



## **Rare Disease Center of Excellence Sign-On Letter**

### **PLEASE SIGN LETTER IN SUPPORT OF A RARE DISEASE CENTER OF EXCELLENCE AT THE FDA**

**To sign this letter please contact Elyssa Malin with Sen. Klobuchar, Kirby Miller with Sen. Wicker, Caitlin Van Sant with Rep. Butterfield or Shayne Woods with Rep. Bilirakis**

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#### **Background**

- A Center of Excellence (COE) within the FDA would elevate rare disease expertise within the agency and help to remove communication barriers across centers and divisions to accelerate and improve the rare disease therapy development process at the U.S. Food and Drug Administration (FDA).
- The FDA already has authority under the 21<sup>st</sup> Century Cures Act to establish Centers of Excellence.
- Three years ago, the FDA established the first FDA Center of Excellence focused on oncology (the Oncology Center of Excellence) which has been extremely successful in bringing new cancer therapies to patients.
- Two years ago, the EveryLife Foundation hosted a Scientific Workshop on this topic with case studies from patient organizations and industry that demonstrated the value of having such a Center of Excellence.
- Given the challenges and, therefore, the unique expertise needed to advance the development and review of products for rare diseases, innovators have long believed that a Rare Disease Center of Excellence would provide the necessary resources and support to allow offices across FDA to more consistently and efficiently review novel products for these rare conditions.

#### **Vision**

- This COE would serve as a consultative and cross-cutting body to build knowledge and capacity, and to consult with review divisions in considering applications for rare-disease therapies.
- The COE would identify and address current and emerging challenges and opportunities in rare disease therapy development, including the development of therapies for individual or very small populations.
- The COE would not supplant any authorities held by FDA review divisions.
- The Rare Disease Center of Excellence could also include a dedicated program focused on the many unique needs associated with developing treatments for individual or very small populations (e.g., N of 1).

*Rare Disease Legislative Advocates (RDLA) is a program of the EveryLife Foundation for Rare Diseases designed to support the advocacy of all rare disease patients and organizations. RDLA is committed to growing the patient advocacy community and working collaboratively, thereby amplifying the patient voice to be heard by local, state, and federal policy makers.*

**Please contact Shannon von Felden ([vonfelden@curetheprocess.org](mailto:vonfelden@curetheprocess.org)) to learn more about RDLA.**