What is AL amyloidosis?

AL amyloidosis is a rare, progressive and potentially fatal disease caused by amyloid deposits composed of misfolded light chains (proteins made by plasma cells) that build up in multiple organs and tissues, leading to damage of structure and function.

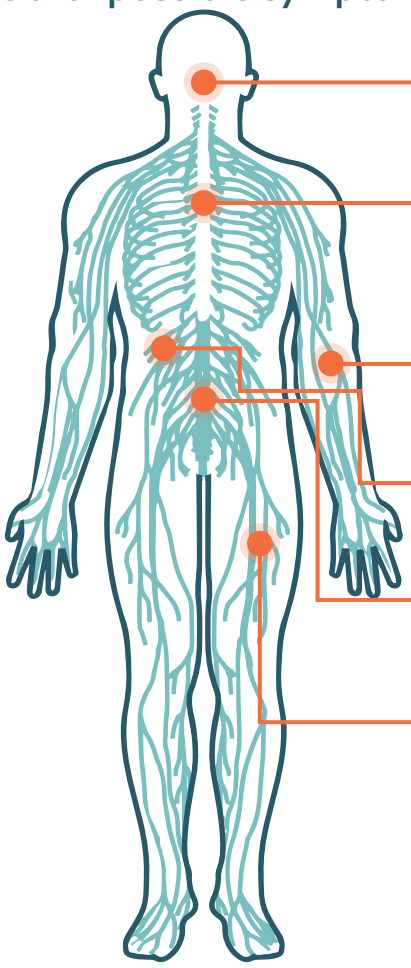
The amyloids can deposit in one or more vital organs, such as the heart and kidneys. The foreign deposits prevent the organs from functioning normally. The damage can lead to organ failure.

The cause of AL amyloidosis is still unknown, though it has been determined that the disease is not inherited or contagious.

What are the symptoms of AL amyloidosis?

People living with AL amyloidosis can experience a wide range of symptoms, depending on where in the body the amyloid builds up. The most common symptoms include weight loss, fatigue (or tiredness), shortness of breath, or edema (swelling in the arms and legs).[1]

Other possible symptoms of AL amyloidosis [1]



Head and neck symptoms
Lightheadedness or dizziness when standing up quickly; purple color on the eyelids and/or around the eyes; enlarged tongue/scalloped tongue; problems with breathing, talking, swallowing or chewing; jaw pain

Heart and lung symptoms
Shortness of breath; palpitations; chest pain; fatigue; swelling in the legs; arrhythmia; syncope (fainting)

Hand and arm symptoms
Carpal tunnel syndrome; numbness, burning, pain and/or tingling; weak fingernails or other nailbed changes

Kidney symptoms
Foamy urine; less frequent urination; getting up in the night to urinate; swelling in the lower legs, stomach, arms; kidney failure requiring dialysis

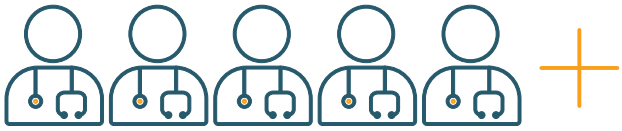
Stomach or intestinal symptoms
Poor appetite; feeling full after eating small amounts of food; nausea and vomiting; unintentional and significant weight loss; bloating; diarrhea and/or constipation; gastrointestinal bleeding; heartburn

Leg symptoms
Swelling of the feet or legs; muscle weakness; weak/brittle toenails; peripheral neuropathy

Other signs or symptoms
Obstructive sleep apnea; skin changes, such as thickening or easy bruising; hoarseness; enlarged shoulder pad (on the back); purple color in skinfolds; bruising or bleeding easily; blood clotting abnormalities

How is AL amyloidosis diagnosed?

AL amyloidosis can manifest in a variety of ways and many of the symptoms are the same as those of more common diseases. This makes the disease difficult to diagnose early. In fact, research shows that a diagnosis is often not established until **one year or more after the onset of initial symptoms.**[2] Most AL amyloidosis patients are diagnosed after the age of 50, though some adult patients have been diagnosed as early as their 20s.



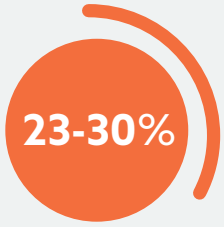
Further, almost one third of patients received a diagnosis after visiting **5 or more physicians.**[2]

Blood and urine tests can help to determine if abnormal proteins are present. A biopsy of the affected organ is the only way to confirm an AL amyloidosis diagnosis.

How is AL amyloidosis classified?

There are several staging classification systems used to stage the prognosis of AL amyloidosis. Among these include the Mayo 2012 Staging system, which uses specific laboratory tests to determine the stage. In this particular staging system, there are four stages of AL amyloidosis, with Stage I being the earliest stage and Stage IV being the stage with the highest risk for early mortality.

It is estimated that approximately 23-30% of all AL amyloidosis patients are **living with Mayo Stage IV AL amyloidosis.**[3,4]



AFFIRM-AL STUDY

How can AL amyloidosis be treated?

There is no cure for AL amyloidosis. Available treatments may help to stop production of light chains and thus possibly slowing progression of disease. Chemotherapy is typically used to treat patients with AL amyloidosis.

Additional treatment options are currently under investigation. One such option is birtamimab that is being investigated in the confirmatory phase 3 AFFIRM-AL trial in Mayo Stage IV. The clinical trial is open for enrollment to eligible patients.

Birtamimab is under investigation and is not approved by any regulatory agency.

For more information about the clinical trial visit: https://affirm-al.com or https://clinicaltrials.gov/study/NCT04973137

References

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[2] Lousada I, et al. Light Chain Amyloidosis: Patient Experience Survey from the Amyloidosis Research Consortium. Adv Ther. 2015 Oct;32(10):920-8. doi: 10.1007/s12325-015-0250-0.

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