Rare Disease Company Coalition

September 14, 2022

The Honorable Patty Murray
Chair
Senate HELP Committee
154 Russell Senate Office Building
Washington, D.C. 20510

The Honorable Richard Burr
Ranking Member
Senate HELP Committee
217 Russell Senate Office Building
Washington, D.C. 20510

The Honorable Frank Pallone
Chair
House E&C Committee
2107 Rayburn House Office Building
Washington, D.C, 20515

The Honorable Cathy McMorris Rodgers
Ranking Member
House E&C Committee
1035 Longworth House Office Building
Washington, D.C. 20515

Dear Chair Murray and Ranking Member Burr; Chair Pallone and Ranking Member McMorris Rodgers,

The Rare Disease Company Coalition (RDCC) is writing in follow up to our comments on S. 4348, the Food and Drug Administration Safety and Landmark Advancements (FDASLA) Act of 2022.

The RDCC commends the work done by the Senate Health, Education, Labor, and Pensions (HELP) and House Energy & Commerce Committees on the Prescription Drug User Fee Act (PDUFA) reauthorization thus far. As we near the September 30 deadline, the RDCC strongly encourages timely reauthorization of the user fees to ensure continuity of critical funding for the Food and Drug Administration (FDA) and avert a delay in the rare disease drug review process.

The RDCC represents 21 innovative life science companies committed to discovering, developing, and delivering treatments for the one in ten Americans living with a rare disease. Collectively, RDCC members invested over $12.4 billion in R&D in 2021; have brought 31 treatments to market to date, the majority of which are first-to-market therapies; and are presently working on more than 200 rare disease development programs, many of which would be the first ever FDA-approved therapy for patients with a given rare disorder.

Biopharmaceutical researchers have made tremendous progress over the years, yet more than 90 percent of rare diseases still do not have any treatment options. This represents a significant and time-sensitive unmet need for patients. RDCC members with rare disease treatments under FDA review rely on agency experts to support the development and approval of safe and effective therapies for rare disease patients. Additionally, rare disease therapies at early stages of development also rely on FDA input and meetings to advance through clinical development such that manufacturers have the data necessary to submit to FDA for review. A furlough of FDA staff resulting from a delay in User Fee Authorization could have serious negative consequences for patients by stalling timely approval of these life-saving therapies. For example, the RDCC represents one small biopharmaceutical company that has upcoming meetings with the FDA to discuss their submission of a lead candidate for a condition affecting 35,000 Americans who currently lack a treatment. These meetings can only occur if the FDA is fully staffed to review their submission. Any delays that occur from FDA staffing shortages would be devastating for this patient population.

The RDCC also represents several member companies that are conducting clinical trials or have submitted New Drug Applications (NDA) or Biologics License Applications (BLA) for FDA review, many of which primarily impact children, that currently have no, or limited, treatment options. If FDA is unable to provide timely input regarding these trials or regarding these NDAs or BLAs, the development and review of these therapies could potentially result in these children's conditions progressing irreversibly and unabatedly.

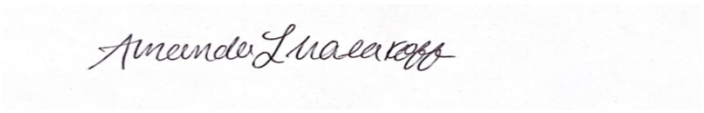
The RDCC underscores its previously submitted comments of support and suggested inclusions for S.4348. Among our recommendations is the Helping Experts Accelerate Rare Treatments (HEART) Act (H.R.6888/S. 4071), modified with suggested language for reporting on FDA reviewer training and maintaining appropriate regulatory flexibility with regards to the accelerated approval pathway. Additionally, if an agreement is reached for including changes to the accelerated approval program, the RDCC respectfully urges Congress to include the FDASLA text, which excludes the House's drug labeling provision. This provision undermines current law, which clearly states that therapies granted accelerated approval must meet the same statutory standards for safety and effectiveness as those granted traditional marketing authorization.

Notwithstanding our comments on additional provisions, our foremost concern is that User Fee Authorization be reauthorized by the deadline to ensure the FDA has the resources it needs to operate at full capacity. We encourage Congress to advance the bill as quickly as possible to prevent a stall in the process of drug approvals, and speed rare disease patients' access to new life-saving medications.

Thank you for your consideration of our recommendations. The RDCC appreciates the opportunity to engage with Committee members and staff to ensure that User Fee Authorization moves forward in a timely manner.

If you have any questions, please feel free to contact Amanda Malakoff at amanda@rarecoalition.com.

Sincerely,



Amanda Malakoff
Executive Director
Rare Disease Company Coalition