Rare Disease
Company Coalition

For Immediate Release

The "Rare Disease Company Coalition" Launches to Share the Unique Challenges and Promise of Rare Disease Therapy Development to Support Continued Progress and Patient Access

With one in 10 Americans living with an often devastating and debilitating rare disease and limited treatment options, a coalition of life science companies cites a "one-size-fits-all" approach to healthcare policy and regulations as detrimental to continued innovation that has potential to systematically improve quality of life for millions

WASHINGTON, DC - May 13, 2021 - **The Rare Disease Company Coalition,** a unified voice of life science companies committed to discovering, developing and delivering rare disease treatments, today announced their organizational launch. This first-of-its-kind alignment of organizations will inform and help educate policymakers on the unique circumstances facing life science companies when developing and bringing to market therapies for very small and differentiated patient populations. The Coalition will also advocate for policies and regulations that enable cost-effective and more timely delivery of treatments by recognizing these differences.

Collectively, the Rare Disease Company Coalition represents life science companies that have brought to market 22 treatments and are presently working on more than 160 rare disease development programs, many of which would be first-to-market therapies if approved. These diseases are devastating and often life-threatening: 80 percent of rare diseases are genetic in origin, 50 percent impact children, and 30 percent of those children won't live to see their 5th birthday. The Coalition is composed of companies continuing to change those statistics by discovering, developing and bringing valuable treatments – and even potential cures – to market for these patients awaiting a treatment approved by the Food and Drug Administration (FDA).

The pharmaceutical industry continues to lead the nation in research and development expenditures by dedicating on average between fifteen to twenty percent of sales to fostering innovation. Companies focused on rare diseases take on an outsized commitment to and risk in research and development. In 2020, Rare Disease Company Coalition members invested more than $4.1 billion in research and development, representing approximately 65 percent of their annual operating budgets. Moreover, the majority of Coalition members spend more annually in research and development than revenues generated, including the over one-third of members that are in the preclinical stage and currently do not generate revenue.

Building on the results of these capital investments, smaller companies are now driving the groundbreaking innovation happening across multiple disease areas. As reported by the Congressional Budget Office, 70 percent of the nearly 3,000 therapies in Phase III clinical trials are from small drug companies or those with annual revenues of less than $500 million. Moreover, about one-third of new treatments approved by the FDA have been developed by companies with annual revenues of less than $100 million.

Expanding on years of advocacy work through successful industry associations and rare disease patient-focused organizations, the Rare Disease Company Coalition will specifically work to:

- Inform and help educate policymakers on the unique circumstances of the rare disease company business model, specifically focusing on the different issues created by working with small and differentiated patient population sizes;
- Advocate for public policy and regulatory frameworks that account for and recognize the distinct considerations of life science companies operating in the rare disease space;
- Work with policymakers to establish long-term, consistent, equitable and sustainable research incentives for rare diseases and access to the resulting innovation;
- Increase awareness of the innovation happening within rare disease companies.

Founding members of the Coalition include:

- Acceleron Pharma
- Aeglea BioTherapeutics
- Agios Pharmaceuticals
- Alnylam Pharmaceuticals
- Harmony Biosciences
- Orchard Therapeutics
- Orphazyme US, Inc.
- Sarepta Therapeutics
- Taysha Gene Therapies
- Ultragenyx Pharmaceutical

In the coming month, the Coalition will engage with policy stakeholders on potentially impactful drug and healthcare policies and regulations currently under discussion, including prescription drug pricing, to highlight the consequences that blanket legislation can have on continued innovation for rare disease treatments.

About

The Rare Disease Company Coalition represents life science companies committed to discovering, developing and delivering rare disease treatments for the patients we serve. As an education and advocacy-focused coalition of companies, our goal is to inform policymakers of the unique challenges and promises of rare disease drug discovery, development and manufacturing for small population sizes in order for critical innovation to continue. To achieve this goal, we will use our unified voice to advocate for long-term, consistent, equitable and sustainable government policies that enable life science companies to continue to bring hope and provide access to approved treatments to people living with rare diseases. For more information, please visit rarecoalition.com

Media Contact:
Anna Stallmann
media@rarecoalition.com
708-476-1258