The EveryLife Foundation for Rare Diseases is a 501(c)3 organization
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1012 14th Street, NW
Washington, DC 20005

December 3, 2020

The Honorable Mitch McConnell
Majority Leader
United States Senate
317 Russell Senate Office Building
Washington, DC 20510

The Honorable Charles Schumer
Minority Leader
United States Senate
322 Hart Senate Office Building
Washington, DC 20510

The Honorable Lamar Alexander
United states Senate
455 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Patty Murray
United States Senate
154 Russell Senate Office Building
Washington, DC 20510

Dear Majority Leader McConnell, Minority Leader Schumer, Chairman Alexander, and Ranking Member Murray,

On behalf of the EveryLife Foundation for Rare Diseases, we are writing to express our support for immediate Senate action to extend the Pediatric Rare Disease Priority Review Voucher (PRV) Program at the FDA as proposed in the Creating Hope Reauthorization Act, S.4010, introduced by Senators Casey and Collins.

Without congressional action, this innovative and cost-effective program will expire on December 11th, eliminating a powerful incentive for the development of treatments for rare diseases. Approximately 93% of the 7,000 rare diseases have no treatment options yet advances in novel technology platforms for therapy development mean that for many, a treatment could be within reach if the right incentives and regulatory pathways exist to make it financially viable for companies.

The PRV program has enabled life-saving treatments to reach children with rare diseases faster without adding new costs to taxpayers. The PRV program has the potential to revolutionize the way we think about ultra-rare therapeutic development business models, but this burgeoning opportunity is in peril if Congress allows the program to expire on December 11th.

As you consider the future of this program and its significance, we are proud to share with you the story of a recently established non-profit organization that is addressing the often-neglected small population diseases that are seeing industry pass on investment opportunities, some of which have the potential to be one-time curative treatments.

The Institute for Life Changing Medicines (ILCM) asserts that one of the most powerful incentives in developing transformative therapies for rare pediatric diseases is the PRV. The value of the PRV comes in two ways. First the value realized through the sale of a PRV is large enough to encourage drug developers to pursue treatments for very rare diseases. Second, in many situations the proceeds of a PRV sale can be directly re-invested into other very rare disease therapeutics providing a virtual cycle of transformative treatments across a broad array of diseases.

The most obvious example of this re-investment is for companies that solely focus on rare diseases. We suggest however that PRVs could also catalyze a new paradigm of drug development for rare diseases in the non-profit setting, an example of which is the recently established the ILCM, 501 c3 organization. This new entity was established in

early 2020 by rare disease stakeholders to develop and distribute transformative therapies to patients with very rare diseases.

Based on extensive collaboration and donations of programs and intellectual property, ILCM is advancing new treatments through clinical development with the goal of filing for registration. Each approval should provide an opportunity for a PRV which can be monetized to allow for additional programs and the distribution of approved products at very affordable prices.

Dr. James Wilson, Director of the Orphan Disease Center at the University of Pennsylvania, is leading the ILCM with a committed group of scientists, financial and market experts and patient advocates. Their innovative approach has a real chance to transform lives, but they need the Senate to act.

The House passed the Creating Hope Reauthorization Act (HR 4439) in September, paving the way for the PRV program to be extended for another 4 years. While the list of urgent issues before the Senate is long, we implore you to add a similar 4-year extension of this cost-effective, innovative program with bipartisan support to your end-of-year agenda. Children and families with devastating and life-threatening rare diseases are counting on you. The EveryLife Foundation stands ready to assist in your deliberations as do our partners at ILCM who offer the most concrete example yet of the power of the PRV incentive.

Thank you for your ongoing commitment to the health of our nation and the 30 million Americans with a rare disease. We look forward to working with you to enact an extension to the Rare Pediatric PRV program. If you have any questions, please contact Jamie Sullivan, Director of Public Policy, by email at jsullivan@everylifefoundation.org, or by phone at (202) 445-4009.

Sincerely,

Julia Jenkins
Executive Director
EveryLife Foundation for Rare Diseases

Annie Kennedy
Chief of Policy & Advocacy
EveryLife Foundation for Rare Diseases

CC:
The Honorable Susan Collins
The Honorable Bob Casey
Jim Wilson, MD, PhD
Mark Dant, Board Chairman
Frank J. Sasinowski, Board Vice-Chairman