The Retaining Access and Restoring Exclusivity (RARE) Act

Introduced by Senators Tammy Baldwin and Bill Cassidy, M.D.

Bipartisan legislation that would respond to the 11th Circuit Court's decision in Catalyst Pharms., Inc. v. Becerra and preserve access to treatments for rare disease patients

The Orphan Drug Act (ODA) of 1983 was enacted to provide incentives to support research and development into drugs for rare diseases with small patient populations. The law established a two part process for obtaining orphan drug status: (1) at an early stage in the drug development process, a company can request that FDA "designate" the drug as an orphan drug to prevent, diagnose or treat a rare disease or condition, which allows the company to receive tax credits for the research and clinical testing on the drug; (2) after completing the necessary clinical studies and obtaining FDA approval, the drug is then awarded seven years of "orphan drug exclusivity" that protects the specific use of the drug that is approved.

For example, a drug sponsor could get a drug designated for "treatment of pulmonary arterial hypertension" but ultimately the drug is only FDA approved for use in a smaller patient population (i.e., adults over age 30 with pulmonary arterial hypertension). FDA has long construed the ODA and regulations to mean that the orphan drug exclusivity protects only the *approved use* of the drug, not the rare disease for which the drug was designated. Unfortunately, the 11th Circuit decision would turn this decades-long practice on its head.

Background on 11th Circuit Case: The drug company Catalyst has an approved drug product granted orphan drug designation for its use in *adult patients* with a rare disease called Lambert-Eaton myasthenic syndrome, or LEMS. FDA's regulations have long interpreted the scope of orphan drug exclusivity (ODE) to be limited to the use or indication within the designated rare disease for which the drug is approved, meaning the agency would not block approval of the same drug from a different manufacturer for different uses or indications, even if it sought to treat the same rare disease or condition. A different drug maker, Jacobus, studied the same drug *for use in children,* and provided dosing and administration information for this specific population. Jacobus then applied for and obtained approval of its drug for use only in children with LEMS. In keeping with its longstanding practice, FDA determined Jacobus's drug for children was not blocked by Catalyst's orphan drug exclusivity for their drug for adults. Catalyst then sued FDA, arguing that the scope of ODE should block FDA from approving another sponsor's application for the same drug for all other uses or indications, even though Catalyst's drug was only approved for adults.

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, holding that under the plain language meaning of the Federal Food, Drug, and Cosmetic Act, orphan drug exclusivity blocks approval of the same drug for the entire disease or condition for which the drug is granted orphan-drug designation, regardless of whether the drug was approved and found to be safe and effective only for a narrower use or indication, such as only an adult population.

As a result of this decision, FDA was forced to withdraw marketing approval for the Jacobus drug, the only approved treatment option for children.

What this means: The court's decision in Catalyst has far-reaching, adverse impacts, especially for children with rare diseases. Without a fix, drug companies are 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 Retaining Access and Restoring Exclusivity (RARE) Act would codify FDA's longstanding interpretation of the Orphan Drug Act to ensure that the scope of the orphan drug exclusivity in Sec. 527 of the Food, Drug and Cosmetic Act 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*.

Supported by: National Organization for Rare Disorders, Children's Wisconsin