15TH SCIENTIFIC WORKSHOP ACTION PLAN

THE EVERYLIFE FOUNDATION FOR RARE DISEASES PRESENTS

A Proposal to Ensure that Scientific Innovation, Evolving Regulatory Tools, & Policy Progress Yield Scalable Outcomes for All Rare Disease Communities

Background

The community-led regulatory action plan was developed during the 15th EveryLife Foundation Scientific Workshop on May 12, 2026. Established to create a forum to address the scientific, regulatory, and policy barriers that slow the development of rare disease therapies, the EveryLife Foundation Scientific Workshop series has consistently focused on advancing practical, evidence-driven solutions.

The EveryLife Foundation for Rare Diseases convened its 15th Scientific Workshop, "Making What Matters Count: Advancing Patient-Centered Rare Disease Therapy Development Methodologies, Tools, and Knowledge Management," at a moment of both unprecedented scientific opportunity and persistent challenges across the rare disease ecosystem. While momentum across the rare disease community has been strong due to the advent of patient-focused drug development tools, innovative statistical designs, and regulatory pathways that recognize the complexity of rare disease, their application has been unpredictable and inconsistent. Many rare disease communities are navigating a number of challenges today, including the absence of natural history data, limited investment, difficulties in establishing and validating meaningful endpoints, and barriers to generating the evidence needed to sustain development programs. The workshop centered on a fundamental question: **Where are recent advances in rare disease regulatory infrastructure, guidance, and legislative policy truly working, and where must we do better?** Coming on the cusp of Congressional activity to reauthorize the FDA's user fee programs, the discussions were timely and will inform rare disease legislative priorities for this upcoming reauthorization.

Throughout the workshop, patient advocates, biopharmaceutical leaders, academic researchers, and federal agency officials explored how greater transparency, consistency, coordination, and knowledge sharing can help ensure that scientific innovation, evolving regulatory tools, and policy progress translate into scalable outcomes for all rare disease communities. ***The yield was an action plan intended for rapid, collective implementation. These actions will enhance the recommendations for regulatory actions and the rare disease priorities for PDUFA VIII, resulting in a comprehensive roadmap in July 2026.***

Contributors

32.52%	37.40%	21.14%	8.94%
Patient and Caregiver Advocate	Industry	Academia/Medical Professionals/Other	Agency Partners

Recommendations – Potential Actions to Meet the Moment

Legislative Actions

- Amend the Food, Drug, and Cosmetic Act (FDCA) to authorize a mechanism for the FDA to share de-identified elements associated with non-approvals in the pre-competitive space, including possible incentives as well as directives to support such sharing.
- Consider the benefits of removing the statutory exemption from PREAs pediatric testing requirements, considering the experiences in oncological diseases.
- Authorize and appropriate sufficient levels of funding to support:
  - The Rare Disease Innovation Hub
  - The Orphan Products Grant Program
  - A mechanism to support pre-competitive novel endpoint development

Regulatory Actions

- Better explain how the agency applies regulatory flexibilities to rare disease product reviews, including both through the level of individual reviews and a possible guidance document or other actions.
- Expand the Rare Disease Innovation Hub's Strategic Agenda to include a mid-to-long-term focus on initiatives to evolve from case studies to more real-time generalizable rare disease learnings across FDA centers and divisions.
- Establish or update data standards and create standard informed consent language to remove barriers to broader data sharing.
- Ensure early interactions with sponsors include discussion about anticipated use of patient experience and related data, comprising real-world evidence/real-world data, to ensure maximum levels of alignment between the agency and sponsors.
- Improve the available guidance to provide more detail for industry on how to complete the PED checklist and what information to provide the agency regarding how PED submitted informed decision-making throughout the trial.
- Create formal meeting structures that enable patient organization participation in sponsor meetings when relevant and create opportunities for patient groups to discuss their precompetitive drug development initiatives.

Knowledge Management Actions

- Establish a voluntary industry precompetitive platform to voluntarily share failed trial data or other learnings to benefit the overall field.
- Create knowledge management systems that leverage the use of artificial intelligence and other tools to enable reviewers across the agency to access information about parallel situations.
- Provide guidance and direction to patient advocacy organizations to support therapy development efforts, including guidance on natural history studies and endpoints.
- Establish data standards, through CPATH or other conveners, to enable and facilitate initiatives to pool data across studies and projects and ensure that it is of regulatory grade.



Themes

Consistent and Transparent Use of Regulatory Flexibility Tools

Stakeholders need greater clarity on how the FDA applies existing regulatory flexibilities in rare disease reviews. Increased transparency around the agency's decision-making, including explanations for differing outcomes across seemingly similar applications, would improve understanding and help inform future development strategies. The FDA has greater latitude in addressing uncertainties in the quantity of evidence, including the use of smaller trials, underpowered studies, confirmatory evidence generation, and other alternative approaches, when the quality of the data is sufficiently robust and scientifically justified. That regulatory flexibility must remain context-specific and cannot be applied uniformly across all applications underscores the importance of clearly communicating the factors that inform the agency's exercise of judgment in individual cases.

Improvements in Data Sharing

There is an urgent need for stronger policies, incentives, and infrastructure to support pre-competitive data sharing in the rare disease field. Valuable lessons from failed or discontinued programs are often lost due to constraints on what the FDA can share, the rapid unwinding of programs that do not meet their endpoints, and the lack of standardized mechanisms or incentives for sharing non-product-specific insights. Greater access to these insights could help prevent duplication of failed approaches, accelerate scientific understanding, and better inform future development decisions across a therapeutic area. Patient organizations can facilitate data sharing, but they need resources, infrastructure, and guidance.

Improve Availability of Natural History Data

For many communities, strong natural history data is the foundation for viable therapy development, but obtaining regulatory-grade data is costly and time-consuming. Early engagement between sponsors and the FDA to align on types of data (e.g., longitudinal studies, registry data) and how they can be used in development programs is essential. Policy must incentivize the development of more meaningful natural history data.

Evolve Patient Focused Drug Development

Over the past 15 years, policies advancing patient-focused drug development have significantly strengthened the integration of patient perspectives into the development and evaluation of rare disease therapies. Patient experience data has gone from a "nice to have" to an essential element of a regulatory submission. Despite this progress, continued efforts are needed to ensure the consistent and meaningful inclusion of the patient voice early and often, and to provide greater clarity on how patient experience data informs regulatory decision-making. Patient groups are increasingly conducting regulatory-grade, pre-competitive development work and need opportunities to engage directly with the agency outside of sponsor-specific meeting mechanisms.

Optimize the Role of the Rare Disease Innovation Hub

If Congress provides appropriate resources, the Rare Disease Innovation Hub can build on its foundational work to drive cross-FDA knowledge management initiatives that enable reviewers to leverage information on prior regulatory approaches to similar issues. The Hub is poised to play a significant role as a convener and a source for translating and disseminating regulatory learnings in rare diseases, enhancing stakeholders' understanding of what the agency needs and expects. With appropriate resources, the Rare Disease Innovation Hub can transform how rare disease community stakeholders, clinical and scientific experts, and product developers engage with the Agency.

EVERYLIFE FOUNDATION FOR RARE DISEASES

About the EveryLife Foundation

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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