

Amyloidosis Facts

Amyloidosis is a rare disorder in which there is a buildup of an abnormal protein called amyloid. Amyloid is usually produced in the bone marrow and can be deposited in any tissue or organ. These deposits can also occur in multiple organs, such as the heart, liver and kidneys, causing the organ not to function properly. The progression of amyloid deposit buildup can be very quick.

There are many different types of amyloidosis, most of which are not associated with a blood cancer. The main type of amyloidosis is light-chain amyloidosis, also called AL amyloidosis. AL amyloidosis can occur by itself or with myeloma.

SYMPTOMS

Some symptoms that are associated with amyloidosis include fatigue, weight loss, heart or kidney failure, skin nodules, joint enlargement, nail changes, neuropathy (numbness, tingling, burning, or pain in the hands and/or feet), hoarse voice and dark discoloration around the eyes.

Although present in a small number of patients, enlargement of the tongue is a feature of AL amyloidosis. This is an important symptom to recognize as it can help to diagnose a patient much faster.

DIAGNOSIS

Amyloidosis is often hard to diagnose because the signs and symptoms can be the same as those of more-common diseases. It is important to diagnosis amyloidosis as early as possible so that further organ damage is prevented. Some tests to diagnose amyloidosis include

- Myeloma serum and urine evaluation for monoclonal protein
- Bone marrow aspiration and biopsy
- Skeletal survey (a series of X-rays performed to evaluate the major bones of the body).

A test called “Congo-red staining” is an essential test to confirm the suspicion of amyloidosis. This is usually done by an abdominal fat pad biopsy. This is an easy test to perform. After local injection of anesthetic, a very thin needle is inserted into the abdominal fat pad. The tissue is then stained to confirm the diagnosis.

Precise diagnosis is important because treatment varies depending on your specific type of amyloidosis.

TREATMENT

Amyloidosis is a very uncommon disease so many doctors may not be familiar with diagnosing and/or treating it. It is important to consult a doctor with experience in treating this disease.

There is currently no cure for amyloidosis. By treating the diagnosis, symptoms can be managed.

The treatments for AL amyloidosis are similar to treatments for myeloma, and can improve survival. For AL amyloidosis, treatment options include:

- **Chemotherapy**, to stop the growth of abnormal cells that produce amyloid and may improve quality of life and prolong survival.
- **Stem cell transplant**, in which your own stem cells are collected from your blood and stored for a short time while you have high-dose chemotherapy. The stem cells are then returned to your body via a vein.
- **Solid organ transplant**, the moving of an organ from one body to another for the purpose of replacing the recipient's damaged or absent organ.
- **Clinical trials**, a research study conducted by doctors to study a new drug or a new use for an approved drug or to compare a new treatment to a standard treatment to find out which one works better.

Along with any treatment option, managing the signs and symptoms of amyloidosis is important. This management may require coordinated care by specialists in multiple disciplines. Your doctors may recommend the following treatment options to help you:

- Pain medication
- Fluid retention medication (diuretic) and a low-salt diet
- Blood-thinning medication
- Medication to control your heart rate

Treatments Under Investigation

More research for types of amyloidosis can lead to better treatment options for patients. These trials, conducted under rigorous guidelines, help researchers to determine the benefits and risks of new treatments. Studies are also conducted to evaluate new indications for therapies that are already approved for other cancers or types of diseases. NCCN encourages patients to participate in clinical trials whenever appropriate.

Some treatments under investigation include

- Ixazomib (also known as MLN9708), an oral proteasome inhibitor, which is a drug in a similar class of drugs to bortezomib (Velcade®) and carfilzomib (Kyprolis™). There is a phase three trial currently ongoing to test ixazomib and dexamethasone.
- Bendamustine (Treanda®) and dexamethasone combined to treat relapsed amyloidosis

- Pomalidomide (Pomalyst®) in combination with a number of other drugs to treat relapsed and refractory amyloidosis.
- Autologous stem cell transplantation with maintenance therapy.

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We're Here to Help

LLS is the world's largest voluntary health organization dedicated to funding blood cancer research, education and patient services. LLS has chapters throughout the United States and in Canada. To find the chapter nearest to you, visit our website at www.LLS.org or contact

The Leukemia & Lymphoma Society

1311 Mamaroneck Avenue

Suite 310

White Plains, NY 10605

Contact an Information Specialist at (800) 955-4572

Email: infocenter@LLS.org

LLS offers free information and services for patients and families touched by blood cancers. The following lists various resources available to you. Use this information to learn more, to ask questions, and to make the most of your healthcare team.

Consult with an Information Specialist. Information Specialists are master's level oncology social workers, nurses and health educators. They can answer general questions about diagnosis and treatment options, offer guidance and support and assist with clinical-trials searches. Language services are available. For more information, please

- Call: (800) 955-4572 (Monday - Friday, 9 a.m. to 9 p.m. EST)
- Email: infocenter@LLS.org
- Live chat: www.LLS.org
- Visit: www.LLS.org/information specialists.

Free Materials. LLS offers free education and support publications about myeloma, clinical trials, lab and imaging tests and other blood cancer-related topics that can be either read online or downloaded. Free print versions can be ordered. For more information, please visit www.LLS.org/publications.

Clinical Trials (Research Studies). New treatments for patients with amyloidosis are under way. Patients can learn about clinical trials and how to access them. For more information, please

- Call: (800) 955-4572 to speak with our LLS Information Specialists who can help conduct clinical trial searches
- Visit: www.LLS.org/clinicaltrials.

Resources

The **Amyloidosis Foundation** has a list of doctors with an interest in researching and treating amyloidosis. www.amyloidosis.org/PatientPrograms/physicianinformation.html

References

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