September 9, 2024

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]

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. 4583 and H.R. 7384, the Creating Hope Reauthorization Act. This bipartisan and bicameral legislation would allow for a continued path forward to additional innovative therapies for rare disease patients by reauthorizing the current Food and Drug Administration (FDA) Rare Pediatric Disease (RPD) Priority Review Voucher (PRV) program, which is set to expire on September 30, 2024.

[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

Rare diseases affect a significant number of individuals, with nearly half of those affected being children.[2] While treatments exist, options remain limited due to high cost of development and utilization by a smaller section of the patient population. To incentivize more therapies, the Food and Drug Administration Safety and Innovation Act (FDASIA) of 2012 created the RPD PRV program. Specifically, a drug developer who receives approval for a drug or biological product for a rare pediatric disease may qualify, if it meets certain criteria, for a voucher that can be redeemed to receive priority review for a different product. In addition, drug makers can also fund current and future research by selling the voucher to another company. As a result, more targeted therapies have the potential to come to market at an earlier time.

Since the start of the RPD PRV program, 53 vouchers for 39 rare pediatric diseases have been awarded which have led to innovations benefitting over 200,000 vulnerable patients. 36 of those rare pediatric diseases had no previously approved therapies on the market at the time of approval. Furthermore, this program has been reauthorized with bipartisan support twice since its inception, both in 2016 and 2020.

We urge your timely support in reauthorizing the bipartisan, bicameral Creating Hope Reauthorization Act before it expires on September 30, 2024. A lapse in this program will negatively impact some of the most vulnerable patients suffering from diseases for which there are no cures or treatments.

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 of these type of novel therapies.

Please contact CSBA Executive Director, Patrick Plues at pplues@bio.org with any questions.

Sincerely,

AR - BIOArkansas
AZ - Arizona Bioindustry Association, Inc. (AZBio)
CA - California Life Sciences
CA - Biocom California
CA - Southern California Biomedical Council
CO - Colorado BioScience Association
CT - BioCT
DE - Delaware BioScience Association
FL - BioFlorida
GA - Georgia Bio
IA - Iowa Bio
ID - Idaho Technology Council
IL - Illinois Biotechnology Innovation Organization (iBIO)
IN - Indiana Health Industry Forum
KS - BioKansas
KY - Kentucky Life Sciences Council

[2] Government Accountability Office. (2021, October). *RARE DISEASES Although Limited, Available Evidence Suggests Medical and Other Costs Can Be Substantial.* https://www.gao.gov/assets/gao-22- 104235.pdf

LA - Louisiana BIO
MA - MassBIO
MD - Maryland Tech Council
ME - Bioscience Association of Maine
MI - Michigan Biosciences Industry Association (MichBio)
MN - Medical Alley
MO - Missouri Biotechnology Association (MOBIO)
MT - Montana Bioscience Association
NC - North Carolina Life Sciences Organization
ND - Bioscience Association of North Dakota
NE - Bio Nebraska
NH – New Hampshire Life Sciences
NJ - BioNJ
NM - New Mexico Biotechnology & Biomedical Association
NV - Nevada Biotechnology & Health Science Consortium
NY - NewYorkBIO
OH - Ohio Life Sciences
OK - Life Science Oklahoma
OR - Oregon Bioscience Association
PA - Life Sciences Pennsylvania
PR - INDUNIV Research Center, Inc. / BioAlliance Puerto Rico
RI - Rhode Island Bio
SC - South Carolina BIO
SD - South Dakota Biotech Association
TN - Life Science Tennessee
TX - Texas Healthcare and Bioscience Institute
UT - BioUtah
VA - Virginia Biotechnology Association
VT - Vermont Biosciences Alliance
WA - Life Science Washington
WI - BioForward Wisconsin
WV - Bioscience Association of West Virginia