

Dear Representative,

The # undersigned organizations represent many of the nearly 30 million patients who live with rare diseases in the United States. We write to you to express our enthusiastic support for the two pieces of legislation designed to expand the Orphan Drug Act (ODA) on the 40th Anniversary of this pivotal law. Both "Leo's Law" (H.R. 1805) and "Cameron's Law" (HR 1350), introduced by Representatives Josh Gottheimer (D-NJ) and Don Bacon (R-NE), would improve upon the incentives provided in the ODA and spur innovation in the development of treatments for the over 7,000 rare diseases.

Despite being called rare diseases, those affecting fewer than 200,000 individuals, these conditions collectively impact 1 in 10 Americans. Rare diseases are notoriously challenging to diagnose, with patients often enduring years of uncertainty before receiving an accurate diagnosis. This delay leads to unnecessary suffering and exacerbates the burden on patients and their caregivers. Even more concerning, a staggering 95% of these rare diseases lack Food and Drug Administration (FDA)- approved treatments¹, leaving countless individuals without access to lifesaving or life-improving interventions which compromises quality of life and causes unnecessary morbidity.

The COVID-19 pandemic further exacerbated the obstacles faced by rare disease patients by disrupting their access to clinical trials, causing delays and uncertainty in the development of potential therapies. Leo's Law offers a comprehensive solution to address these critical issues faced by the rare disease community, aiming to address these challenges head-on by providing a framework that ensures continuity and resilience in rare disease clinical trials.

Cameron's Law would restore the tax credit for clinical testing expenses from 25% back to the original ODA level of 50%. Increasing the tax credit to the original parameters would make it more economically feasible for drug companies to develop rare disease treatments and provide the needed certainty for continued investment.

We would sincerely appreciate your championship of this critical issue for rare disease patients and believe that passage of both Leo's Law and Cameron's Law will have a lasting impact on the lives of countless individuals and families within the rare disease community. By ensuring that Congress continues to support and enhance the proven success story of the ODA, we can offer hope to patients who are all too often marginalized due to the rarity of their conditions.

Sincerely,

¹ National Institutes of Health. (2023, January). Delivering Hope for Rare Diseases. National Center for Advancing Translational Sciences. https://ncats.nih.gov/Giles/NCATS_RareDiseasesFactSheet.pdf.