Amyloidosis (am-uh-loi-doh-suhs) is the term for a group of diseases that involve the buildup of abnormal protein, called amyloid deposits, which progressively damage and impair the function of tissues and organs. Amyloidosis can be localized or systemic, and it can be acquired or genetically inherited. The most common types include AL, which is caused by the malformation or "misfolding" of proteins produced by the bone marrow; AA, which relates to a protein that the body produces in response to inflammatory conditions like rheumatoid arthritis and inflammatory bowel disease; Aβ₂-M, which usually occurs during end stage kidney failure after a patient has been on dialysis for many years; and ATTR, which involves a protein produced in the liver. ATTR amyloidosis can be hereditary (hATTR) or wild type (wtATTR), which is not genetically inherited. Amyloidosis can be life-threatening, and despite remarkable advances in treatments due to recent research, there is currently no cure.

TODAY

All types of amyloidosis combined affect almost 200,000 Americans.5

hATTR amyloidosis affects an estimated 50,000 people worldwide.6

An estimated 4,000 Americans develop AL amyloidosis each year.7

Research Delivers Solutions

Options for treatment of many types of amyloidosis were extremely limited until very recently. Fortunately, advances in research have produced therapies revolutionizing amyloidosis treatment.11, 12

After decades lacking any treatment for hATTR, the first therapeutic treatment for polyneuropathy symptoms caused by hATTR, patisiran, was approved by the FDA in 2018. The infusion treatment is based on RNA interference, a strategy that researchers discovered just 20 years ago. Researchers found that it can block the production of the TTR protein in the liver, which prevents the buildup of amyloid deposits in a patient's nervous system and reduces their symptoms.13

Both AL and ATTR amyloidosis can lead to cardiac amyloidosis, where amyloid deposits form in the heart, potentially leading to heart failure. Fortunately, research has led to effective treatments. In 2019, the first drug treating cardiac amyloidosis, tafamidis meglumine, was approved in the U.S.14 Another option, heart transplantation, can be as effective for patients with cardiac amyloidosis as it is for patients with other causes of heart failure.15

Majority Say Current Spending on Research to Prevent, Cure, and Treat Disease Is Not Enough

The U.S. spends about 5 cents of each health dollar on research to prevent, cure and treat disease and disability. Do you think that this is too much, the right amount or not enough?

Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in January 2020
Amyloidosis

Then. Now. Imagine.

THEN
Amyloidosis was recognized as a disease 150 years ago, but widely available treatments only began to emerge 25 years ago. 

NOW
Treatments for patients with amyloidosis continue to be developed, and several first-of-their-kind drugs for hATTR amyloidosis have been released within the past two years. Survival among patients with AL amyloidosis has improved over the past three decades, and research shows that trend is continuing.

IMAGINE
A cure.

Difficulties in Diagnosis

Since the types of amyloidosis are so varied, patients are frequently misdiagnosed or experience delays in diagnosis, which can lead to worse health outcomes. Studies have revealed that it takes patients two years on average to get diagnosed with amyloidosis, and almost a third of patients reported seeing at least five physicians before receiving a diagnosis. Of amyloidosis patients who present with advanced, irreversible cardiac damage and die within 12 months of diagnosis, about 25% had a late diagnosis. These delays are largely attributed to the wide variety of symptoms in amyloidosis patients that can be mistaken for other diseases. For example, motor impairment caused by hATTR amyloidosis may be misdiagnosed as amyotrophic lateral sclerosis (ALS), a different disease that impairs motor function.

Genetic screening plays a key role in early identification of patients with hereditary forms of amyloidosis, even those that are not yet exhibiting symptoms. Early diagnosis allows patients to begin treatment quickly, saving lives.

Amyloidosis Mortality Rates in the U.S.
(Average over the years 1979 - 2015)

Source: JAMA Cardiology, “Geographic Disparities in Reported US Amyloidosis Mortality From 1979 to 2015”

14. “Pfizer’s Tafamidis Approved in First for Rare Heart Disease” BioSpace. 2019.