

U.S. Sen. Baldwin, Colleagues: Introduce bipartisan legislation to preserve access to treatments for rare disease patients

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WASHINGTON, D.C. – Today, U.S. Senators Tammy Baldwin (D-WI) and Bill Cassidy, M.D. (R-LA) introduced bipartisan legislation, the *Retaining Access and Restoring Exclusivity (RARE) Act*, which responds to a 2021 court decision and helps preserve access to treatments for rare disease patients, especially children.

On September 30, 2021, the U.S. Court of Appeals for the 11th Circuit issued its decision in *Catalyst Pharms., Inc. v. Becerra*, and ruled in favor of Catalyst Pharmaceuticals, Inc., turning a decades-long practice by the Food and Drug Administration (FDA) regarding “orphan drug exclusivity” on its head. As a result, FDA was forced to withdraw marketing approval for a drug that provides the only approved treatment option for children with a rare disease called Lambert-Eaton myasthenic syndrome, or LEMS.

“The court’s decision in Catalyst has far-reaching, adverse impacts, especially for children with rare diseases and our bipartisan legislation aims to fix it so that patients aren’t locked out of approved treatments they should have access to,” **said Senator Baldwin.**

“Vague laws can be exploited, resulting in fewer patients getting the treatments they need to survive. This bill clarifies the law to ensure incentives to innovate continue to produce lifesaving cures for our rarest diseases,” **said Dr. Cassidy.**

Because of the 2021 court decision, drug companies are currently incentivized to

seek the broadest orphan drug designation as possible and then focus clinical studies only on the narrowest patient populations that would support approval. In so doing, companies could then rely on the broader designated orphan disease and block approval for any different uses or other patient populations.

The bipartisan *RARE Act* would fix this issue by codifying FDA's longstanding interpretation of the Orphan Drug Act of 1983 to ensure that the scope of the orphan drug exclusivity is clarified to apply only to the same approved use or indication within such rare disease or condition instead of the same disease or condition. This would give FDA the necessary authority to approve the same drug from different manufacturers if they aim to serve different patient populations and combat manufacturers' efforts to take advantage of the Court's ruling in *Catalyst*.

"NORD applauds Senator Baldwin and Senator Cassidy for recognizing the urgent need to safeguard the Orphan Drug Act (ODA) and preserve the original intent of the law, which has benefited millions of Americans with rare diseases and their families," **said Peter L. Saltonstall, President and CEO, National Organization for Rare Disorders.** "This narrowly targeted legislation will simply codify the decades-long interpretation and practice by the FDA to award exclusivity only for the specific approved use of rare disease drugs. Since more than 90% of the 7,000 known rare diseases still have no treatment or cure, it is critical the robust incentives, as envisioned by the ODA, continue to exist so that new orphan drugs are developed into the future."

Baldwin and Cassidy's bipartisan legislation is supported by the National Organization for Rare Disorders and Children's Hospital of Wisconsin.

A backgrounder on the RARE Act can be found [here](#).

An online version of this release is available [here](#).