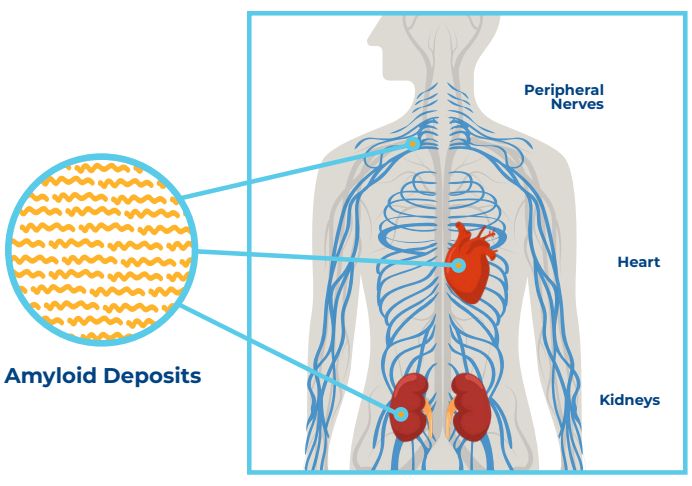
Amyloid light chain Amyloidosis (AL)

ALEXION
AstraZeneca Rare Disease

WHAT IS AMYLOIDOSIS?

Amyloidosis is a **group of complex rare diseases** caused by abnormal proteins that **misfold and clump together to form amyloid deposits** in tissues or organs, including the heart, kidney and peripheral nerves.[1-3]

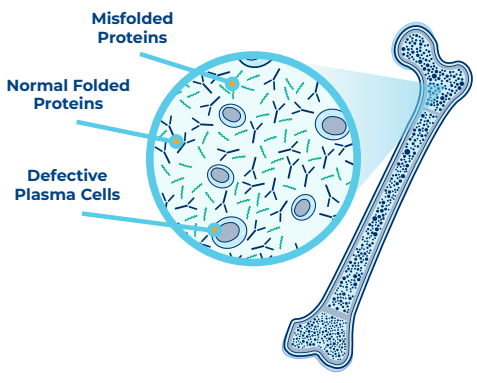
This build up can result in **significant organ damage and organ failure** that can severely impact quality of life and can ultimately be fatal.[1-3]



WHAT IS AL?

Amyloid light chain amyloidosis, also referred to as AL, is a systemic and progressive type of amyloidosis. In AL, a type of protein called **light chains are produced abnormally by defective plasma cells in the bone marrow**. These proteins misfold and form amyloid deposits.[4,5]

Accumulation of amyloid, particularly in the heart and kidneys, can cause **progressive damage** and may lead to **premature death**, most commonly due to **cardiac failure or irregular heartrate** (arrhythmia).[4,5]



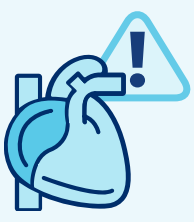
Diagnosed prevalence in adults is

~17K[6] ~14K[6] ~13K[6] ~2K[6]

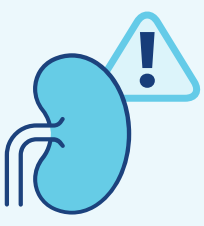
AL often occurs **from ages 50-80** but can be diagnosed in people **as early as their late 20s**.[7]

The median age of diagnosis is **60-67 years**.[7]

About 60% of people diagnosed with AL **are males**.[8]



More than 75% of people with AL **develop cardiomyopathy,** where the amyloid buildup **causes the heart to have a harder time pumping blood** to the rest of the body.[9]



More than 60% of people with AL **develop nephropathy,** where the amyloid buildup **impairs kidney function.**[9]

People with AL may experience a range of signs and/or symptoms, including:[9-11]

Swollen arms and legs

Extreme shortness of breath

Abnormal heartbeat

Nausea

Diarrhoea

Tingling in the extremities

Carpal tunnel syndrome

Enlarged tongue

Rash around eyes

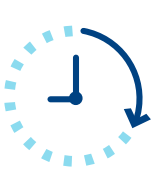
and many other **vague symptoms that mimic other diseases** that often complicate diagnosis.

HOW IS AL DIAGNOSED?

Diagnosis of AL can be relatively straightforward but is **often delayed due to non-specific signs and symptoms** that can vary by person. It can take **an average of 3 years** to receive a correct diagnosis.[12]

Once suspected, blood and urine tests are conducted first, followed by a tissue biopsy to confirm the type of amyloidosis.[13,14]

Imaging of the impacted organs may determine the severity of the condition.[13]



For many people, AL is **not accurately diagnosed until the later stages of the disease,** when **treatment options are limited and prognosis is poor.**

Rapid, accurate diagnosis leading to initiation of treatment is essential to mitigate the impact of this disease on **survival and quality of life.**[5,14]

WHAT ARE CURRENT TREATMENT NEEDS?

There are no approved treatments that address the significant organ damage caused by the disease.[5,14]

Most existing amyloidosis therapies focus on preventing and/or suppressing the formation of amyloid deposits. As a result, **the disease and organ damage may continue to progress** due to the existing amyloid deposits and ultimately **lead to organ failure and death.**[5,14]

Given the progressive nature of AL and its significant impact on quality of life, **there remains a need for increased awareness of the disease and continued innovation to improve outcomes for people living with AL.**[4,5,15]

Content created by Alexion, AstraZeneca Rare Disease

References

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