Rare Disease
Company Coalition

2024 Outlook

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Welcome Letter

Dear Colleagues,

Three years ago, the Rare Disease Company Coalition was formed to unify and amplify the voices of life science companies working to **discover, develop, and deliver treatments for patients living with a rare disease**. The need for this coalition was and remains clear: we are at a crossroads in this era of unprecedented scientific innovation. For four decades, the pace of rare disease drug development has skyrocketed. Prior to the enactment of the Orphan Drug Act in 1983, there were only 38 treatments available for rare diseases; now, there are over 600 treatments for over 1,000 rare diseases - **an increase of over 1,500 percent**. Despite the successes of proven, smart policy that enabled such rapid advances in rare disease drug development, we now find ourselves fighting policies that threaten to dismantle the very system that enabled such progress over the past decades

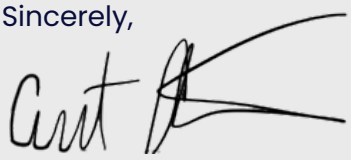
Since its inception in 2021, the RDCC has established itself as a **trusted leader, thought partner, and resource** in the rare disease community. And with the partnership of champion policymakers, patient advocacy organizations, and key third-party stakeholders, we maintain our commitment to **providing hope** for the patients living with one of the 95 percent of rare diseases with no FDA-approved treatment.

2024 will be a critical year for the rare disease community - with crucial policy challenges and opportunities, a competitive election cycle, and continued economic uncertainty, it is now more important than ever for the RDCC to be a fierce advocate for rare disease innovation and access.

Our advocacy centers around two core principles: **incentivize innovation for rare disease drug development, and ensure access for patients living with a rare disease**. Our 2024 Strategic Plan contains ambitious goals to address concerns and opportunities within both pillars, and empowers the RDCC to harness the support and engagement of our member companies and broader rare disease community.

It is my pleasure and honor to serve as Chair of the RDCC in 2024. I am extraordinarily grateful for the leadership of our 2024 Executive Committee, Committee Chairs, and staff. With #OneRareVoice, we can accomplish anything.

Sincerely,



Curt Oltmans
Chair, RDCC Board of Directors

2024 Leadership Team

Executive Committee

Chair
Curt Oltmans
Fulcrum Therapeutics

Vice-Chair
Del Lebel
Alexion, AstraZeneca Rare Disease

Treasurer
Chris Porter
Travere Therapeutics

Secretary
Kathy Perez
argenx

Member-At-Large, Commercial
Diane Berry
Sarepta Therapeutics

Member-At-Large, Pre-Commercial
Max Bronstein
Crinetics Pharmaceuticals

Committee Chairs

Policy Committee Co-Chair
Lisa Kahlman
Ultragenyx

Policy Committee Co-Chair
Stephanie Kelly
Kyowa Kirin, Inc.

Communications Committee Chair
Molly Cameron
Marinus Pharmaceuticals

Coalition Staff

Executive Director
Stacey Frisk
Rare Disease Company Coalition

Chief of Staff
Yoko Rosenbaum
Rare Disease Company Coalition

Coalition Overview

23+
Member companies

50
States with member company facilities and/or employees

58%+
Percent of expenditures spent on R&D

$17B+
Amount invested in R&D

45
Approved products on the market

200+
Development programs in the pipeline

Our Members

Abeona Therapeutics
agios
Alexion AstraZeneca Rare Disease
Alnylam Pharmaceuticals
argenx
astria Therapeutics
BIOMARIN
bridgebio
Chiesi global rare diseases
Crinetics Pharmaceuticals
Fulcrum Therapeutics
inozyme pharma
Kyowa Kirin
MARINUS
Ovid Therapeutics
prothena
Rezolute
Sarepta Therapeutics
Soleno Therapeutics
Stealth BioTherapeutics
Travere Therapeutics
ultragenyx

2023: Year in Review

The Landscape

2023 marked the 40th anniversary of the landmark Orphan Drug Act (ODA). Throughout the year, rare disease companies continued to lead at the forefront of scientific innovation and cutting-edge research. Over half of the 58 novel U.S. drug approvals in 2023 were for orphan drugs - and 8 of those 28 approved drugs were developed by RDCC member companies.[1]

The Coalition

2023 was also a landmark year for the RDCC. Since our founding in 2021, we have established ourselves as a trusted and reputable voice in the rare disease ecosystem - and we continue to expand both as an organization and in reputation.

In 2023, We...

Grew our membership by 20%

Submitted 14 comment letters

Increased our operating budget by 12%

Released a paper on policy's impact on rare disease investment

Held 2 fly-ins for our Board of Directors

Published 3 policy platforms

Our 2024 Priorities

What's Ahead?

2024 has been and will continue to be a pivotal year for rare disease policy, with significant challenges, opportunities, and deadlines on the horizon. We identified the three areas most important to our members, and look forward to continuing to lead the charge throughout the year.

I. Support the reauthorization of the Rare Pediatric Disease Priority Review Voucher Program

1 in 2 patients diagnosed with a rare disease in the United States are children - and **30 percent o**f those children won't live to see their fifth birthday.[2] Pediatric rare disease research is critical to **provide hope** for these children and their families. Congress established the **Rare Pediatric Disease Priority Review Voucher (RPD PRV) program** in 2012, which grants companies a PRV if they successfully develop a therapy for a rare pediatric disease. The program has proven to be innovative and cost-effective throughout the past decade. It is a critical incentive designed to spur the research and development of rare disease treatments for the most vulnerable **populations at no additional cost to taxpayers**. However, the program expires on **September 30, 2024** - and if Congress does not act quickly, hope for millions of children will vanish.

Since 2012, over 50 RPD PRVs have been granted, benefitting over 40 rare diseases [3]

The Solution

The RDCC strongly urges Congress to act quickly and pass **H.R. 7384, the Creating Hope Reauthorization Act**, to reauthorize the Rare Pediatric Disease Priority Review Voucher program for the next four years.

II. Advocate for timely access to rare disease treatments with both public and private payers.

Even the most innovative and effective rare disease treatments are **only as valuable as they are accessible**. For rare disease patients who have an FDA-approved treatment for their condition, we must advance policies that **facilitate timely access** so patients can benefit from treatments as soon as they are available. In 2024, we will work to advance policies to ensure timely access to rare disease treatments with both public and private payers.

The Solutions

1 **Support S.3131/H.R.5539, the ORPHAN Cures Act, a bipartisan, bicameral piece of legislation that would strengthen and expand the orphan drug exclusion in the Inflation Reduction Act (IRA).**

2 **Support legislation that promotes timely access to rare disease treatments for Medicaid populations, including S.2372/H.R.4758, the Accelerating Kids' Access to Care Act.**

3 **Support efforts to revise and reform the Employee Retirement Income Security Act (ERISA), to ensure rare disease patients with employer-sponsored health plans have equal access to therapies they need.**

Interested in learning more? Scan the QR code to read about the RDCC's policy solutions for rare disease access issues.

III. Develop a framework to ensure consistent application of regulatory flexibility for rare disease reviews across the FDA.

Rare disease drug development is **inherently risky** - and costly. 83 percent of all developmental candidates fail;[4] it takes 15 years on average to bring a drug to market;[5] and clinical development costs throughout those years can total hundreds of millions of dollars. While the scientific journey behind rare disease drug development may be unpredictable, the path to approval should not be. It is critical that the FDA ensures consistent application of regulatory flexibility for rare disease reviews across the agency, to reduce and eliminate uncertainty in an already-uncertain practice.

Coming Soon

This year, the RDCC will develop and publish a framework of policy recommendations based on the experiences of our own member companies to inform policymakers about the obstacles and opportunities of the regulatory process in the United States.

Citations

1. Center for Drug Evaluation and Research, Advancing Health Through Innovation: New Drug Therapy Approvals 2023
2. National Center for Advancing Translational Sciences, Delivering Hope for Rare Diseases
3. AgencyIQ, Priority Review Voucher Tracker
4. Biotechnology Innovation Organization, Clinical Development Success Rates and Contributing Factors 2011-2020
5. Tufts Center for the Study of Drug Development, Patent-to-launch Time for Orphan Drugs is 2.3 Years Longer vs. Other Drugs

Rare Disease
Company Coalition

We are dedicated to being a reliable and trusted resource for our leaders in Congress and the Administration, and a productive partner to rare disease-minded industry, academic and patient groups, by educating on the issues and opportunities that affect rare disease companies and advancing our shared mission to improve the lives of people living with rare diseases.

For more information, please visit www.rarecoalition.com or contact info@rarecoalition.com.
Follow us on Twitter at @RareCoalition and on LinkedIn.