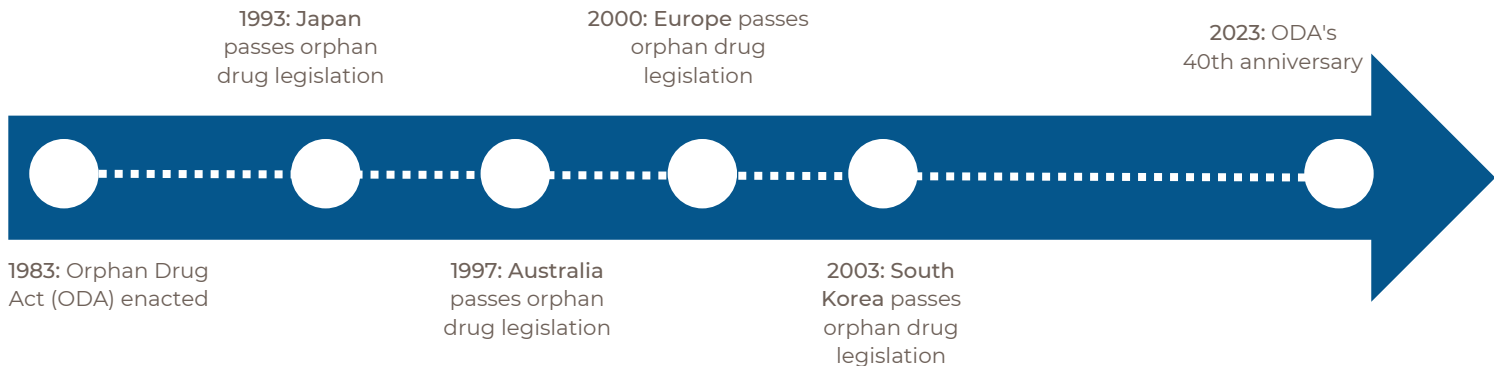


Orphan Drug Act: Celebrating 40 Years



2023 marks the 40th anniversary of the Orphan Drug Act (ODA), a landmark law that has benefited millions of Americans living with rare diseases and has led to orphan drug policies across the globe. The law's incentives have catalyzed rare disease research, leading to scientific breakthroughs resulting in more than 600 safe and effective treatments. Most important, the ODA gives hope to millions of rare disease patients and their families, for whom there are often very few treatment options.



More than 7,000 known rare diseases



More than 90% without treatment



38 rare disease drugs approved pre-ODA



More than 30 million rare disease patients in the U.S.

THE ODA:



Defines a rare disease as affecting 200,000 or fewer people.



Set a framework that led to the approval of >600 orphan drugs since 1983.



Led to more than 1,000 approved indications and more than 6,300 overall orphan drug designations since 1983.

ODA INCENTIVES:



Orphan Drug Tax Credit (ODTC)

Provides a tax credit to offset qualified clinical testing costs.



FDA Application User Fee Waiver

Waives the \$3.1 million application fee required for FDA review.



Orphan Drug Exclusivity

Grants 7 years of market exclusivity.



Research Grants

FDA research grants for clinical trials and natural history studies to advance rare disease product development.