

# WHAT IS THE ORPHAN DRUG ACT?

1983

The Orphan Drug Act was enacted in 1983 and became the world's first law to incentivize biopharmaceutical companies to invest in the development of drugs and biologics, known as "orphan drugs," to treat individuals with rare diseases.

A RARE DISEASE IS ANY CONDITION AFFECTING FEWER THAN  
**200,000 AMERICANS**

## HOW DOES THE ORPHAN DRUG ACT WORK?

THERE ARE  
**4**  
INCENTIVES  
in the ODA to encourage  
drug development

**7**  
YEARS  
of market exclusivity

**25%**  
TAX CREDIT  
for clinical testing expenses<sup>1</sup>

**EXEMPTION**  
from FDA marketing  
application fees<sup>2</sup>

**FEDERAL  
RESEARCH  
GRANTS**  
for clinical testing of new  
therapies to diagnose  
and treat rare diseases



Prior to 1983, there were fewer than 40 FDA-approved therapies to treat rare diseases, leaving millions of patients without treatments.<sup>3</sup>



Today, there are 880+ FDA-approved treatments.<sup>4</sup>



More than 95% of rare diseases are still WITHOUT any FDA-approved treatment.<sup>5</sup>

Today, more than 30 million Americans living with a rare disease have new options for treatment and hope of more innovations in the years to come. However, less than 5% of 10,000 known rare diseases have treatments and so we fight on.<sup>6</sup>

References: 1. Orphan Drugs in the United States: An Examination of Patents and Orphan Drug Exclusivity (2021). 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).

4. [ojrd.biomedcentral.com/articles/10.1186/s13023-023-02790-7](https://ojrd.biomedcentral.com/articles/10.1186/s13023-023-02790-7)

5. [ncats.nih.gov/sites/default/files/NCATS\\_RareDiseasesFactSheet.pdf](https://ncats.nih.gov/sites/default/files/NCATS_RareDiseasesFactSheet.pdf)

6. [fda.gov/industry/medical-products-rare-diseases-and-conditions/fda-rare-disease-innovation-hub](https://fda.gov/industry/medical-products-rare-diseases-and-conditions/fda-rare-disease-innovation-hub)

We cannot fight alone – we need your help. Learn how to join us in the fight: [rarediseases.org/get-involved](https://rarediseases.org/get-involved)

