Rare Disease Company Coalition

BY ELECTRONIC DELIVERY

September 16, 2024

The Honorable Ron Wyden
Chair, United States Senate Committee on Finance
221 Dirksen Senate Office Building
Washington, DC 20002

The Honorable Mike Crapo
Ranking Member, United States Senate Committee on Finance
239 Dirksen Senate Office Building
Washington, DC 20510

RE: RDCC Supports S.3131/H.R. 5539, the ORPHAN Cures Act

Dear Chair Wyden and Ranking Member Crapo,

The Rare Disease Company Coalition (RDCC) is proud to support the bipartisan and bicameral Optimizing Research Progress Hope And New Cures (ORPHAN Cures) Act (S. 3131/H.R. 5539).

This critical legislation seeks to ensure biopharmaceutical companies are able to continue to meet the clear and urgent need for continued innovation in rare disease research & development (R&D).

The RDCC represents innovative life science companies committed to discovering, developing, and delivering treatments for the 1 in 10 Americans living with a rare disease.[1] Collectively, RDCC members invest over $17 billion in R&D annually; have brought over 50 treatments to market; and are presently working on more than 200 rare disease development programs, many of which will be first-to-market therapies if approved.[2] On average, RDCC members invest nearly 60 percent of their annual revenue back into R&D.[3]

Rare disease in the United States is defined as a condition that affects fewer than 200,000 people.[4] There are 10,000 identified rare diseases that impact an estimated 25 to 30 million Americans.[5] These diseases are often devastating and life-threatening: 80 percent of rare diseases are genetic in origin, 50 percent impact children, and 30 percent of those children will not live to see their fifth birthday.[6] With 95% of rare diseases without a Food and Drug Administration (FDA)-approved treatment option, we are urgently working to meet the needs of

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[1] NAT'L CENTER FOR ADVANCING TRANSLATIONAL SCIENCES, GENETIC AND RARE DISEASES INFORMATION CENTER, ABOUT GARD (Dec. 5, 2024), https://rarediseases.info.nih.gov/about.
[2] RARE DISEASE COMPANY COALITION, https://www.rarecoalition.com/.
[3] *Id.*
[4] GARD, *supra* note 1.
[5] *Id.*
[6] RARE DISEASES AND ORPHAN PRODUCTS: ACCELERATING RESEARCH AND DEVELOPMENT (Marilyn J. Field & Thomas F. Boat eds., 2010), https://www.ncbi.nlm.nih.gov/books/NBK56189/.

patients and their caregivers with currently limited or no treatment options.[7]

Congress has long recognized the importance of investment in rare disease drug development, starting with the Orphan Drug Act (ODA) of 1983. Indeed, the FDA has also long recognized that "orphan drugs are desperately needed by patients with rare diseases,"[8] while also acknowledging that "[d]rug, biologic, and device development in rare diseases is challenging for many reasons."[9] It is vital that Congress continue to preserve and strengthen the critical incentive system that allows rare disease companies to continue discovering, developing, and delivering rare disease treatments in the United States. And the ORPHAN Cures Act is essential to protect innovation for rare disease drug development.

Currently, the Inflation Reduction Act's (IRA) Orphan Drug Exclusion (ODE) offers a price negotiation exemption for orphan drugs that are designated for only one rare disease or condition and for which the only approved indication is for such disease or condition.[10] By limiting the exclusion to drugs that are designated and approved to treat only one condition, the IRA discourages rare disease companies from further exploring promising research that could lead to additional treatment options.

1 in 5 orphan drugs are FDA-approved for more than one use, and 60% of those second indications are for another rare disease.[11] According to a Health Capital Group study, limiting the average treatment to just one orphan indication would reduce the number of expected patients treated by at least 24%.[12] For RDCC member companies that invest over half of their annual revenues back into R&D, improving the ODE by removing the one-use limitation will have a significant impact in delivering desperately-needed therapies and hope to underserved patient and caregiver communities.

We believe this legislation will allow biopharmaceutical companies to meet the clear and urgent need for continued innovation in rare disease R&D while preserving the intent of the IRA.

If you have any questions or would like to discuss further, please contact me at stacey@rarecoalition.com.

Sincerely,

Stacey Frisk
Executive Director
Rare Disease Company Coalition

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[7] NIH NATIONAL HUMAN GENOME RESEARCH INSTITUTE. RARE GENETIC DISEASES, https://www.genome.gov/dna-day/15-ways/rare-genetic- diseases.
[8] U.S. FOOD & DRUG ADMIN., CENTER FOR DRUG EVALUATION & RESEARCH, SMALL BUS. CHRONICLES (July 13, 2023), https://www.fda.gov/media/83372/download.
[9] U.S. FOOD & DRUG ADMIN., RARE DISEASES AT FDA (Dec. 13, 2022), https://www.fda.gov/patients/rare-diseases-fda.
[10] 42 U.S.C. § 1192(e)(3)(A).
[11] National Organization for Rare Disorders, Orphan Drugs in the United States: Rare Disease Innovation and Cost Trends THROUGH 2019 (Dec. 2020), https://rarediseases.org/wp-content/uploads/2021/03/orphan-drugs-in-the-united-states-NRD-2020.pdf.
[12] Health Capital Group, Rare Disease Companies in the Public Markets: Challenging PERFORMANCE AGAINST A BACKDROP OF POLICY UNCERTAINTY (Oct. 2023), https://www.healthcapitalgroup.com/rdcc