



December 7, 2023

The Honorable Chuck Schumer
Majority Leader
United States Senate
Room S-221, The Capitol
Washington, DC 20510

The Honorable Mitch McConnell
Republican Leader
United States Senate
Room S-230, The Capitol
Washington, DC 20510

The Honorable Mike Johnson
Speaker of the House
U.S. House of Representatives
Room H-305, The Capitol
Washington, DC 20510

The Honorable Hakeem Jeffries
Democratic Leader
U.S. House of Representatives
Room S-230, The Capitol
Washington, DC 20510

Dear Majority Leader Schumer, Republican Leader McConnell, Speaker Johnson, and Democratic Leader Jeffries:

The Council of State Bioscience Associations (CSBA) is a coalition of independent state and territory-based non-profit trade associations, each of which advocates for public policies that support responsible development and delivery of innovative life-sustaining and life-saving biotechnology solutions. Convened by the Biotechnology Innovation Organization (BIO), CSBA's collective voice represents the true grassroots network of innovators, researchers, manufacturers, and accelerators across the country. According to a recent industry report, U.S. bioscience industry employment in 2021 reached 2.1 million jobs in more than 127,000 businesses across every state in the U.S. and Puerto Rico. The total economic impact of the bioscience industry on the U.S. economy, as measured by overall output, totaled \$2.9 trillion dollars in 2021.¹ (TEconomy/BIO, 2022)

The majority of CSBA's member companies are research-intensive biotechnology companies working on cutting-edge innovations. Their pipelines have the potential to benefit millions of patients suffering from diseases for which there are no cures or treatments.

We are writing to urge your support for S. 3131 and H.R. 5539, the Optimizing Research Progress Hope and New Cures Act (ORPHAN Cures Act). This bipartisan and bicameral legislation is aimed at boosting rare disease drug development to ensure that patients have access to innovative therapies. Orphan drugs are drugs that target rare diseases, which are defined as diseases that

¹ TEconomy/Biotechnology Innovation Organization. (2022). *The U.S. Bioscience Industry: Fostering Innovation and Driving America's Economy Forward*. <https://www.bio.org/csba-resources-and-reports>

affect fewer than 200,000 people in the United States. Currently, fewer than 5 percent of rare diseases have an FDA-approved treatment. Because orphan drugs benefit small patient populations, incentives to invest in these treatments are limited due to the inherent risk of such investments.

The Inflation Reduction Act (IRA) that was enacted into law currently exempts orphan drugs from government price negotiations – but only if the drugs are approved for a single indication. This ultimately means drug manufacturers are disincentivized to test whether an orphan product can treat other indications. If enacted, the ORPHAN Cures Act would support existing incentives and boost research into new treatments by incentivizing research in follow on indications; thus bringing hope to the 30 million Americans currently suffering from any of more than 7,000 rare diseases. Additionally, the bill would incentivize critical follow-on investment into rare disease drug development.

We urge you to support the bipartisan, bicameral ORPHAN Cures Act and encourage your members to support innovation for patients with rare diseases. On behalf of the innovators working every day to address unmet patient needs, we thank you for your support and leadership in preserving the possibility that novel therapies can be developed for the millions of patients suffering from diseases for which there are no cures or treatments.

Please contact CSBA Executive Director, Michele Oshman at moshman@bio.org with any questions.

Sincerely,



Michele M. Oshman
Executive Director, Council of State Bioscience Associations
Vice President, External Affairs
Biotechnology Innovation Organization