AL Amyloidosis

WHAT IS AL AMYLOIDOSIS?

Amyloidosis is a group of rare diseases causing abnormal proteins that misfold and clump together to form toxic amyloids, and deposit in organs or tissues. One type is amyloid light chain, or AL, amyloidosis where proteins that function as antibodies, also produced abnormally by defective plasma cells in the bone marrow.

Amyloid can build up in many organs, particularly in the heart and kidneys, which can result in organ damage and organ failure that can ultimately be fatal.

SYMPTOMS MAY INCLUDE:

- Diarrhea
- Numbness or tingling in extremities
- Enlarged heart
- Slow around eyes
- and may mimic vague symptoms that mimic other diseases that often complicate diagnosis.

HOW IS AL AMYLOIDOSIS DIAGNOSED?

Diagnosis of AL amyloidosis can be relatively straightforward, but it is often delayed and can take 6–18 months after symptoms begin. Imaging of the impacted organs can help determine the severity of the condition and assist to confirm amyloidosis.

CAEL-101 is a novel investigational, first-in-class therapy designed to recognize, bind to and remove existing amyloid deposits from organs and thereby improve end-organ function of damaged organs, enhance quality of life and ultimately lead to organ failure and death.

WHAT TREATMENT APPROACH IS BEING STUDIED BY ALEXION?

There are no approved treatments that address the devastating organ damage caused by AL amyloidosis. Current treatments include therapy, particularly in the heart and kidneys, which can result in organ damage and organ failure that can ultimately be fatal.

WHAT ARE CURRENT TREATMENT NEEDS?

Stage of disease and prognosis mainly depend on the extent of cardiac involvement and are based on blood tests:

Stage I
- Median Overall Survival: 10+ years

Stage II
- 4.5 years

Stage IIIa
- 2 years

Stage IIIb
- 6 months

Cardiac Amyloid Reaching for Extended Survival (CARES) Clinical Trial Program in AL Amyloidosis

Two parallel, double-blind, randomized Phase 3 studies are being conducted to evaluate the efficacy and safety of CAEL-101 combined with current treatments in patients with AL amyloidosis.

TARGET PATIENT POPULATION

Design of these trials is anticipated to include patients who are newly diagnosed and have not yet started treatment.

CARES-101 is a first-in-class investigational, first-in-class therapy designed to recognize, bind to and remove existing amyloid deposits from organs and thereby improve end-organ function of damaged organs, enhance quality of life and ultimately lead to organ failure.

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CARES-101/102 study locations include patients across: North America, the United Kingdom, Europe, Japan, Korea, and Australia.

This document does not contain all possible information. Please see full Prescribing Information for complete information. CAEL-101 is not approved for the treatment of AL amyloidosis. For more information, see full Prescribing Information. For a list of current trials, please visit Alexion.com/trials. How a Revolution is Made

References: