Amyloid Treatment and Research Program key research findings:

- Definition of the electron microscopic structure and x-ray diffraction pattern of amyloid fibrils in 1967, providing key insight and first guiding principles in understanding the unique characteristics of insoluble amyloid proteins.
- Development of a procedure for isolating and purifying amyloid proteins from tissues, making possible biochemical analyses and precise identification of the proteins.
- Creation of an animal model of secondary amyloidosis using casein injections, permitting testing of AA amyloid formation and treatment in a controlled laboratory environment.
- Identification of the protein deposits in familial amyloidosis as transthyretin, which for the first time defined the biochemical nature and source of the amyloid fibril in this form of amyloidosis.
- Characterization of the protein deposits in dialysis-associated amyloidosis as β2-microglobulin, a protein not properly cleared from the circulation by dialysis membranes.
- Development of a simple electrophoretic method to differentiate primary and hereditary amyloidosis, through an isoelectric focusing technique to identify transthyretin mutant protein in serum.
- Construction of ALBase, a public database of containing >3000 deidentified light chain nucleotide and amino acid sequences available to researchers worldwide.
- Development of mouse models of AL amyloidosis for studying the process of AL amyloidogenesis.
- Development of siRNA approaches for reducing AL light chain production, and collaborating with Alnylam Co. on siRNA for ATTR.
- Development of novel methodologies for analysis of transthyretin (TTR) aggregates in serum and tissues for better understanding of familial and age-related transthyretin amyloidosis.