November 10, 2015

Dear Chairman Alexander and Ranking Member Murray,

The EveryLife Foundation for Rare Diseases strongly supports the prompt confirmation of Dr. Robert Califf as the next Commissioner of the Food and Drug Administration (FDA). The FDA plays a critical role in the development and review of new treatments for patients affected by rare diseases, and having a confirmed leader is essential for the Agency to function optimally.

Dr. Califf lead a distinguished career as the head of the Clinical Trials Transformation Initiative, an organization focused on improving the quality and efficiency of clinical trials. This mission has never been more vital at a time when rare disease patients face a staggering 7,000 diseases, only 400 of which have FDA-approved treatments. In order to successfully treat these diseases, and those that have yet to be discovered, we will need to consider new paradigms and approaches to accelerate the clinical trial process. Dr. Califf’s experience in this area will be instrumental as the FDA considers new tools to accelerate clinical trials such as expanding the use of biomarkers and Bayesian adaptive trial designs.

Patient engagement has been a theme throughout Dr. Califf’s career, evident in his work at CTTI and as a co-principal investigator at PCORnet, a multi-stakeholder initiative to improve healthcare. The patient voice is an essential component for determining disease burden and the impact of therapies in clinical development. This is especially true for rare diseases, as clinical knowledge may be limited and investigators have small patient populations to study and engage.

Dr. Califf has the experience necessary to help foster and accelerate medical innovation on behalf of rare disease patients and all Americans. The Foundation strongly endorses him for the role of Commissioner and urges the Senate to act swiftly to confirm him.

Sincerely,

[signature]

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