May 10, 2022

Rep Cathy McMorris Rogers
United States House of Representatives
Washington, DC 20515

Rep. Frank Pallone, Jr
United States House of Representatives
Washington, DC 20515

Rep. Brett Guthrie
United States House of Representatives
Washington, DC 20515

Rep. Anna Eshoo
United States House of Representatives
Washington, DC 20515

RE: Food and Drug Administration User Fee Agreements legislative package

Dear Representatives McMorris Rogers, Pallone, Guthrie, and Eshoo,

On behalf of patients impacted by rare diseases, the EveryLife Foundation for Rare Diseases is grateful for the unwavering efforts of the Committee to ensure that the infrastructure needed to support life-saving research and regulatory momentum is achieved through the passage of the User Fee Agreements of 2022. The EveryLife Foundation 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.

The most recent estimates suggest there are up to 10,000 rare diseases. While the majority of rare diseases (93-95%) lack any FDA-approved disease-modifying therapies, we are grateful for recent breakthroughs that have extended the life expectancies of once-fatal conditions and we are optimistic about the research that is ongoing to develop treatments for additional conditions. For more than a decade, the rare disease patient community has played a significant role in the paradigm shifts and statutory changes that have occurred with respect to the inclusion of patients and patient experience data within clinical trial design and regulatory review. Many of these statutory inflection points that have had direct, significant impact on Americans living with rare diseases have yielded from Congress' efforts in PDUFA V and PDUFA VI as life-saving product approval pathways were established, patient focused drug development (PFDD) became codified as a part of FDA's mission, and patient experience data was formally integrated into the regulatory review.

We are pleased to see the FDA User Fee Agreement legislative package that was released on May 4th reflect so many of the key priorities of our rare disease community. The package reflects an ongoing evolution of a critical paradigm shift in therapeutic development, infrastructure, and stakeholder engagement within the ecosystem that has yielded robust development pipelines in disease areas with significant unmet need. We are particularly gratified to see a commitment to FDA engagement with external experts, advancing knowledge of endpoints, understanding "small population differences",

clarifying authorities (exclusivity application, payor communication), and protecting and strengthening the accelerated approval pathway.

We look forward to opportunities to continue to engage and expand upon new innovations and infrastructure to support rare disease science, therapeutics, medical technology, and patient focused drug development, including patient communities and clinical experts as key stakeholders within development and regulatory review.

We also appreciate your continued interest and willingness to work with us to consider further enhancements to the introduced legislation that seek to build upon the principles in the draft to further strengthen policies to advance development of therapies for rare disease patients. We are hopeful that the inclusion of targeted amendments will further our joint goals and objectives, including helping to ensure expertise in rare disease therapy development can be better coordinated and shared across all FDA Centers.

On behalf of the EveryLife Foundation, we thank you and your colleagues for your continued commitment to science driven public policy. We look forward to now continuing to work alongside you to build upon the successes of the passage and implementation of the landmark PDUFA VI – and realize the potential of existing opportunities to accelerate the development of therapies and access for Americans living with rare diseases. For additional information, please contact Jamie Sullivan jsullivan@everylifefoundation.org or Annie Kennedy akennedy@everylifefoundation.org .

Sincerely,

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

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Senior Director of Policy
EveryLife Foundation for Rare Diseases

CC: Mark Dant, Board Chairman
Frank J. Sasinowski, Board Vice-Chairman