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

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 include:
    - 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 we Must Keep Moving Forward

- In recent months, new policies and initiatives were announced flagging agency leadership support for rare disease therapy innovation.
- Yet, a series of FDA actions on rare disease product applications appears misaligned with new commitments 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 were 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. In some cases, meetings expected to discuss rare disease products that later received a negative regulatory decision were cancelled.
- The Special Senate Committee on Aging is exploring opportunities to improve the FDA's approach to rare disease product applications during a hearing on February 26, 2026.

To Support the Implementation of Regulatory Flexibilities as Congress Intended, Congress Can:

- **Conduct outreach to the Agency to better understand its approach to:**
    - **The application of the accelerated approval pathway to rare disease therapies;**
    - **Improving the predictability and consistency of the 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.**
- **Support policies that enable the FDA to leverage expert and patient insights to solve challenging regulatory science issues, like the establishment of a Rare Disease and Condition Advisory Committee and a Science-Focused Drug Development initiative, as proposed in the EXPERT Act (H.R. 1532/S. 822).**

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