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Better Late Than Never? FDA Receives Long-Awaited Statutory Fix for Orphan Drug Exclusivity in Recent Appropriations Act

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The Consolidated Appropriations Act, signed into law on February 3, 2026, avoided another government shutdown. But notably for the US Food and Drug Administration (FDA) and drug developers, it also codified FDA's long-standing approach toward the scope of orphan drug exclusivity that had become a subject of litigation in recent years.

The Orphan Drug Act of 1983 was intended to incentivize the development of drugs for rare diseases. Among these incentives, perhaps the most sought after is the seven-year period of exclusivity.[1] This exclusivity precludes FDA from approving another drug with the same active ingredient for the orphan disease during the exclusivity period. While the text of the Orphan Drug Act previously precluded FDA from approving another application for the same drug for the "same disease or condition," FDA has historically narrowed the scope of orphan drug exclusivity to the use or indication for which it was approved.

Though courts have reached a different conclusion based on the plain meaning of the statute, FDA has justified this interpretation as consistent with the purpose of the Orphan Drug Act, because it incentivized sponsors to develop drugs for all persons affected by a rare disease or condition, even after approval of a drug for an indication that only affected certain subsets within a rare disease patient population.[2] The agency's long-standing approach toward orphan drug exclusivity meant that FDA could approve a later-in-time application for the "same drug" if it approved the later-in-time drug for a specific indication that differed from that of the approved orphan drug despite the approved drug's seven-year orphan drug exclusivity.

Catalyst and *Neurelis* decisions

In recent years, FDA faced two challenges to its approach toward orphan drug exclusivity. In both cases, the courts have held that this approach is a violation of the "clear command" of the Orphan Drug Act that ties the scope of exclusivity to the disease or condition for which the drug received orphan drug designation.[3]

In *Catalyst Pharms., Inc. v. Becerra,*[4] the manufacturers of the amifampridine drug Firdapse challenged FDA's award of orphan drug exclusivity to Ruzurgi. Firdapse was approved and awarded orphan drug exclusivity for Lambert-Eaton myasthenic syndrome (LEMS) in adults. During the seven-year exclusivity period for Firdapse, FDA granted approval and a separate period of orphan drug exclusivity to Jacobus' Ruzurgi, which also contained the active ingredient amifampridine, for treatment of LEMS in patients ages 6 to 17.

Catalyst, Firdapse's sponsor, sued FDA in the US District Court for the Southern District of Florida for its approval decision given the orphan drug exclusivity attached to Firdapse. The district court, applying the *Chevron* doctrine, determined that the language "same disease or condition" was ambiguous, and that Catalyst presented no authority that called into question FDA's interpretation of the phrase.[5] But the US Court of Appeals for the 11th Circuit rejected FDA's interpretation of the Orphan Drug Act, noting that Congress could have written the law to tie the scope of exclusivity to the specific use or indication for which the drug was approved but chose not to use those terms.[6] Following the 11th Circuit's decision, FDA published an announcement explaining that while the agency complied with the court's order in *Catalyst* to set aside Ruzurgi's approval, it intended to continue to apply its regulations and tie the scope of orphan drug exclusivity to the use or indication of the approved drug for decisions beyond the scope of *Catalyst*.[7]

It was not long before FDA again found its interpretation of orphan drug exclusivity challenged. In August 2022, FDA approved Libervant, a buccal film formulation of diazepam, for treatment of acute repetitive seizures in

patients ages 2 to 5. The following year, Neurelis, whose diazepam nasal spray Valtoco had previously been approved and received orphan drug exclusivity for treatment of acute repetitive seizures in adults, submitted a supplemental New Drug Application (NDA) seeking approval for this indication in patients ages 2 to 5.

FDA granted tentative approval for this indication due to Libervant's exclusivity in this patient population. In the subsequent litigation, *Neurelis Inc. v. Califf,* the US District Court for the District of Columbia determined that FDA's orphan drug exclusivity approval of Libervant violated the Orphan Drug Act, adopting the 11th Circuit's opinion in *Catalyst*, finding that the text of the Orphan Drug Act ties the scope of exclusivity to the orphan drug designation, and that the Orphan Drug Act is "clear" and decisive" in this regard.[8]

The court also rejected novel arguments made by Libervant manufacturer Aquestive and FDA in support of FDA's interpretation of the scope of orphan drug exclusivity. The court found Aquestive's reliance on the word "application" in 21 USC §360cc as evidence of congressional intent to tie exclusivity to the scope of approval to be misplaced. The court also rejected arguments that Congress' lack of action to update the scope of orphan drug exclusivity constituted acquiescence to FDA's interpretation, finding that there was simply not enough evidence of such acquiescence.

Congress adopts FDA's interpretation of orphan drug exclusivity

On February 3, 2026, FDA received the congressional fix it had been waiting for. Section 6605 of the Consolidated Appropriations Act of 2026 amends 21 USC 360cc, which addresses the exclusivity award that attaches upon approval of a drug designated for a rare disease or condition. Significantly, the new legislation replaces the phrase "same disease or condition" with "same approved use or indication within such rare disease or indication." It also adds a definition for "approved use or indication" to mean the use or indication FDA approves in a submitted NDA or Biologics License Application for an orphan-designated drug. Congress also added an additional subsection to the statute to clarify that these amendments to the statute apply to any orphan-designated drug, regardless of the date on which it was designated or the date of approval.

This is not the first time Congress has instituted a legislative fix to seemingly endorse FDA's interpretation of the Orphan Drug Act. Following the decisions in *Depomed, Inc. v. HHS*[9] and *Eagle Pharm., Inc. v. Azar*,[10] where the courts rejected FDA's "clinical superiority" requirement for granting exclusivity to a subsequent drug that is the "same drug" for the "same disease or condition," Congress amended the Orphan Drug Act in 2017. These amendments updated §360cc(a), replacing "such drug" with "same drug" and codifying the clinical superiority requirement, which requires a sponsor of a drug otherwise the same as a previously approved drug for the same disease or condition to demonstrate clinical superiority to the approved drug in order to obtain a period of orphan drug exclusivity. While these changes have not completely warded off legal challenges to FDA's clinical superiority determinations, courts have upheld the clinical superiority requirement, finding that the 2017 amendments constituted congressional endorsement of this requirement.[11]

What does this change mean for sponsors?

While Congress amended 21 USC 360cc, it left 21 USC 360bb, which addresses orphan drug designation, as is. Orphan drug designation still applies to the entire disease or condition. Critically, this avoids any tension with existing FDA regulations regarding orphan drug designation requests, which require a sponsor seeking orphan drug designation for a drug that is the same drug as an already approved drug for the same rare disease or indication to present a plausible hypothesis that its drug is clinically superior to the first. Thus, while these statutory changes could lead to more orphan drug exclusivity awards following approvals within the same orphan disease –particularly in cases where multiple sponsors currently hold orphan designation for the same drug – they may not necessarily lead to a flood of new orphan drug designation requests, particularly for previously approved drugs.

That said, current sponsors should reconsider their regulatory strategy in light of these amendments. For example, sponsors that previously planned their submission strategy based on orphan drug exclusivity that extended to the whole rare disease population will want to consider how these amendments may narrow any existing or planned exclusivity. Alternatively, this statutory change may present an opportunity for approval where another previously approved drug has orphan drug exclusivity even without comparative data

demonstrating clinical superiority.

We are also interested to see how this statutory change may impact label negotiations, now that FDA is on surer footing for its statutory interpretation. We would anticipate that FDA will favor more specific labels during the approval process, which would pave the way for the agency to approve more drugs to treat rare diseases based on these amendments, particularly given their retroactive applicability.

Cooley lawyers are available to discuss how this latest update to the orphan drug provisions of the Federal Food, Drug, and Cosmetic Act may impact your regulatory strategy. Feel free to reach out to any of the attorneys listed below.

Notes

1. This exclusivity is different from patents, which grant the inventor exclusive rights to their invention during a 20-year patent term. Exclusivity granted by FDA provides for delays and prohibitions on the approval of competitor drugs that attach upon FDA approval of the drug. Some exclusivity periods, such as seven-year orphan drug exclusivity, run parallel to patent protections, while six-month pediatric exclusivity, attaches to the end of existing patents and exclusivities, but only extends FDA protections against competitor approvals.
2. 76 Fed. Reg. 202, 64871 (October 19, 2011).
3. *Neurelis Inc. v. Califf*, No. 24-1576 (D.D.C., Feb. 14, 2025).
4. 14 F.4th 1299, (11th Cir. 2021).
5. *Catalyst* at 1306.
6. Id. at 1309.
7. US Food and Drug Administration, FDA's Overview of *Catalyst Pharms., Inc. v. Becerra*, (last updated Jan. 23, 2023).
8. *Neurelis Inc.*, No. 24-1576 at 16.
9. 66 F.Supp.3d 217 (D. D.C. 2014).
10. 952 F.3d 323 (D.C. Cir. 2020).
11. *Jazz Pharmaceuticals, Inc. v. Kennedy*, No. 24-5262 (D.C. Cir. 2025).

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