Task the US Food and Drug Administration With Creating a *Rare Disease Center of Excellence*

Subcommittee: Agriculture, Rural Development, Food and Drug Administration, and Related Agencies

Title: Rare Disease Center of Excellence

Requesting Organization: EveryLife Foundation for Rare Diseases

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Agency/Program: Food and Drug Administration, Office of the Commissioner

Requested Report Language:
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Intercenter Institute convene a Part 15 Hearing to inform the establishment of the Intercenter Institute and promulgate draft guidance detailing how existing guidance interpreting the evidentiary standard established by the Drug Amendments of 1962(P.L. 87-781) could be revised in the face of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.

Justification:
Over the past four decades, tremendous strides have been made in treating some rare diseases and disorders, those conditions that affect less than 200,000 people in the United States. Individually, rare diseases may affect relatively small populations compared to highly prevalent conditions such as heart disease, but collectively they take a major toll with estimates suggesting one of every 10 Americans is living with a rare disease. The enactment of the Orphan Drug Act and refinement of patient engagement over the last decade has made a tremendous impact on the rare disease community, but we must continue to build on that progress to ensure that the remaining 95 percent of rare diseases communities without an FDA-approved drug can share in the progress of the last 40 years.

Over the years, various FDA initiatives have attempted to create a framework for communication within the Agency. For example, from 2016 to 2019, the FDA operated a Rare Disease Council, which was able to address many of the obstacles facing patients, industry, and the Agency. The council comprised FDA medical product center directors, the director of the Office of Orphan Products, and, at the discretion of a center director, a programmatic lead from that center. Each center would present an active issue (be it policy, or product, related) for discussion with the goal of resolving any controversial or difficult questions. The ultimate aim was to reach consensus, which invariably was achieved—the charter specified that if consensus was not achievable, the RDC would raise the issue with the Commissioner for resolution. The Rare Disease Council has since been allowed to wither, but its work is more important today than ever.

Additionally, as part of the CDER reorganization, FDA established an Office of Rare Diseases, Pediatrics, Urologic, and Reproductive Medicine Division of Rare Diseases and Medical Genetics, and, more recently, it has been said that a Chief Medical Officer within the office of the Commissioner as part of the pending FDA reorganization will be tasked with coordinating on issues including rare diseases.

While appreciated, these efforts are not sufficient to address the rare disease regulatory challenges. Small patient populations create challenges that require broad FDA expertise to address. Identifying the natural progression of disease, disperse clinical trial sites, the ability to detect clinically meaningful outcomes, and alternative clinical trial design are all common across the rare disease community, but can be unique for a review team. In addition, the dispersion of rare disease experts across the entire FDA limits the ability to share best practices on how to address these challenges.

Through the 21st Century Cures Act, the FDA received the authority to establish one or more intercenter institutes for a major disease area or areas. Just one month after the Cures Act was enacted, the FDA established the Oncology Center of Excellence, which has proven a resounding success, having launched pilot programs to expedite authorization to co-develop diagnostics and therapeutics, to allow the use of less complex trial designs, and to address health inequities, among many more accomplishments. The creation of a Rare Disease Center of Excellence would have a similar impact.

A Rare Disease Center of Excellence would help to bring together the extensive rare disease expertise across the FDA in one central location. A Center of Excellence would help organize all FDA resources – such as statisticians, regulatory scientists and experts in clinical trial design for small populations – within a single structure to avoid duplication and disciplinary silos as well as to make concentrated resources available to multiple review divisions. It would recognize that despite the wide diversity in clinical symptoms and organ systems affected by rare diseases, the barriers to effective therapeutic development are similar for all rare diseases.

Stakeholder supporting this report language request: EveryLife Foundation for Rare Diseases