Rare Disease Company Coalition

November 30, 2022

The Honorable Josh Gottheimer
United States House of Representatives
203 Cannon House Office Building
Washington, DC 20515

The Honorable Don Bacon
United States House of Representatives
1024 Longworth House Office Building
Washington, DC 20515

Dear Representatives Gottheimer and Bacon,

The Rare Disease Company Coalition (RDCC) is grateful for your recognition of the challenges that clinical research has faced during the pandemic and would like to express our support for your legislation, H.R. 8641, *The Orphan Drug COVID -19 Mitigation Act*.

The RDCC represents 22 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.

Our members are actively investing in the mission to bring life-saving treatment to rare disease patients every day with 206 rare disease development programs in progress collectively, many of which would be the first ever FDA-approved therapy for rare disease patients. Rare disease biotechnology companies know firsthand the challenges that clinical research faced during the COVID-19 pandemic and are thankful to you for recognizing this as an issue and seeking a pragmatic policy solution.

When the pandemic began in March 2020, the scientific community reacted with a dramatic increase in COVID-19 clinical trials, but progress in other therapeutic areas stalled due to safety concerns. The new safety and logistical challenges added by the pandemic resulted in increased costs, delays, and uncertainty in trial initiation and completion for rare disease drug manufacturers. Rare disease patients have conditions that make them more susceptible to and higher risk for COVID-19, making it especially difficult for this population to participate in clinical trials.

Even prior to the pandemic, rare disease companies faced obstacles with clinical trial recruitment due to the small, geographically dispersed patient populations, disease heterogeneity, and low—or even lack of—disease awareness leading to delays in diagnosis. Further, approximately half of rare diseases affect children, adding another logistical complexity for rare disease companies in clinical trial planning and execution.

By October 2020, an estimated 80 percent of non-COVID trials were stopped or delayed during the pandemic. Over 700 trials experienced disruptions since the beginning of the pandemic, with over 250 suspending enrollment due to the pandemic. In May 2020, NIH Director, Dr. Francis Collins estimated that $10 billion of research outside of COVID-19 had been lost. Given the tumultuous two years that have followed, the amount of lost research has continued to multiply.

Many of the companies investing in rare disease research are small biotechnology companies that face particularly stark financial challenges, leaving them with exceedingly difficult choices on whether to resume their trials or stay in business altogether. Much of the critically necessary rare disease clinical research remains stalled, while millions of patients are waiting for breakthrough treatments and cures for the diseases that threaten their lives.

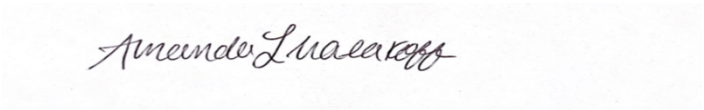
Research in the era of the COVID pandemic faces a crisis that demands new incentives to drive research. The clinical research ecosystem faces extreme disruption, and, as a result, many patients with rare diseases will be left to wait even longer for needed treatments and cures if we don't act quickly to restore resources to research.

Modeling the success of the *Food and Drug Administration Modernization Act* (FDAMA)'s pediatric exclusivity provision, *The Orphan Drug COVID-19 Mitigation Act* will provide companies with the needed flexibility to resume these stalled clinical trials, ensuring treatments and cures get to the patients who need them.

The RDCC is grateful for your leadership on this important issue which will help to bring hope and needed cures to the at least 95 percent of rare disease currently lacking an FDA-approved treatment. Given the time-sensitive nature of advancing clinical trials for rare disease patients, we will work with you to urge Members of Congress to co-sponsor *The Orphan Drug COVID-19 Mitigation Act* and push for its inclusion in the next available legislative vehicle.

If you have any questions or would like to discuss further, please feel free to contact Amanda Malakoff at amanda@rarecoalition.com.

Sincerely,



Amanda Malakoff
Executive Director
Rare Disease Company Coalition