A Call to Action

Amyloidosis Research Consortium

Rare Diseases are Not a Rare Problem

1 in 10 people live with a rare disease. A rare disease is defined as a condition affecting 200,000 or fewer patients. When combined, these diseases are not “rare” as they affect more than 30 million Americans.

Barriers to Drug Development for Rare Diseases

For many rare diseases, the potential to develop treatment now exists due to successful scientific advances. However, the cost and complexities of the clinical trial process combined with the challenging regulatory environment means that development has slowed or even stopped progress.

About Amyloidosis

Systemic amyloidosis is a group of progressive and fatal rare diseases that cause damage to organs including the heart, kidneys, liver, GI tract, and nervous system, leading to multi-organ failure and death. There are many different types of amyloidosis, some of which are hereditary and ravage generations of a family, whereas others are acquired over time for reasons we still don’t understand.

Despite the fact that each type of amyloidosis is so different and recent advancements have included the approval of new therapies for some of the more common types, experts agree that universally all types of amyloidosis are significantly underdiagnosed and none of the current treatments have shown to be curative.

Medical challenges include the lack of diagnostic tools, natural history studies and adequate therapies to treat these multisystemic, heterogeneous diseases. There is a massive unmet medical need in this population.

Act Now

Together, we need to advance the development of potentially lifesaving treatments by accelerating innovation for rare disease treatments through science-driven public policy. We can do more with the science we already have and bring life-saving treatments to millions of people suffering from rare diseases by improving the regulatory process from clinical trials through approval and market access, ensuring safe and effective treatments.

We Need You to:

Support the Rare Disease Caucus

The Caucus helps bring Congressional awareness to the needs of the rare disease community and creates opportunities to address roadblocks in access to and development of crucial treatments. The Caucus gives a permanent voice to the rare disease community on Capitol Hill.

Protect the Orphan Drug Tax Credit

The current draft of the Build Back Better Act includes language to amend the Orphan Drug Tax Credit (ODTC) in ways that would severely undermine efforts to ensure more patients have access to a safe and effective drug for their rare condition. Protect the Orphan Drug Tax Credit by urging Senate leadership to remove Section 128141 from the Build Back Better Act.

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