Please Support Funding and Report Language in FY 2027 for the Rare Disease Innovation Hub

In the House – Please join a sign-on letter led by the Co-Chairs of the Rare Disease Congressional Caucus, Representatives Gus Bilirakis (FL-12) and Doris Matsui (CA-07)

In the Senate – Please include the Rare Disease Innovation Hub funding and language request in your office's FY27 priorities

Request:

Bill: Agriculture, Rural Development, Food and Drug Administration, and Related Agencies
Section: Food and Drug Administration, Office of the Commissioner, Rare Disease Innovation Hub
Request: Provide $5 million for the Rare Disease Innovation Hub, and support the following language:

Rare Disease Innovation Hub – The Committee is pleased with the work of the FDA Rare Disease Innovation Hub in its first full year of operations, including its launch of the RISE Workshop Series and its focus on improved alignment and coordination of rare disease activities across FDA functions. The Committee appropriates $5,000,000 to support the Hub's work and implementation of its current strategic agenda, particularly actions to expand opportunities for patient and clinical community stakeholders to engage earlier and more fully in the therapy development, and to enhance implementation of efforts to support and sustain cross-center consistency on rare disease review standards.

Background:

The Rare Disease Innovation Hub was established to enhance collaboration across the FDA to address common scientific, clinical, and policy issues related to rare disease product development[1].

A sustainable and appropriately resourced Rare Disease Innovation Hub is needed because:

- ✓ Only 5% of the more than 10,000 rare diseases have FDA-approved treatments.
- ✓ Treatment development and evaluation for very small populations requires specific expertise and a consistent approach to applying regulatory flexibility per Congress's intent.
- ✓ Rare disease expertise is unevenly dispersed across the FDA, limiting opportunities to share best practices and advance rare disease regulatory science.

The broad rare disease community supports the work of the Rare Disease Innovation Hub:

- ✓ The STAT Act, supported by hundreds of patient organizations, originally called for the creation of a similar function within the FDA to improve coordination for rare diseases, stakeholder engagement, and policy development, as described further here.

[1] U.S. Food and Drug Administration. (n.d.). FDA Rare Disease Innovation Hub. https://www.fda.gov/industry/medical-products-rare-diseases-and-conditions/fda-rare-disease-innovation-hub

- ✓ In 2024, rare disease stakeholders supported report language calling for the FDA to form a rare disease center of excellence, language that would ultimately lead to the formation of the Rare Disease Innovation Hub in late 2024.
- ✓ Since its creation, over 4,000 community members have engaged with the Hub through its RISE Workshop Series, and more than 100 organizations have connected directly with the Hub about its goals and FDA regulatory practices.

The Rare Disease Innovation Hub is a strategic and efficient approach to improve the FDA's rare disease actions and impact for the 30 million Americans living with rare diseases:

- ✓ In 2025, the Hub's first full year of operations, with only one full-time employee and support of staff on-loan from other product Centers, it built cross-center collaborations through the Rare Disease Policy and Portfolio Counsel, hosted two RISE Workshops tackling specific regulatory science issues applicable across rare disease communities, and led the development of new evidence principles for rare disease therapy development.
- ✓ The Hub's 2026 Strategic Plan lays out an ambitious agenda to grow its role in facilitating the dissemination and uptake of rare disease best practices across the Agency, host three additional RISE Workshops on timely topics that can catalyze therapy development, create opportunities for rare disease patient organizations and experts to inform the therapy development and evaluation process, and streamline the navigation of FDA's rare disease resources.
- ✓ 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.

For House Members interested in joining the sign-on letter, please use the Quill link, https://quill.senate.gov/letters/letter/32469/opt-in/view/5e9d0916-a96f-4e72-831d-b32d9070ffb2/, also available via the QR Code below, or contact:

Leigh Ann Fairley, Congressman Gus Bilirakis's office at LeighAnn.Fairley@mail.house.gov or Jackie Weinrich, Congressman Doris Matsui's office at Jackie.Weinrich@mail.house.gov.

For general inquiries about the Rare Disease Community's Appropriations Priorities for the Rare Disease Innovation Hub, please contact: Dylan Simon, Senior Director of Policy, at dsimon@everylifefoundation.org.