Congress of the United States
Washington, DC 20510

May 1, 2024

The Honorable Andy Harris, M.D.
Chairman
Agriculture, Rural Development, FDA,
& Related Agencies Subcommittee
2362-A Rayburn House Office Building
Washington, D.C. 20515

The Honorable Sanford Bishop, Jr.
Ranking Member
Agriculture, Rural Development, FDA,
& Related Agencies Subcommittee
1036 Longworth House Office Building
Washington, D.C. 20515

Dear Chairman Harris and Ranking Member Bishop,

As you develop the Fiscal Year 2025 Agriculture, Rural Development, Food and Drug Administration and Related Agencies Appropriations Act, we write to urge that you include report language to strengthen how the FDA reviews therapies to treat rare diseases and conditions, conditions that combined affect more than 30 million Americans and cost about $1 trillion annually in health and non-health expenditures.[1] Specifically, we are requesting that you include language directing the FDA to establish an Intercenter Institute – or Center of Excellence – focused on rare diseases.

An FDA Intercenter Institute on Rare Diseases will help better coordinate activities across the FDA focused on development and review of therapies – including drugs, cell and gene therapies, and medical devices – to treat rare diseases. Intercenter Institutes were authorized by the 21st Century Cures Act. Those established to date focus on an array of diseases or topics including oncology, digital health, and drug compounding. While each center is unique, they are united by shared aims of improving knowledge, strengthening working relationships and opportunities for collaborations, and driving efficiencies to expedite their work. We believe an Intercenter Institute focused on rare diseases is urgently needed to build upon existing agency activities and ensure the FDA is positioned to meet future needs and challenges.

While progress has been made in developing therapies to treat rare diseases in the more than 40 years since the Orphan Drug Act was enacted, the reality is that most rare disease – 95 percent – lack any FDA approved treatments. An Intercenter Institute focused on rare diseases will help better organize FDA expertise on rare disease therapy development, such as expertise in statistics, trial design, and other factors associated with studies involving small populations, to achieve efficiencies in the review process. It will also help eliminate disparities in rare disease knowledge and expertise across centers or review divisions to help expedite the review of any rare disease therapeutic application. This will be particularly important as the pace of innovation continues, particularly in novel areas like gene therapy. Finally, an Intercenter Institute for Rare Disease will provide a single clear home for all of FDA's current and future rare disease activities, providing clarity for all stakeholders including product developers and patients.

To advance this initiative, we request that you include the following report language provision in the FY 25 Agriculture, FDA report:

[1] See https://www.healthaffairs.org/content/forefront/economic-burden-rare-diseases-quantifying-sizeable-collective-burden-and-offering

***Intercenter Institute for Rare Disease**s. – The Committee recognizes that the development of rare disease therapies offers unique regulatory challenges, such as the small patient populations for clinical studies, poorly understood natural history, and hurdles in manufacturing and commercialization. To ensure that therapies for rare diseases remain a top priority for the FDA, the Committee requests that within one year, the agency establish an Intercenter Institute or Center of Excellence for rare diseases under existing authorization from Section 3073 of the 21st Century Cures Act (P.L. 114-255). This Intercenter Institute should serve as the FDA's coordinating office for engagement with rare disease stakeholders across all therapies including drugs, cell and gene therapies, and medical devices. The Committee requests that the Intercenter Institute convene a Part 15 Hearing to inform the establishment of the Intercenter Institute and promulgate draft guidance detailing how existing guidance interpreting the evidentiary standard established by the Drug Amendments of 1962(P.L. 87-781) could be revised in the face of the unique development challenges posed by rare diseases. Lastly, the Committee asks that the FDA submit a report to Congress within six months after establishing the new Intercenter Institute that outlines its progress to date, including the status of its Part 15 Hearing, summary of the charter (such as the responsibilities of the Chair, required participants, and criteria for initial populations and regulatory review), and any other relevant information the agency deems appropriate.*

On behalf of the more than 30 million Americans impacted by rare disease, we urge you to consider this request and thank you for considering this important request. If you have any questions or need additional information, please do not hesitate to contact us.

Sincerely,

Gus M. Bilirakis
Member of Congress

Doris Matsui
Member of Congress