FDA Rare Disease Innovation Hub Meeting
October 16, 2024
Oral Testimony – Annie Kennedy

Good morning – I am Annie Kennedy, and I am pleased to provide remarks on behalf of the EveryLife Foundation for Rare Diseases.

My comments today reflect the insights of our diverse coalition comprised of patient organizations, industry leaders, and other relevant stakeholders who together inform our policy efforts.

Since convening a series of townhall meetings to assess rare disease community priorities -- followed by our Scientific Workshop in 2018 -- our community has advocated for the creation of an inter-center institute for rare diseases to serve as the FDA's coordinating office for the optimization of rare disease expertise, processes, and engagement with rare disease stakeholders.

While the Rare Disease Innovation Hub will not be the formal inter-center institute proposed by hundreds of patient advocates (many of whom are pictured above or are here today) -- we are delighted by the progress that today signifies.

We believe that the Hub can achieve similar goals if its foundational documents, leadership structures, operational guidelines, and metrics for community impact are thoughtful, thorough, and transparent. **We urge the prioritization of projects and infrastructure that will enhance consistency and improve the predictability of how any given office, division, or center will approach the application of regulatory flexibility.**

Highlights of the initiatives we recommend the Hub undertake include:

- **Establishing Cross-functional Consultation Processes**
  that will enable internal experts to be efficiently consulted at the request of either FDA staff or a sponsor.

- **Supporting the Creation of a Knowledge Management System**
  that will increase access to - and awareness of - information on how FDA has approached the application of regulatory flexibility in rare disease.

- **Supporting the Dissemination of FDA Pilot Program Learnings & Data**
  to enable rapid application of information to relevant rare disease work.

- **Advancing Ultra-rare Policy**
    - By issuing guidance that can operationalize consistent approaches to evaluating ultra-rare therapies within the FDA's existing authority.

- **Enhancing Advisory Committee's Rare Disease Knowledge**
    - by establishing a Rare Disease Advisory Committee within the Hub to serve in a consultative and disease agnostic approach, enabling external experts to support review committees across the Centers, and providing guidance on emerging issues.

We look forward to a continued productive partnership with the FDA to ensure that the Rare Disease Innovation Hub fulfills its mission to streamline and transform the regulatory pathways for rare diseases. It is our hope that FDA will commit to sustained and robust implementation, inclusive of all three product centers -- and incorporating meaningful engagement with rare disease stakeholders and agency experts.

Thank you.