July 28, 2020

The Honorable Frank Pallone
Chairman, House Committee on Energy & Commerce
2125 Rayburn House Office Building
Washington D.C. 20515

The Honorable Greg Walden
Ranking Member, House Committee on Energy & Commerce
2322-A Rayburn House Office Building
Washington D.C. 20515

Dear Chairman Pallone and Ranking Member Walden:

We, the 31 members of the Council of State Bioscience Associations (CSBA), would like to provide comments on the extension of the Rare Pediatric Disease Priority Review Voucher program (RPD PRV). The member companies, scientists and researchers in our states strive daily to develop new therapies and cures for patients. This research is incredibly risky, resource-intensive, and often pushes the boundaries of science. It is also incredibly rewarding when our companies are able to deliver a new therapy that changes the course of a child's life.

Currently, Congress is considering two pieces of legislation to reauthorize the RPD PRV. This program was originally created to address unmet medical needs for many of our nation's most vulnerable patients. Since the program was enacted in 2012, 21 therapies have been approved to treat 18 rare diseases including cystic fibrosis and spinal muscular atrophy (SMA). Representatives G.K. Butterfield (D-NC) and Michael McCaul (R-TX) are the lead House sponsors (H.R. 4439), and Senators Susan Collins (R-ME) and Robert Casey (D-PA) recently introduced their own legislation in the Senate (S. 4010) to make the RPD PRV program permanent. As you may know, there are more than 7,000 identified rare diseases, more than 90% of which do not have a treatment. Approximately 50% of rare diseases impact children, and tragically 30% of those children will die before the age of 5. We believe that both the House and the Senate bills represent a critically important step toward providing hope to patients and their families, and we strongly urge Congress to permanently reauthorize the program.

Attracting investment and resources for such a limited market is a great challenge for drug developers, making legislative action by Congress imperative to incentivize research and development for rare pediatric diseases. Under this program, the Food and Drug Administration (FDA) can award a PRV to drug developers who receive an approval for a drug or biologic to treat a rare pediatric disease. That voucher can then be redeemed to receive priority review of a subsequent marketing application for a different product, potentially reducing FDA review of that product from 10 months to just 6. Importantly, a drug developer that receives a voucher may sell that voucher to another drug developer, thus providing an important

source of capital for emerging biotechnology companies. Often, that capital is then reinvested to advance other therapies in the drug developer's pipeline.

Unfortunately, the RPD PRV program is beginning to expire. The FDA will not be able to issue new PRVs for therapies unless those therapies have been both designated to treat a rare pediatric disease by September 30, 2020 and are approved by September 30, 2022. Furthermore, due to the 60 day review period for RPD PRV applications, the FDA cannot commit to providing sponsors with a response on their RPD PRV request if it is submitted after July 31, 2020. Without reauthorization, we fear a substantial drop in the number of new treatments for some of our nation's most vulnerable children. We must ensure the progress we have made in addressing rare diseases can continue unhindered with the support of patient-centered public policy.

Again, we strongly urge the reauthorization of the Rare Pediatric Disease Priority Review Voucher program and we urge Congress to pass these important bills before September 30.

We look forward to additional opportunities to work together on this issue and to provide feedback that may help guide policy development. Please contact Michele Oshman, Executive Director of the Council of State Bioscience Associations at moshman@bio.org if you have any questions or if we can provide additional information. Thank you for your consideration.

Respectfully submitted,

Council of State Bioscience Associations
Arizona BioIndustry Association, Inc. (AZBio)
California Life Sciences Association (CLSA)
Colorado BioSciences Association (CBSA)
BioCT
DelawareBio
BioFlorida
Georgia Bio
Idaho Technology Council
Illinois Biotechnology Innovation Organization (iBIO)
Indiana Health Industry Forum (IHIF)
IowaBio
BioKansas
Kentucky Life Science Council

Life Science Tennessee
Maryland Tech Council
Michigan Biosciences Industry Association (MichBio)
Missouri Biotechnology Association (MOBIO)
Montana Bioscience Alliance
BioNJ
New Mexico Biotechnology & Biomedical Association
NewYorkBIO
North Carolina Biosciences Organization (NCBIO)
Oregon Bioscience Association (Oregon Bio)
Life Sciences Pennsylvania
SCBIO
Southern California Biomedical Council (SoCalBio)
South Dakota Biotech Association (SDBA)
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