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Hearing on
"From Regulator to Roadblock: How FDA Bureaucracy Stifles Innovation"
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Summary of Testimony

Congress has Powered Rare Disease Therapy Development Progress

- Since 1983, Congress has created incentives and policies that recognize the inherent complexities in developing treatments for rare diseases.
- Congress has explicitly given the FDA authority to uphold the highest standards of regulatory safety and rigor, while applying tailored approaches (i.e. "regulatory flexibility"). Such approaches included:
    - Establishment of the accelerated approval pathway
    - Consideration of the totality of evidence in the regulatory review
    - Inclusion of Patient Experience Data (PED) in clinical trial design & regulatory processes
    - Utilization of innovative clinical trial designs and real-world evidence (RWE)
- Nearly 1,400 orphan-designated therapies are changing the lives of patients and families, but 95% of rare diseases remain without an FDA-approved treatment.

Progress is Uneven, but Some Developments Can't be Ignored

- A series of FDA actions on rare disease product applications appears to contradict public pledges to expand the use of regulatory flexibility in the evaluation of rare disease therapies.
- At least 23 Complete Response Letters declining to approve rare disease therapies have been issued since the start of 2025, many of which were being considered under the accelerated approval pathway.
- In 2025, the FDA held 65% fewer advisory committee meetings for prescription drugs, biologics, and related topics than in 2024, reducing opportunities for external expertise and patient insights to inform FDA decisions. In some cases, meetings expected to discuss rare disease products that later received a negative regulatory decision were cancelled.

Key Opportunities

Congress can:

- Conduct oversight to better understand the FDA's approach to:
    - The application of the accelerated approval pathway to rare disease therapies;
    - Resolving the inconsistent and unpredictable application of regulatory flexibility; and
    - Resuming the use of Advisory Committee Meetings to receive external expertise on product reviews and key policy topics.
- Provide the necessary resources and direction to optimize the Rare Disease Innovation Hub's ability to improve outcomes for rare disease patients through enhanced coordination and alignment between medical product centers.
- Urge FDA to establish a Rare Disease and Condition Advisory Committee to ensure the Agency can leverage external expertise and patient insights in its approach to rare disease regulatory reviews.

Testimony

Thank you, Chairman Scott, Ranking Member Gillibrand, and distinguished members of the Committee, for convening this critical hearing to explore how the Food and Drug Administration can enhance regulatory clarity and predictability – and foster a more patient-centered, efficient review process for rare disease therapies. These changes, if made, will strengthen U.S. biomedical leadership and ensure lifesaving therapies reach our rare disease patient community as soon as possible.

My name is Annie Kennedy, and I serve as the Chief Mission Officer for the EveryLife Foundation for Rare Diseases. I am especially honored to be here today on behalf of the more than 800 rare disease advocates who have joined us in Washington for Rare Disease Week on Capitol Hill. Our families have traveled great distances and from most every state – including each state represented on this committee – to be here this week – and we each proudly represent the more than 30 million Americans living with rare diseases.[i]

The Orphan Drug Act defines a rare disease as a condition that affects fewer than 200,000 people in the United States. Today, there are more than 10,000 distinct rare diseases,[ii] about 70 percent of which start in childhood.[iii] Some of these are more common, such as Cystic Fibrosis and Duchenne muscular dystrophy. Others are so rare that they are considered N of 1 and are named by their genetic mutation. Collectively, our rare community comprises more than 10% of the U.S. population.

Congressional Efforts Yielded a Movement That Reshaped Methodology in Rare Disease

A stable and predictable regulatory environment is critical to the rare disease therapy development ecosystem. Over the past decade, our rare disease community has seen hundreds of life-altering and life-saving therapies become reality, and we appreciate that each and every day, researchers and drug developers are working to develop therapies for the 95 percent of the community that is still waiting for their first approved therapy.

For patient communities comprised of small numbers whose diagnoses typically occur after long, heartbreaking, and expensive diagnostic odysseys during which the disease has progressed – and function has declined – time is a precious commodity.

Randomized, double blind, placebo-controlled trials that are traditionally conducted in conditions with larger, well characterized, and slowly progressing disease populations are neither appropriate, nor ethical, when considering the challenges and urgency of rare disease.

Congress has long recognized that statutory “regulatory flexibility” is a means to accelerate treatments for patients living with rare diseases. Over the last two decades, your leadership has provided therapy developers and regulators tools that have not only rocketed the United States into renown as the most competitive developer of rare disease products – but most

importantly, have yielded life-changing medicine approvals for thousands of children and adults within our rare disease community.

More than 40 years ago, Congress enacted the landmark Orphan Drug Act (ODA) to create a designation, incentives and other processes to help evolve what to that point had been a largely neglected sector, devoid of approved products. Since that time, Congress has further recognized the complexities and challenges associated with rare disease therapy development – and has unleashed a decade-plus of innovation through the establishment of scientific, clinical, and regulatory infrastructure intended to create an environment of rapid and tailored development.

Through the 21st Century Cures Act, multiple FDA user fee cycles, the recent reauthorization of the Rare Disease Pediatric Priority Review Voucher program, and other actions, we have seen the advent of life saving therapy development incentives, the patient focused drug development movement, the establishment of the FDA Rare Disease Innovation Hub, and application of the accelerated approval pathway to rare disease – all while ensuring that the highest standards of safety and efficacy were upheld.

The application of the accelerated approval pathway to rare disease therapy development called for access to emerging therapies that have achieved safety and efficacy based on the earliest signals of promise, when considered against known alternative disease outcomes. And while over 250 therapies have been approved using the accelerated approval pathway, only 20% [iv] of these have been for rare non-oncological diseases.

As a result of Congress' leadership, rare disease patient advocacy organizations have witnessed improved engagement and understanding of the patient perspective through various approaches, including the Patient-Focused Drug Development workshops, the development of the FDA Benefit-Risk Framework, the formation of rare disease-focused initiatives within CDER and CBER, as well as reporting on the use of patient experience data within the regulatory review process.

Also transforming community engagement and sponsor development, legislation has spurred FDA's issuance of numerous guidance documents that are informing the conduct of patient-focused product development activities for drugs, cell- and gene-based therapies, diagnostics, and medical devices that has been critical to our pipelines.

In fact, nearly 1,400 orphan-designated therapies are changing the lives of patients and families.[v]

These past Congressional efforts yielded a movement that reshaped methodology.

And while this movement and the critical application of methodology have yielded benefit for some, we have only just begun. Still, the vast majority of our communities living with the more

than 10,000 rare diseases still have no FDA approved treatments. To date, fewer than five percent of rare diseases have an FDA approved treatment – and none have been cured.

In other words, the majority of our nation's rare disease community are living with rapidly progressive and debilitating conditions for which there is no treatment. This is the challenge before us today. Unfortunately, despite four decades of positive scientific momentum, progress has stalled.

Momentum Has Shifted

We are here today because our community has experienced worrisome trends with devastating consequences.

While we have been incredibly heartened by announcements flagging support of rare disease therapy development initiatives such as the Rare Disease Evidence Principles (RDEP) framework and the Plausible Mechanism Pathway, our community has experienced a series of FDA actions on rare disease product applications that seem misaligned with recent public pledges to expand the use of regulatory flexibility in evaluating rare disease therapies.

- Since the start of 2025, at least 23 Complete Response Letters (CRLs) declining to approve rare disease therapies have been issued – many of which were being considered under the accelerated approval pathway.

- Several of the recent CRLs include comments that indicate a hesitation to apply regulatory flexibility on issues such as the use of surrogate endpoints, natural history studies, external controls, and real-world evidence.

- Previously, novel product reviews encountering complex discernment might initiate the convening of a product – or topic – specific advisory committee for the inclusion of insights of external experts to inform decision making.

- Yet in 2025, the FDA held 65% fewer advisory committee meetings for prescription drugs, biologics, and related topics than in 2024[vi], sharply reducing opportunities for external expertise and patient insights to inform FDA decisions. In some cases, meetings that were expected to discuss rare disease products – that later received a negative regulatory decision – were cancelled.

Congressional Action to Ensure Today's Patients Will Benefit from Robust Rare Disease Treatment Pipelines

In order to ensure that this generation of patients living with rare diseases benefit from the innovation within our nation's robust therapy pipelines, we ask that Congress conduct oversight of the following:

- The application of the accelerated approval pathway to rare disease therapies;
- Resolving the inconsistent and unpredictable application of regulatory flexibility; and
- Resuming the use of Advisory Committee Meetings to receive external expertise on product reviews and key policy topics.

We also ask that Congress provide the necessary resources and direction to optimize the Rare Disease Innovation Hub's ability to improve outcomes for rare disease patients through enhanced coordination and alignment between medical product centers.

Finally, within the remit of the Rare Disease Innovation Hub, we urge FDA to establish a Rare Disease and Condition Advisory Committee to ensure the Agency can leverage external expertise and patient insights in its approach to rare disease regulatory reviews. While not a product-review committee, this would provide a clear mechanism for FDA to obtain the necessary perspective from rare disease stakeholders to inform this work.

Closing

While the FDA has taken actions to implement rare disease related provisions of the user fee bills and created new rare disease infrastructure through the Rare Disease Innovation Hub, the impact of these and other actions has not yet been fully realized for the benefit of the rare disease community.

The uneven application of rare disease policies and recent actions across the agency are resulting in increased unpredictability and risk that we fear could slow or prevent promising therapies from reaching those who need them most.

At a time when advances in science and understanding of diseases have put life-alerting treatments within reach for many communities, the uneven application of regulatory tools created by Congress is threatening our rare disease patient community's future.

Time is the most precious commodity for rare disease community.

And when a promising therapeutic target faces delays or demise due to the complexities in rare disease and strain on the existing regulatory infrastructure, investment wanes, future scientific promise is unfulfilled, and lives are lost.

About the EveryLife Foundation for Rare Diseases:

The EveryLife Foundation for Rare Diseases is a 501(c)(3) nonprofit, nonpartisan organization powered by the rare disease community to improve health outcomes by driving change through evidence-based policy, leading science-driven policy and regulatory research, activating the community to advocate for their rights and needs, and strengthening the rare disease community.

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[i] National Institutes of Health- National Center for Advancing Translational Sciences. (n.d.). Genetic and rare diseases information center. Genetic and Rare Diseases Information Center. https://rarediseases.info.nih.gov/

[ii] National Institutes of Health- National Center for Advancing Translational Sciences. (n.d.). Genetic and rare diseases information center. Genetic and Rare Diseases Information Center. https://rarediseases.info.nih.gov/

[iii] Nguengang Wakap S, Lambert DM, Olry A, Rodwell C, Gueydan C, Lanneau V, et al. Estimating cumulative point prevalence of rare diseases: analysis of the Orphanet database. Eur J Hum Genet. 2020;28(2):165–73.

[iv] Expediting treatments in the 21st century: orphan drugs and accelerated approvals | Orphanet Journal of Rare Diseases | Springer Nature Link

[v] Food and Drug Administration: Office of Orphan Products Development. (n.d.)*Orphan Drug Product Designation Database*. https://www.accessdata.fda.gov/scripts/opdlisting/oopd/index.cfm

[vi] https://insights.citeline.com/pink-sheet/product-reviews/us-advisory-committees/us-fda-sees-advisory-committee-volume-collapse-in-2025-T265ZITIFREIRBHA7PFGREWACM/