Statement from EveryLife Foundation for Rare Diseases
House Energy & Commerce Committee Markup:
September 18, 2024

Chair Rodgers and Ranking Member Pallone and Members of the Committee,

On behalf of the EveryLife Foundation for Rare Diseases, we thank you for convening this markup to advance bipartisan legislation that will help to support the needs of people and families impacted by rare diseases and disorders. Rare diseases impact more than 30 million Americans and cost our nation more than a $1 trillion each year, yielding devasting economic tolls on families through non-medical and indirect costs[1].

The EveryLife Foundation for Rare Diseases is a 501 (c)(3) nonprofit, nonpartisan organization dedicated to empowering the rare disease patient community to advocate for impactful, science-driven legislation and policy that advances the equitable development of and access to lifesaving diagnoses, treatments, and cures. EveryLife Foundation's establishment of the Community Congress, a diverse coalition comprised of patient advocacy organizations, industry leaders, coalition groups, and other relevant stakeholders guides our policy efforts and provides advice and insight on important policy issues impacting the rare disease community.

Tomorrow, the Committee will discuss The Give Kids a Chance Act (H.R. 3433), which now includes multiple bills that will accelerate the development and availability of therapies for rare diseases. We urge the Committee to vote in support of the bill while avoiding harmful amendments to ensure that the included legislation described below can progress. Individually and collectively, they represent important progress to bring therapies for the 95 percent of rare diseases without an FDA-approved therapy.

H.R. 7384, the Creating Hope Reauthorization Act of 2024
Without Congressional action, the Rare Pediatric Priority Review Voucher (PRV) Program will expire on September 30, 2024, leaving one less tool to bring treatments to a rare disease community in which only five percent of diseases have an FDA-approved treatment. It is imperative that we continue to advance solutions to treat these devastating diseases. Allowing the Rare Pediatric PRV program to expire would eliminate a powerful incentive for the

[1] Yang G, Cintina I, Pariser A, Oehlrlein E, Sullivan J, Kennedy A. The national economic burden of rare disease in the United States in 2019. Orphanet J Rare Dis. 2022;17:163

development of treatments for the 70 percent of rare diseases that start in childhood[2]. PRVs have been associated with an increased rate of progress throughout the clinical trial process, leading to life-altering product approvals to treat almost 40 rare diseases such as Progeria syndrome, Spinal Muscular Atrophy, Sickle Cell Disease, Rett syndrome, and other conditions that previously lacked any FDA-approved treatments.

Importantly, the PRV program is a market-based incentive that comes at no cost to taxpayers. A simple change in the timeline for the proposed authorization has also alleviated previous concerns expressed by FDA leadership, many of whom now acknowledge the importance of maintaining programs focused on encouraging more pediatric rare disease therapy development. The Creating Hope Reauthorization Act, as included in the Give Kids a Chance Act, ensures that a critical rare disease development incentive remains intact for another six years. As more than half of rare pediatric designations have occurred in the last four years, we know the PRV program is working to address unmet needs and efforts to fundamentally change the program at this critical juncture will only serve to undermine the program's effectiveness. We urge the Committee to advance the bill and call upon Congress to ensure a timely reauthorization after Committee action.

H.R. 7383, the Retaining Access and Restoring Exclusivity (RARE) Act:
A 2021 court ruling determined that orphan drug exclusivity under the Orphan Drug Act grants a manufacturer exclusivity across an entire disease or condition, even if it has only had a drug approved for one population or indication. This was contrary to how the FDA has interpreted the Orphan Drug Act over the last several decades. This legislation would specify that the seven-year market exclusivity period for drugs for rare diseases or conditions (i.e., orphan drug exclusivity period) prohibits the approval of other drugs for the same approved use or indication with respect to the disease or condition rather than applying to all uses within the disease or condition. The proposal would ensure that the FDA can continue to approve a drug that aims to serve different patient populations such as pediatric approval for a drug that has exclusivity based on an adult approval.

Conclusion
Thank you for your long history of supporting the rare disease community. This week's markup signifies important progress for the rare disease community, and we look forward to supporting your efforts to shepherd these bills into law. We stand ready to assist the Committee as you continue your consideration of these and other polices relevant to all impacted by rare diseases.

[2] Nguengang Wakap S, Lambert DM, Olry A, Rodwell C, Gueydan C, Lanneau V, et al. Estimating cumulative point prevalence of rare diseases: analysis of the Orphanet database. Eur J Hum Genet. 2020;28(2):165–73.