

Why are we here

Dr. Gertz, Mayo Clinic

Your potential to be eligible for a clinical trial. Effective treatments depend on trials.

Survey opportunities – so we can better understand the natural history of this disease

There are many Amyloid subtypes.

ATTR is about $\frac{1}{4}$ of the amyloid that Mayo Clinic sees. Primary amyloid (AL) is about 60%

Age at diagnosis – Mode in the US is in age 61-65, with about half from 56-70, and the rest about evenly divided before and after then (slightly more before)

At Mayo, $\frac{2}{3}$ rd of pts. are men, although the gene is probably present 50/50 in the affected population

Age at Sx onset – mode is 60 yr. old, with about $\frac{1}{2}$ 55-60. The delay from Sx to Dx is understandable – this is a rare disease, and most MDs may only see one patient for diagnosis in their career, so they often do not recognize it.

Common symptoms – Sx vary with ATTR variant. Common Sx are:

- peripheral neuropathy,
- autonomic neuropathy,
- cardiomyopathy,
- weight loss