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

We represent the community of emerging biotechnology companies, many of which are still working towards bringing our first products to market. Our scientists and researchers 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 we are able to deliver a new therapy that changes a child's life.

Currently, Congress is considering two pieces of legislation to reauthorize the Rare Pediatric Disease Priority Review Voucher program (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, 22 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 know, there are more than 7,000 identified rare diseases, more than 90% of which do not have a treatment. It is also estimated that approximately 50% of rare diseases impact children, more sobering, 30% of those children will die before the age of 5. We believe both bills represent an important step in providing hope to patients and their families, 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. Without reauthorization, we fear a substantial drop in the number of new treatments for some of our 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 this critical program and urge Congress to pass these important bills before September 30.

Respectfully Submitted,

ReNetx BIO
Amicus Therapeutics
MacroGenics Inc.
Vivace Therapeutics
Ovid Therapeutics
CRISPR Therapeuticcs AG
Antiva Biosciences
Sutro Biopharma
MediSix Therapeutics
Athersys, Inc.
Global Blood Therapeutics
Syros Pharmaceuticals
GlycoMimetics, Inc.
Alnylam Pharmaceuticals, Inc.
Orchard Therapeutics
Ashvattha Therapeutics
Cerevast Medical, Inc.
Veracyte, Inc.
AGTC
PTC Therapeutics
Sana Biotechnology
Gossamer Bio
Principia Biopharma
Sangamo Therapeutics, Inc.
Ardelyx, Inc.
KPMG LLP
CymaBay Therapeutics
bluebird bio
Deerfield Management
Aeglea BioTherapeutics
Aytu BioScience