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Congress Enacts Long-Awaited Fix to Orphan Drug Exclusivity in 2026 Consolidated Appropriations Act

On February 3, 2026, Congress enacted the 2026 Consolidated Appropriations Act (CAA) (HR7148), delivering a significant legislative fix for the pharmaceutical industry. Section 6605 of the CAA, part of the Mikaela Naylon Give Kids a Chance Act, contains the long-anticipated legislative fix to the U.S. Court of Appeals for the Eleventh Circuit's 2021 decision in *Catalyst Pharm., Inc. v. Becerra*. The amendment restores the U.S. Food and Drug Administration's (FDA's) longstanding interpretation of "same disease or condition" related to orphan drug exclusivity and resolves years of regulatory uncertainty and litigation.

The Catalyst Decision and Its Aftermath

The dispute in *Catalyst* centered on the scope of orphan drug exclusivity under the Orphan Drug Act (ODA). The ODA provides that FDA may not approve another application for "the same drug for the same disease or condition" during the exclusivity period. Historically, FDA interpreted this language to mean that exclusivity applied only to the specific "use or indication" for which the orphan-designated drug was approved, not to the entire designated rare disease or condition.

The Eleventh Circuit rejected that interpretation. The court concluded that the statutory phrase "same disease or condition" unambiguously referred to the designated rare disease or condition itself, not to a narrower approved indication within that disease. As a result, the court held that orphan drug exclusivity blocks approval of the same drug for the entire designated disease or condition, even if the competitor sought approval for a different use or indication within that disease. In practical terms, the decision significantly broadened the scope of orphan exclusivity, potentially preventing approval of the same molecule for any other use or indication

within the broader disease category during the initial period of orphan drug exclusivity. For example, the first approval for a disease or condition is often in adults only, and if the same drug for the same disease or condition were for all indications or uses, then additional uses or indications, e.g., pediatric uses, would be discouraged.

Following the *Catalyst* decision, FDA announced in the *Federal Register* that it would apply the ruling narrowly, stating it would "continue to apply its regulations tying the scope of orphan-drug exclusivity to the uses or indications for which a drug is approved" except with respect to the specific product at issue in *Catalyst*. FDA maintained its longstanding interpretation in all other contexts. Unsurprisingly, this approach prompted additional litigation and prolonged uncertainty for sponsors developing therapies for rare diseases. For example, in *Neurelis, Inc. v. Brenner*, the district court held that FDA improperly approved a competing diazepam product (Libervant) for a younger age group during Neurelis's exclusivity period for its own drug (Valtoco), because both drugs treated the same rare disease. The court concluded that FDA's interpretation of orphan drug exclusivity violated the ODA's plain text and the Administrative Procedure Act. Like *Catalyst*, the *Neurelis* decision held that the ODA unambiguously provides broader, "disease-specific" exclusivity covering the entire rare disease, not merely the approved subpopulation. These rulings created an environment that discouraged rare disease research by limiting incentives for companies to study existing orphan-designated drugs in new subpopulations, such as children. Following *Catalyst*, FDA orphan drug approvals declined significantly, from 217 approvals in the 16 months preceding the decision to 95 approvals afterward, suggesting potential ramifications on innovation and investment in rare disease development. Critics also argued that the rulings ignored scientific realities, particularly that children respond differently to diseases and treatments, and conflicted with congressional efforts to promote pediatric drug research.

Section 6605: The Legislative Fix

Section 6605 of the CAA squarely addresses the issue. The amendment replaces the statutory phrase "same disease or condition" with "same approved use or indication within such rare disease or condition." This change makes explicit what FDA had long

maintained: orphan drug exclusivity blocks approval of the same drug only for the same approved use or indication, not for all uses within the broader designated disease. The amendment also applies retroactively. The legislation states that the revised provision governs "regardless of the date on which the drug was so designated, and regardless of the date on which the drug was approved."

Practical Implications

The retroactive nature of the amendment carries meaningful operational consequences. FDA will now need to review its database of active orphan drug exclusivities to determine the precise scope of each exclusivity under the amended statute. Considering the substantial number of drugs currently benefiting from orphan exclusivity, this undertaking may take time.

For industry stakeholders, however, the amendment restores predictability. Sponsors developing therapies for rare diseases can once again rely on the principle that orphan exclusivity is indication or use-specific. This clarification may also encourage continued development of new indications within rare disease categories, particularly where multiple sponsors are investigating different uses of the same molecule.

A Win for Development of Additional Orphan Uses and Indications

The legislative fix marks the culmination of years of advocacy by FDA, which had consistently urged Congress to clarify the statute following the *Catalyst* decision. By codifying the agency's interpretation, Congress has resolved the circuit-level disruption and aligned the statute with FDA's longstanding regulatory framework.

For FDA, the amendment represents both a policy victory and a restoration of regulatory consistency. For sponsors, it brings long-awaited clarity. And for the broader rare disease community, it reestablishes a balance between protecting innovation and allowing development of additional therapies within the same rare disease space. As advocates to develop orphan drug indications, such as the National Organization for Rare Disorders (NORD), have indicated, this legislation necessary to preserve patient access to safe and effective treatments for distinct rare disease populations while ensuring continued incentives for innovation and

development of new rare disease therapies.

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