Alliance for Regenerative Medicine

February 26, 2024

The Honorable Michael T. McCaul
United States House of Representatives
2300 Rayburn House Office Building
Washington, DC 20510

The Honorable Anna G. Eshoo
United States House of Representatives
272 Cannon House Office Building
Washington, DC 20510

The Honorable Gus Bilirakis
United States House of Representatives
2306 Rayburn House Office Building
Washington, DC 20510

The Honorable Nanette Diaz Barragan
United States House of Representatives
2312 Rayburn House Office Building
Washington, DC 20510

The Honorable Lori Trahan
United States House of Representatives
2439 Rayburn House Office Building
Washington, DC 20510

The Honorable Michael C. Burgess
United States House of Representatives
2161 Rayburn House Office Building
Washington, DC 20510

Re: H.R.7384: Creating Hope Reauthorization Act of 2024

Dear Representatives McCaul, Eshoo, Bilirakis, Barragan, Trahan and Burgess:

The Alliance for Regenerative Medicine (ARM) thanks you for introducing *H.R.7384: Creating Hope Reauthorization Act of 202*4 which provides a timely and clean reauthorization of the Food and Drug Administration's (FDA) Rare Pediatric Disease Priority Review Voucher (PRV) program.

ARM represents more than 400 emerging and established biotechnology companies, academic and medical research institutions, and patient organizations. We strongly support the PRV program given its success in fostering the development of durable, potentially curative, cell and gene therapies (CGTs) for pediatric patients with life-threatening diseases.

Under Section 529 of the Federal Food, Drug, and Cosmetic Act (FD&C Act), the FDA awards PRVs to sponsors of approved rare pediatric disease product applications that meet certain criteria. This voucher can be redeemed to receive a priority review of a subsequent marketing application for a different product by the original sponsor or an external purchaser. The program does not cost the US taxpayers – but rather uses market forces and regulatory flexibility to achieve the goal of accelerating the provision of medical advances to children that often have no other treatment options. Similarly, this program does not negatively affect the FDA's budgetary capabilities as companies that use vouchers must still pay the FDA's user fees.

More than 90 percent of the estimated 10,000+ rare diseases have no FDA-approved products, and about half of these diseases uniquely affect children. Because a significant number of rare pediatric disorders have genetic causes, they are increasingly being addressed by the growing armament of CGTs – including gene therapy and gene editing – which offer paradigm shifts away from chronic or supportive care modalities. Given the hope CGTs bring to the medical community, it is particularly important that developers have the regulatory certainty, incentives, and mechanisms to facilitate timely development of these products. The Rare Pediatric Disease PRV program is one key component of the successful ecosystem created by a bipartisan Congress and supported by the FDA.

To date, several CGT sponsors have received PRVs for therapeutics that treat a range of devastating pediatric diseases with high mortality and morbidity, including acute lymphoblastic leukemia; an inherited retinal disease that leads to blindness; progressive fatal neurologic and neuromuscular diseases (including spinal muscular atrophy, cerebral adrenoleukodystrophy, and Duchenne muscular dystrophy); congenital athymia; life-threatening blood disorders (such as sickle cell disease and beta thalassemia); as well as dystrophic epidermolysis bullosa, a disorder characterized by extremely fragile skin, leading to painful and debilitating blisters.

The currently successful PRV program, established by a bipartisan Congress in 2012, is set to expire on September 30, 2024. While the statute allows products late in the pipeline to be "grandfathered" into vouchers through 2026, disruption of the program may have a significant negative impact on companies making decisions regarding their early-stage pipelines which ultimately impacts children's ability to maintain hope for access to future treatments for their rare diseases.

ARM greatly appreciates your leadership on reauthorizing the Rare Pediatric Disease PRV program in a timely fashion. We look forward to helping you continue to build bipartisan support and advocate for the program's continuation and success. Feel free to reach out to me with any questions at ecischke@alliancerm.org.

Sincerely,

Erica Cischke, MPH
Vice President, Government Affairs
Alliance for Regenerative Medicine