



Support the Creation of an Interagency Coordinating Committee for Rare Diseases

Please include report language supporting the creation of the ICC in your appropriations requests.

Overview

Agencies across the federal government have long implemented policies and programs to support rare disease patients and their families. The expansion of newborn screening to include rare diseases, the FDA's Accelerating Rare Disease Cures Program, and the recently announced CMS Cell and Gene Therapy Access demonstration project are all examples of federal initiatives that focus on the rare disease community. Beyond specific initiatives like these, federal agencies provide services, make policies, and prioritize resources on a host of issues that are relevant to the health and quality of life for people living with rare diseases. Considering the expansive scope of federal initiatives and the substantial unmet needs within the rare disease community, it is imperative to establish a mechanism that fosters optimal coordination and collaboration and ensures resources are efficiently utilized.

What Types of Roles Does the Federal Government Fill Related to Rare Diseases

The federal government's role in rare disease policy is broad and includes:

- Funding a robust biomedical research enterprise spanning from basic research to clinical trial grants to support important innovation;
- Reviewing and approving rare disease therapies, ensuring that both the drugs are safe and effective while working to approve therapies in a timely manner;
- Supporting public health infrastructure that help us understand the impact of rare diseases;
- Providing services that address the needs of patients and families beyond medical challenges; and
- Delivering benefits as part of our safety net system.

Proposal

The Department of Health and Human Services (HHS) has a long history of convening interagency coordinating committees on topics related to public health and in specific disease areas. Interagency committees elevate the awareness of the associated issues, provide forums for collaboration across agencies and even departments and ensure effective and efficient resource utilization. **Please support the inclusion of the below report language in the Labor, Health and Human Services, Education, and Related Agencies FY25 appropriations bill:**

Rare Diseases – The Committee understands the unique challenges the rare disease community faces throughout both the healthcare and research systems. There are critical, ongoing federal efforts at multiple agencies to improve the lives of members of the rare disease community, and they are optimized when conducted in collaborations. This Committee encourages the Secretary of HHS to provide additional leadership to better coordinate these efforts across the federal government through the creation of an Interagency Coordinating Committee for Rare Diseases. The coordinating committee should provide an annual report assessing all federal agency activities concerning rare diseases, including but not limited to regulatory flexibilities in rare disease reviews, projects involving two or more agencies, overall programmatic funding, and other potential measurable outcomes to include in future reports. This information will help HHS further bolster and improve its many efforts to address the many challenges of the rare disease community.

Point of Contact

If you have questions, please contact either Jamie Sullivan (jsullivan@everylifefoundation.org) or Dylan Simon (dsimon@everylifefoundation.org) at the EveryLife Foundation for Rare Diseases.