National Ataxia Foundation

FARA Friedreich's Ataxia Research Alliance

# RARE ACT

H.R. 7383 / S. 1214

**Support the Retaining Access and Restoring Exclusivity (RARE) Act**

## Background

The Orphan Drug Act, enacted in 1983, intended to create incentives for drug manufacturers to develop treatments for rare diseases. This act gives the FDA authority to issue orphan drug exclusivity to manufacturers for seven-year periods when they develop a new treatment. The FDA has interpreted this act to provide exclusivity only over the specific use the drug was approved for, and not to mean that a manufacturer holds exclusivity rights over any other specific use of the drug to treat the same disease. In 2021, the FDA was sued by the drug manufacturer Catalyst when another manufacturer was approved to develop the same drug for a separate specified use within the same disease. The *Catalyst* decision sided with the manufacturer to grant exclusivity of the use of the drug over the entire disease for seven years.

## RARE Act

The RARE Act aims to remove ambiguity as to the scope of the orphan drug exclusivity period by rewriting portions of the Federal Food, Drug, and Cosmetic Act to honor the intention of the bill and to codify the FDA's longstanding interpretation of the legislation. The RARE Act clarifies that the exclusivity period is for the same approved use within a rare disease, not just for the same rare disease as a whole.

## Impact

Support for the RARE Act matters to ensure that patient populations are able to access treatments for their rare diseases. Manufacturers continue to be incentivized to get a treatment approved for a specific use or population within a disease, but exclusivity does not prohibit other patient groups or defined uses from being approved at the same time.

## Sponsors

Introduced by Senator Tammy Baldwin (D-WI) and co-sponsored by Senator Mike Braun ((R-IN). Introduced to the House by Representative Doris Matsui (D-CA-7) and cosponsored by Representatives Gus Bilirakis (R-FL-12) and Richard Hudson (R-NC-9).