Impact Statement

Contributions to society

Kelly established the first effective treatment for a human amyloid disease. His pioneering work catalyzed the development of multiple aggregation-modulating therapies now targeting transthyretin amyloidosis, light chain amyloidosis, and other protein misfolding disorders. As many as 15% of individuals worldwide over the age of 50 suffer from diseases linked to amyloid accumulation. Hence the therapeutic strategy Kelly established has enormous potential impact.

Impact on TTR amyloidosis patients

Tafamidis, discovered in Jeff Kelly's laboratory at The Scripps Research Institute, has proven to be a safe and highly effective therapy for transthyretin amyloidosis. More than 65,000 patients have been treated, prolonging both their healthspan and lifespan, especially when early treatment is possible, and this is the most important aspect of Kelly's work.

Before Kelly's work, only liver transplantation slowed hereditary TTR amyloid disease progression. Tafamidis paved the way for additional TTR-directed therapies, including gene-silencing agents that reduce TTR production. Currently, six aggregation-modulating strategies are approved for TTR amyloidosis, transforming patient prognosis.

Impact on understanding amyloid disease etiology

Kelly provided the first pharmacologic evidence that transthyretin aggregation directly causes post-mitotic tissue degeneration. Clinical outcomes demonstrating that aggregation inhibition slows disease progression decisively validated the aggregation hypothesis in humans. This achievement shifted scientific and medical consensus toward embracing aggregation modulation as a viable therapeutic strategy in amyloidosis and neurodegeneration.

Impact on the field of amyloidosis

In the early 1990s, Kelly and Ron Wetzel demonstrated that conformational change alone is sufficient to convert proteins into amyloid fibrils. This foundational work helped define the molecular basis of amyloidosis. Kelly's subsequent success in therapeutically stabilizing protein conformation motivated broad scientific and commercial investment in aggregation-modulating strategies and helped drive the development of additional approved therapies.

Impact on the economy

Kelly's work catalyzed a multibillion-dollar and rapidly expanding therapeutic market for amyloid diseases. More importantly, his strategy of pharmacologically controlling protein conformation has influenced therapeutic development in other protein misfolding disorders. He also demonstrated that earlier intervention yields greater benefit in amyloid disorders—a principle that is being broadly applied across this class of diseases.