

UPDATES

Key Inflation Reduction Act Amendment Broadens U.S. Protection for Orphan Drugs

July 15, 2025

On July 4, 2025, President Donald Trump signed into law the One Big Beautiful Bill Act (OBBBA), a sweeping piece of legislation with far-reaching implications for the healthcare industry. Among its numerous provisions was a significant, but easily overlooked, amendment expanding the Orphan Drug Exclusion under the Inflation Reduction Act (IRA) Medicare Drug Price Negotiation Program.

Background: IRA's Original "Single Orphan Indication" Exclusion

As originally enacted, the IRA (signed into law on August 16, 2022) excluded from the Negotiation Program orphan drugs and biologics but only if the product was indicated to treat a single rare disease or condition. Specifically, the exclusion was limited to drugs and biologics designated "for *only one rare disease or condition* under section 526 of the Federal Food, Drug, and Cosmetic Act and for which *the only approved indication (or indications) is for such disease or condition*." If a product lost its Orphan Drug Exclusion status, then, under [implementing guidance](#), the Centers for Medicare & Medicaid Services (CMS) used the date of first approval or licensure, not the date of the first non-orphan indication, to determine when the product would first be eligible for negotiation.

This narrow exclusion (sometimes referred to as the Single Orphan Indication Exclusion) was of significant concern to patient and provider groups, in addition to manufacturers, because it operated to discourage innovation for rare diseases.

Expansion Under the One Big Beautiful Bill Act

The OBBBA substantially broadens the scope of the Orphan Drug Exclusion and delays eligibility under the Negotiation Program for orphan drugs and biologics that are later approved for non-orphan indications.

In particular, the OBBBA:

1. amends the IRA to exclude from the Negotiation Program any drug or biologic "that is designated ... for *one or more rare diseases or conditions* under section 360bb of title 21 and for which *the only approved indication (or indications) is for one or more such rare diseases or conditions* (as such term is defined in section 526(a)(2) of the Federal Food, Drug, and

Cosmetic Act),” and

2. clarifies that, with respect to a drug or biologic that loses Orphan Drug Exclusion status, the IRA’s price negotiation eligibility period begins “the first day after” the date of non-orphan approval (for drugs) or non-orphan licensure (for biologics), rather than the original approval date.

These amendments bring about two key changes:

First, products with more than one orphan designation and more than one approved indication will now remain exempt from price negotiation, so long as each approved indication is for a rare disease or condition.

Second, for drugs or biologics with orphan designation(s), the Negotiation Program’s eligibility period will begin only when the product is approved for a *non-orphan* indication, thereby delaying the applicability of price negotiation, potentially significantly, for those products that lose Orphan Drug Exclusion status through the approval of a non-orphan indication.

Looking Ahead

Manufacturers with current or anticipated orphan-designated products should consider — and possibly reevaluate — their development pipelines and pricing analyses in light of this expanded exclusion.

Sidley is closely tracking implementation of the OBBBA and its implications across the pharmaceutical industry. For further guidance on how these changes may affect your products or portfolio strategy, please contact a member of our team.

CONTACTS

If you have any questions regarding this Sidley Update, please contact the Sidley lawyer with whom you usually work, or

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