disease)
- Cystic fibrosis
- Sickle cell disease
- Duchenne muscular dystrophy (DMD)
- Hemophilia

...and thousands more you've perhaps never heard before

Hear what people living with rare diseases say about the ODA

"How does anyone live without hope?...As parents, we have to do the very best we can for our kids. We understand the real cost of treating devasting diseases, rare or otherwise, but we live every day with the knowledge that the consequence of doing nothing for children like Taylor is pure and certain death. But doing our best for people like Taylor isn't simply a family matter. It's a responsibility for all of us. We [need] the ODA if we want to protect hope for suffering patients and families. Together we can create outcomes for our children and for future children and families who will face a rare disease."

- Sharon Kane, founder and co-president, Taylor's Tale

"There are a lot of rare diseases like ours that do not have any type of therapies or drugs, and we are still in the disease discovery phase. [Without] this bill...that eliminates the possibility for my child to have a bright and a happy future."

- Andrea Taylor, founder and president, A Twist of Fate

"Without the Orphan Drug Act, we wouldn't have had this [treatment] for Dustin, and the rest of the Gaucher kids would not have it to help retain their lives. I don't think it's fair for any mother to have to watch their son—their baby—bury their baby."

- Julie Potter, grandmother and caregiver to a child with Gaucher disease

*The ODA initially set the orphan drug tax credit at 50%, but this was reduced to 25% as part of the Tax Cuts and Jobs Act of 2017.
†In 1997, Congress created an additional incentive when it granted companies developing orphan products an exemption from "user" fees charged by the FDA as part of the FDA Modernization Act of 1997.

References: 1. Orphan Drug Act, Pub L No. 97-414, 96 Stat 2049 (1983). **2.** Tax Cuts and Jobs Act, Pub L No. 115-97, 131 Stat 2054 (2017). **3.** Food and Drug Administration Modernization Act, Pub L No. 105-115, 111 Stat 2297 (1997).

Join us in our quest to reimagine what's possible and transform lives. Visit **rarediseases.org**

**Alone we are RARE.
Together we are STRONG.®**