Rare Disease
Company Coalition

A Primer on Rare Disease Drug Development

Policy and regulatory decisions can impact the ability of rare disease life sciences companies to survive or thrive. Surviving often means struggling to secure the funding needed to advance clinical trials and research. Thriving can mean a world with more opportunities, a better quality of life, and new treatments for rare disease patients. With 95% of rare diseases lacking an FDA-approved treatment today, patients cannot afford to wait.

Due to the complex nature of rare diseases, rare disease companies dedicate a comparatively higher percentage of operating expenses to research and development (R&D). Treatments are developed for small population sizes, but the costs to bring them to market remain the same or higher than that of drugs for larger populations due to the complex diagnosis odyssey, lack of natural history, limited access to patients for participation in clinical trials, and the often-unprecedented regulatory pathway.

To meet the high cost of rare disease research, many commercial-stage companies must continue raising billions of dollars from investors to sustain R&D, often spending more on research than they make in revenue. Similarly, clinical-stage companies do not bring in product revenue, and are largely dependent on capital markets to fund their research to remain viable.

"One-size-fits-all" health policies that do not consider the unique risks, challenges, and costs associated with rare disease drug development send a negative signal to investors and threaten continued capital that companies need to sustain their work to bring new treatments to patients.

The Rare Disease Company Coalition (RDCC) calls on our leaders in government to consider the unique challenges associated with rare disease drug development and the high unmet need for patients by providing the appropriate flexibilities, considerations, and incentives needed to support continued innovation for rare diseases.

Rare Disease by the Numbers

10,000+
rare diseases [1]

95%+
of rare diseases do not have an FDA-approved treatment [2]

1 in 10
Americans live with a rare disease (30 million people) [3]

50%
Percent of rare diseases affect children [4]

7.6 years
Average time to diagnosis [5]

$1 trillion
Cost on health care system annually [6]

1 https://rare-x.org/wp-content/uploads/2022/05/be-counted-052722-WEB.pdf
2 https://globalgenes.org/learn/rare-disease-facts/
3 https://rarediseases.info.nih.gov/about
4 https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6441966/
5 https://globalgenes.org/wp-content/uploads/2013/04/ShireReport-1.pdf
6 https://everylifefoundation.org/wp-content/uploads/2021/02/The_National_Economic_Burden_of_Rare_Disease_Study_Summary_Report_February_2021.pdf

Rare Disease Drug Development by the Numbers

Key Data Points	Rare Disease Treatments	Prevalent Disease Treatments
Average time to bring a drug to market	**15 years**[7]	**10 years**[7]
Average % of budget invested in R&D	**52%**[8]	**25%**[9]
First-in-Class* status	**50%+**[10]	**26%**[10]
% of Total U.S. Drug Spending	**11%**[11]	**73%**[11]

* First-in-class drugs are ones that use a new and unique mechanism of action for treating a medical condition.[12]

Realizing the Potential for a Brighter Future

RDCC members are collectively working to advance more than 200 rare disease treatments, many of which would be the first ever FDA-approved therapies for patients with a given rare disease. To realize this promising potential, we ask that our representatives in Congress:

Support robust development and innovation

- Preserve and strengthen the research incentives established by the Orphan Drug Act, including the Orphan Drug Tax Credit and orphan exclusivity
- Maintain support for expedited programs, including accelerated approval, Priority Review Voucher, Fast Track, Breakthrough Designation, and Regenerative Medicine Advanced Therapy Designation
- Encourage the FDA to use all of the tools in their toolbox to provide flexibilities for rare disease clinical trials, including patient-focused drug development, use of real world evidence, and expert consults
- Improve exemptions for rare disease treatments in the *Inflation Reduction Act* and further drug pricing policies to ensure that future research for new rare disease indications is not put at risk

Ensure accessibility

- Exempt rare disease treatments from one-size-fits-all value assessment frameworks
- Preserve patient access to rare disease treatments by requiring coverage for all drugs determined safe and effective through the accelerated approval pathway
- Strengthen nondiscrimination requirements for health plans and issuers coverage of rare disease treatments

Create a nationwide, standardized enrollment process for out-of-state providers treating rare disease patients

Enable earlier diagnosis

- Advance the continued funding and modernization of the newborn screening system and reauthorize funding
- Enable coverage for whole genome sequencing

Rare Disease Company Coalition

The RDCC is a coalition of 21 life science companies committed to discovering, developing, and delivering life-changing therapies for rare disease patients around the globe. The RDCC works to educate key policy stakeholders on the unique considerations of life science companies when developing and bringing to market therapies for small and differentiated patient populations.

For more information, please visit www.rarecoalition.com or contact info@rarecoalition.com.

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7 http://phrma-docs.phrma.org/sites/default/files/pdf/rd_brochure_022307.pdf
8 Rare Disease Company Coalition 2022-2023 Annual Report
9 https://www.cbo.gov/publication/57126
10 https://www.bio.org/clinical-development-success-rates-and-contributing-factors-2011-2020
11 https://icer.org/wp-content/uploads/2022/04/ICER-White-Paper_The-Next-Generation-of-Rare-Disease-Drug-Policy_040722.pdf
12 https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5221712/