



Biotechnology Innovation Organization

1201 New York Ave, NW
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February 26, 2024

The Honorable Mike Johnson
Speaker of the House
U.S. House of Representatives
Washington, DC 20515

The Honorable Charles Schumer
Majority Leader
U.S. Senate
Washington, DC 20510

The Honorable Hakeem Jeffries
Democratic Leader
U.S. House of Representatives
Washington, DC 20515

The Honorable Mitch McConnell
Republican Leader
U.S. Senate
Washington, DC 20510

We are writing to express our strong support for the bipartisan Optimizing Research Progress Hope and New Cures (ORPHAN Cures Act). The legislation, which was introduced by Reps. John Joyce (R-PA) and Wiley Nickel (D-NC) in September,¹ corrects a significant flaw in the Inflation Reduction Act (IRA) concerning medicines for rare diseases. Senators Tom Carper (D-DE) and John Barrasso, M.D., (R-WY) introduced the Senate companion to the legislation.

Each of our firms is committed to finding and developing therapies for rare diseases -- conditions which, in many cases, lack any effective treatments. That task is challenging under the best of circumstances. The IRA added to the difficulties of creating these life-saving medicines.

The ORPHAN Cures Act removes this harmful feature from the IRA, clearing the way for much-needed progress in treating a wide range of rare diseases.

A rare disease is one that afflicts fewer than 200,000 American patients² -- or fewer than 40,000 in the case of rare cancers.³ But while the patient population for any individual rare disease is small, the combined effect of these conditions is anything but.

Right now, more than 30 million Americans suffer from some form of rare disease.⁴ And by one estimate, rare forms of cancer account for a quarter of all cancer deaths each year.⁵

¹ <https://www.congress.gov/118/bills/hr5539/BILLS-118hr5539ih.pdf>

² <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6174191/>

³ <https://www.cancer.gov/pediatric-adult-rare-tumor/rare-tumors/about-rare-cancers>

⁴ <https://www.fda.gov/patients/rare-diseases-fda>

⁵ <https://www.cancer.gov/pediatric-adult-rare-tumor/rare-tumors/about-rare-cancers>

Because the market for treatments for these diseases is by definition quite small, Congress enacted the Orphan Drug Act in 1983 to incentivize investment in them. While the Orphan Drug Act was a landmark achievement that led to numerous medical breakthroughs, only 5% of rare conditions today have treatments approved by the Food and Drug Administration (FDA).⁶ Our companies are poised to improve on that.

To their credit, lawmakers recognized the challenge the IRA's pricing system posed to orphan drug development. They sought to preserve the existing incentive system by exempting orphan drugs from price-setting.

In doing so, however, they introduced a policy error. Orphan drugs are eligible for the exemption provided they are approved for one rare disease only. If approved to treat a second medical condition, they lose their exemption and become subject to the IRA's pricing system.⁷

In effect, the IRA creates a strong financial disincentive against companies like ours investigating whether an orphan drug might have multiple uses -- as they often do.

If this provision remains in place, investigation into the efficacy of new treatments for a wide range of rare diseases will decline, leaving potentially life-saving applications unexplored. Quite simply, the financial "reward" for investment in such investigations, if successful, could actually be negative -- due to the loss of the exemption from the IRA price-setting procedure.

The ORPHAN Cures Act undoes this damaging provision, removing the impediment to full investigation of each new treatment by retaining the exemption for treatments effective against more than one rare disease.

Beating back the threat of cancer -- including rare cancers -- remains one of humanity's most urgent and difficult challenges. And initiatives like President Joe Biden's Cancer Moonshot⁸ have given much-needed support to the search for new treatments. The IRA shouldn't stand in the way of that mission.

Major legislation is never perfect and often requires changes. The ORPHAN Cures Act is a chance to correct this mistake, and to ensure that successful new treatments reach all patients who will benefit from them. We urge you to advance this important bipartisan IRA change to restore hope to Americans living with rare diseases.

Sincerely,

Ted Love, M.D., Chairman
Biotechnology Innovation Organization (BIO)

Rachel King, Interim CEO
Biotechnology Innovation Organization (BIO)

⁶ <https://ncats.nih.gov/news-events/news/rare-disease-day-at-nih-2023-putting-hope-into-action>

⁷ <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC10427936/#:~:text=The%20IRA%20exempts%20orphan%20drugs,with%20a%20single%20ap%20proved%20indication.>

⁸ <https://www.whitehouse.gov/cancermoonshot/>



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