Rare Disease Company Coalition

Hello,

Today marks a significant milestone for the Rare Disease Company Coalition: the fifth anniversary of our founding in May 2021.

The RDCC was created for a singular and critical mission: to give rare disease innovators One Rare Voice to advocate for policies that protect innovation and ensure access for patients living with a rare disease.

Over the past five years, RDCC has developed into a leading voice for rare disease innovators. Our 36 member companies are leaders in rare diseases and represent some of the most forward-thinking biopharmaceutical companies in the world. We have delivered nearly 100 rare disease treatments to market and have over 230 programs in development, many of which would be first in class or best in class. On average, our members invest over half of all expenditures in research and development, totaling over $25 billion annually.

Because of the support from our members, and in collaboration with our friends and partners across the rare disease community, RDCC has played a leading role in delivering significant policy wins:

- Safeguarded rare disease patient access and innovation from blunt drug pricing policies at both the federal and state levels, including passage of the ORPHAN Cures Act and protection for rare diseases therapies from pharmaceutical tariffs.
- Protected key rare disease incentives, including the reauthorization of the Rare Pediatric Disease Priority Review Voucher (PRV) program and the preservation of the Orphan Drug Tax Credit (ODTC).
- Expanded Medicaid access to rare disease therapies through the passage of the Accelerating Kids' Access to Care Act, promotion the patient voice through state Rare Disease Advisory Councils (RDACs), and protection of rare disease therapies from Prescription Drug Affordability Boards.
- Advanced rare disease initiatives at FDA, including the creation of the Rare Disease Innovation Hub and other programs to ensure continued progress toward flexibility and predictability through regulatory processes.

But despite the tremendous progress we've made, there remain significant challenges and obstacles ahead for the rare disease community. The need for continued progress remains urgent if rare disease companies are to launch and sustain development programs for patients who are waiting for hope, and ultimately ensure everyone who needs a life-changing rare disease treatment can get one With over 95 percent of rare diseases lacking an FDA-approved treatment, every day is precious.

Our deepest thanks to everyone who has played a part in our journey, and in particular, our thanks to the patients and caregivers who have walked with us every step of the way. We stand ready to continue this important work with One Rare Voice through the next five years and beyond.

Chris Porter
Chair
Rare Disease Company Coalition