Amyloidosis Research Consortium

A GUIDE TO

AUTOLOGOUS STEM CELL TRANSPLANTS IN AL AMYLOIDOSIS

KNOWLEDGE IS POWER

ABOUT THE AMYLOIDOSIS RESEARCH CONSORTIUM

The Amyloidosis Research Consortium (ARC) is a nonprofit organization dedicated to driving advances in the awareness, science, and treatment of amyloid diseases. Its mission is to improve and extend the lives of those with amyloidosis. ARC is committed to collaborative efforts that accelerate the pace of discovery, expand patient access to the most effective care, and improve short- and long-term outcomes. Working with partners in industry, government, and academia, ARC seeks to spark innovation and to bring promising treatments from labs to clinics. Its outreach and education inform and empower patients, families, caregivers, physicians, and researchers.

To learn more about ARC, visit **www.arci.org** or call **(617) 467-5170**.

Amyloidosis Research Consortium

CONTENTS

ARC gratefully acknowledges Nancy Wong, NP at the Bone Marrow Transplant and Cellular Therapy department of the UCSF Medical Center for her contributions to the development of this booklet.

INTRODUCTION

ABOUT AMYLOIDOSIS

Amyloidosis is the term for a group of rare diseases in which abnormal proteins deposit as amyloid in tissues and organs. Amyloid is produced when abnormal proteins in the body "misfold" and collect together in various tissues and organs. As the amyloid builds up, this starts to cause organ damage and impair quality of life.

Different proteins can cause amyloidosis. The unique protein involved determines what symptoms you may experience, and which treatments are right for you. This guide will discuss autologous stem cell transplantation as a treatment option for patients with Light chain amyloidosis (**AL amyloidosis**).

AL amyloidosis is caused by abnormal plasma cells located in the bone marrow. Normal plasma cells function to form **immunoglobulins**, which are antibodies that help the body fight off infections. In AL amyloidosis diseased plasma cells create an abundance of parts of immunoglobulins called light chains, which misfold and cluster together into **amyloid fibrils** (insoluble starch-like deposits).

Currently available therapies aim to suppress or eliminate abnormal plasma cells in the bone marrow and the free light chains they produce. When the rate of free light chain production is slowed or stopped, amyloid buildup declines or halts, and organ function is hoped to stabilize or improve.

Until recently, stem cell transplants were the preferred treatment for eligible patients. As treatment options have increased in recent years this is not necessarily the case. It is important to discuss with your physician whether a stem cell transplant is an option along with other available treatments, so that with your care team you can make an informed decision about the best treatment choice.

AL Amyloidosis can cause a range of symptoms. To learn more about the effects of AL amyloidosis on the body, please view the Disease and Treatment Overview: Light Chain Amyloidosis booklets in our online library: arci.org/booklets

BONE MARROW AND STEM CELLS

Bone marrow is the soft-spongy tissue located in the center of bones, where stem cells are produced. Stem cells are very important, as they are the body's master cells. Stem cells eventually develop into many different types of cells that our body needs to function.

Each cell generated from a stem cell has its own vital function:

- Red blood cells are responsible for carrying oxygen throughout the body.
- White blood cells are a part of the immune system. They help to fight off infection and harmful bacteria. Plasma Cells are a type of white blood cell.
- Platelets help stop bleeding by forming clots.

WHAT IS AN AUTOLOGOUS STEM CELL TRANSPLANT?

There are two main types of stem cell transplants: **allogeneic transplants** and **autologous stem cell transplants** (ASCT). In either form of transplant, high dose chemotherapy is given as part of the transplant preparation, which destroys healthy and diseased plasma cells as well as various blood cells. Stem cells are then transplanted into the body, allowing for the regeneration of healthy cells.

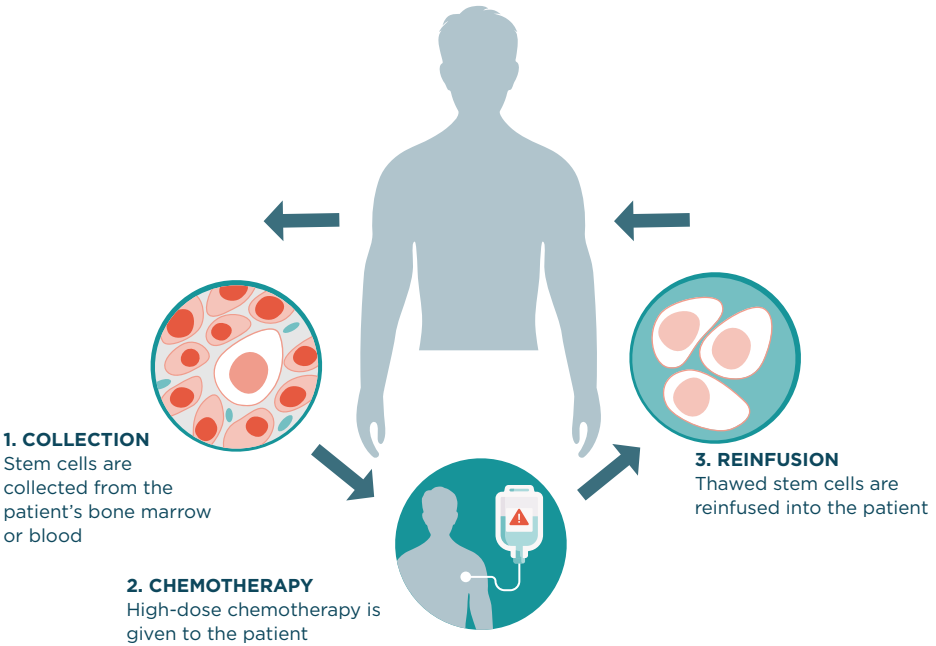
Allogeneic means "from another person." This is because allogeneic transplants involve stem cells donated by another person. Autologous means "from the same person." During an autologous stem cell transplant, a patient's healthy stem cells are collected and transplanted back into their own body after

administration of high dose chemotherapy. In AL amyloidosis treatment, ASCTs are preferred due to its effectiveness and the decreased chance of experiencing side effects associated with receiving donor cells (such as post-transplant **engraftment** failure or **graft versus host disease**).

TRANSPLANT PROCEDURE

High-dose chemotherapy (HDC) is used to eradicate all blood-forming stem cells made in the bone marrow. After HDC, the patient's previously collected healthy stem cells are reinfused back into the patient, in order to "rescue" the bone marrow and grow new cells. In receiving ASCT, there are three overall steps: 1) Collection of stem cells, 2) High-dose chemotherapy, and 3) Stem Cell Transplant.

AUTOLOGOUS STEM CELL TRANSPLANTATION (ASCT)



COLLECTION

Stem cells are collected (also known as harvested) from the blood prior to receiving HDC. Cells are collected via a **catheter** placed into the arm or neck of the patient, which is removed after completion of the draw.

Before the blood is drawn stem cells are stimulated to multiply and move out of the bone marrow and into the bloodstream for easy collection. This process is called stem cell mobilization. Stem cells are mobilized using medications known as granulocyte-colony stimulating factor or G-CSF, such as filgrastim and/or plerixafor, which is given through subcutaneous injection. Mobilization medications are typically given for about 4-5 days prior to stem cell collection. Side effects of the mobilization medications may include bone pain, diarrhea, shortness of breath, edema, or low blood pressure. Side effects can be treated with pain medications and an anti-diarrheal.

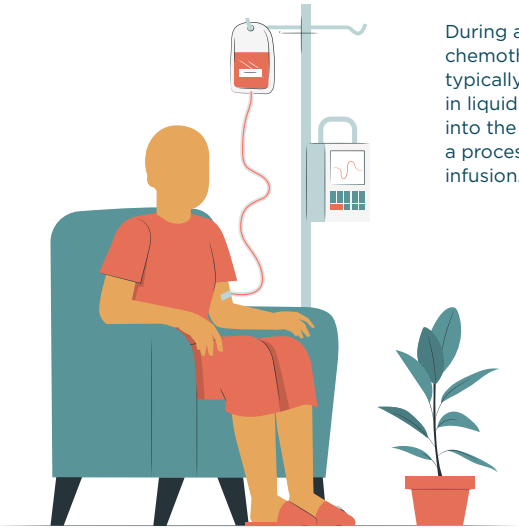
As blood is drawn, it filters into a machine that separates and collects stem cells, while returning the remaining blood components back into the patient's bloodstream. This process is known as apheresis. The stem cell collection process should be painless and takes about 1 to 3 days to complete. Potential side effects of the draw include shortness of breath, chills, tremors, dizziness, muscle cramps and edema.

Once the stem cells are collected, they are processed to purify and concentrate the stem cells. Next, they are frozen and stored until they are ready to be returned to the patient during the transplant phase.

HIGH-DOSE CHEMOTHERAPY (HDC)

During an ASCT, the purpose of HDC is to destroy the plasma cells responsible for producing abnormal light chains. Mephalan, also known as Alkeran, is an HDC that is very effective in destroying plasma cells, as well as bone marrow tissue. Because of its effectiveness, Mephalan is the more commonly used preparatory regimen prior to ASCT in AL Amyloidosis.

- Although the purpose of the HDC is to rid the bone marrow of bad cells, healthy stem cells in the bone marrow are also destroyed during the process. This causes the immune system to become weaker and harder to fight off infections. It is very important to report any infection symptoms, even mild ones, to your transplant team so that the proper tests are done to identify the sources of the infection and the appropriate treatment is given.
- Your transplant team will provide preventative antibiotics to decrease the risk of infections while the white blood cells and other immune cells continue to recover.



During an ASCT, chemotherapy is typically administered in liquid form directly into the vein, through a process known as IV infusion.

TRANSPLANTATION OF STEM CELLS

After HDC, the patient's own stem cells are reinfused to "rescue" the bone marrow and make it grow new cells. The cells are returned through an intravenous line either through a vein in the arm or a central line. Pain is rarely felt, and the thawing and infusion of stem cells can take between 2 and 4 hours. After entering the bone marrow from the blood, stem cells begin to make new cells. This is known as **engraftment**. Early engraftment can take 10-14 days.

The care team will monitor engraftment progression by drawing blood daily and monitoring blood count.

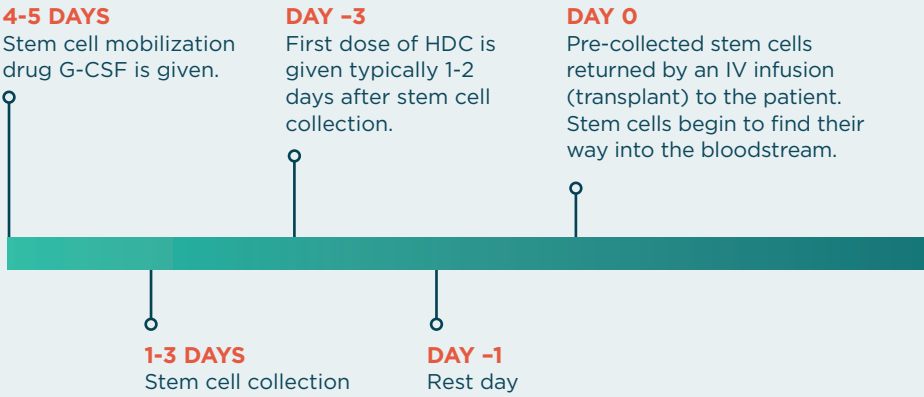
ARC is here to help you find treatment centers staffed with clinicians who specialize in the care of patients with AL amyloidosis.

TRANSPLANT CONSIDERATIONS

CANDIDATES FOR TREATMENT

Amyloidosis patients diagnosed with AL amyloidosis have ASCT as a treatment option if they are eligible. About 25% of newly diagnosed AL patients are eligible for ASCT. To reduce the risk of life-threatening complications, the following factors may be considered to determine health outcomes:

STEM CELL TRANSPLANT TIMELINE

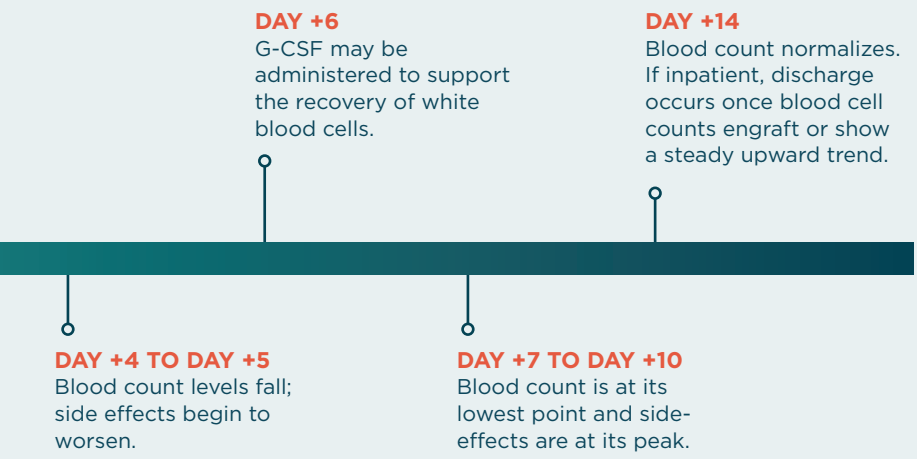


- Age
- Level of organ dysfunction
- Pre-existing medical issues and overall health status.

A patient's health status will be reviewed by the care team to determine eligibility. It is important to discuss different treatment options with the physician, including eligibility for an ASCT. A stem cell transplant may not be recommended as the first line of treatment but may be considered at a future date after trying other therapies. It is also important to discuss both short- and long-term side effects that may occur.

BENEFITS OF AN ASCT

- The procedure can provide a deeper and more durable response, improvement in symptoms, and improved quality of life.
- It can be a very effective treatment for patients who did not otherwise respond adequately to prior therapy.
- No risk of the body rejecting the stem cells, since it is from the patient's body.



LIMITATIONS OF AN ASCT

- There is a risk of side effects and complications, including infections, bleeding, and further organ damage.
- ASCT may not be suitable for all patients, especially those with significant organ involvement, elder age, or poor performance status.

Patients with wild-type or hereditary ATTR amyloidosis do not qualify for ASCT therapy. This is because ATTR amyloidosis is caused by abnormal transthyretin (TTR) protein, which is generated in the Liver.

PREPARING FOR THE PROCEDURE

PRE-TRANSPLANT EVALUATION AND TESTING

Become familiar with the care team

Each member of the care team plays an important role in providing care throughout the ASCT process. Meeting with the care team allows patients to familiarize themselves and establish a relationship with those who will be supporting them on their transplant journey.

Titles may vary slightly by center, but the care team can include members fulfilling the functions listed below:

- **Attending Physician:** Usually a hematologist that specializes in stem cell transplantation and directs care throughout treatment.
- **Nurse Practitioner or Physician's Assistant:** Works with the attending physician to provide primary care through the transplant process.
- **Nursing Staff:** Provides frequent monitoring of patients, while managing care.
- **Transplant Care Coordinator:** Coordinates the education, evaluation, and preparation of patients for the ASCT. Organizes

appointment logistics and is the main point of contact for patients and family members to address any inquiries.
- **Social Worker:** Offers social-emotional support while providing resources to fulfill patient needs.

Specialists such as a cardiologist, nephrologist, and/or gastroenterologist may support the attending physician in monitoring the health of any previously affected organs. The care team could also include a dietician, physical or occupational therapist, depending on the needs of a patient pre-and post-transplant.

Solidify caregiver and community support

Patients are typically required to identify a primary caregiver who can provide consistent care post-transplant. The caregiver role requires availability to the patient on a full-time basis; especially in the first few weeks following the transplant. This responsibility applies to caregivers supporting loved ones receiving an in-patient or out-patient transplant procedure. The level of care needed should be taken into consideration when selecting a caregiver. It is best to limit the caregiver role to 1-2 people, as patients will be in a state of isolation following the transplant. The caregiver role is very important and responsibilities range from meal prep, grocery shopping, medication management, to driver to and from appointments, and furthermore.

Get a medical workup

Patients with AL Amyloidosis undergo a complete medical checkup before they can proceed with an ASCT, to determine how amyloidosis is affecting their organs. While this process may be specific to the patient, standard tests may include blood and urine labs, **electrocardiogram (EKG), transthoracic echocardiogram (TTE)** to measure of heart function, **pulmonary function test (PFT)** to measure lung health. Disease burden may be assessed by observing the percentage of amyloid producing plasma cells in the bone marrow, in comparison to healthy plasma cells. This can be assessed during a **bone marrow biopsy**. The aggregate of these test results help determine if the patient's organ function can tolerate the entirety of the transplant process. The pre-transplant work up will also serve as a baseline for future assessment of treatment response.

INPATIENT VS OUTPATIENT CARE

Depending on the transplant center or institution, patients may be offered the stem cell transplant in an inpatient or outpatient setting. Either option will have its own advantages and disadvantages. There are a few factors that may go into consideration when deciding whether a patients' ASCT will be done on inpatient, outpatient, or hybrid (a combination of outpatient and inpatient).

An inpatient ASCT admits a patient into the hospital where they will remain until their blood counts recover to an acceptable level. The decision for an inpatient or outpatient ASCT, can depend on location of the transplant center, the proximity of the transplant center to the patient's home, susceptibility to infection, caregiving situation, and overall patient health status. An outpatient ASCT allows a patient to reside nearby the transplant center and usually require daily close follow up throughout the transplant course. Hospitals may offer nearby housing to allow for safe, and efficient transportation between appointments. Hybrid ASCT will offer part of the transplant process outpatient and the patient will be admitted at a specified time. Reasons to consider hospitalization include, poorly managed nausea, diarrhea, signs of dehydration, fever, and infection symptoms, just to name a few.

RECOMMENDATIONS FOR PATIENTS RECEIVING ASCT AS INPATIENT

Undergoing an ASCT could mean spending about two weeks in a hospital. For this reason, patients are encouraged to bring items that remind them of home, to provide comfort and familiarity. Items such as personal pillows, blankets, robes, sleepwear, and images of loved ones could all contribute to an environment of comfort for patients. Button-down shirts or loose-fitting clothing are also encouraged, to allow for ease of access to any medication lines that may need to be inserted. Patients may also consider entertainment items for use during down time, which may include books, puzzles, laptops, tablets etc.

Personal care items such as moisturizers, mild shampoo or shower gel, toothbrushes, eye masks or ear plugs can also be brought along. After receiving HDC a patient may experience significant

hair loss. To prepare for this, patients may wish to bring along various hats or wigs to the hospital.

Visitor policies may vary by center, so it is important to be aware of the visitor policy ahead of time. In most cases you will be allowed a limited number of visitors, in order to limit the risk of exposure to potential infections. It is also important to be aware of additional hospital rules surrounding what patients can have in their rooms. Generally, live plants, flowers, and fragrances are prohibited as they increase the risk of infection or irritation to the body.

SIDE EFFECTS OF ASCT

PHYSICAL SIDE EFFECTS

HDC can cause various symptoms, including a low immune response. While the recovery stage is unique to each patient, symptoms can be expected following the transplant:

PHASE	SYMPTOMS
Stem Cell Mobilization and Harvesting	• Bone pain • Low calcium • Fatigue • Muscle cramps • Tingling of fingertips • Twitching or tingling around the mouth
Transplant day to engraftment: Neutropenia 10-30 days post-transplant	• Low blood counts (red blood cells, platelets, white blood cells) • Risk of infection at highest peak • Gastrointestinal symptoms: nausea, vomiting, diarrhea, mouth sores, decreased appetite • Skin rashes • Hair loss • Fever and/or chills • Loss of taste and/or smell
Early Recovery: Discharge-1 year post transplant	• The immune system remains weakened • Risk of infection lowers but continues

Symptoms experienced in the stem cell mobilization and harvesting stage are considered short-term, as they should improve after treatment. Adverse effects associated with the HDC and stem cell transplant can take longer to recover from, sometimes up to a year. To help alleviate the symptoms, a care team may provide the below remedies:

- **Low calcium levels (hypokalemia):** A drop in calcium levels may cause symptoms such as muscle cramps, fatigue, and tingling of the fingertips and/or around the mouth. To offset symptoms, medications containing calcium may be administered via a pill or IV format. Calcium carbonate drugs, such as Tums (antacid) are also commonly used as a calcium source.
- **Nausea and vomiting:** an **antiemetic** medication may be given to reduce feelings of nausea and vomiting; such drugs can be given orally, sublingually or intravenously. For patient with cardiac involvement of amyloidosis, close monitoring of fluid status (daily weights) and electrolytes is very important. Altered electrolyte levels and fluid imbalance can trigger heart arrhythmias.
- **Mouth and gastrointestinal sores:** to help reduce the risk of mouth sores patients may be given ice cubes to consume during HDC treatment. Antibacterial and antifungal mouthwashes may also be recommended to fight off any potential infections. In the case mouth sores develop, pain killers can also be administered for pain management.
- **Hair loss:** Cold caps may help to mitigate the amount of hair loss experienced by patients. Cooling the scalp during chemotherapy decreases the amount of blood flowing into the hair follicle, giving the chemotherapy less access to the hair follicles.
- **Immune System/White blood cell count:** Patients are without protection provided by the immune system for 3-4 days and are given preventative antibiotics, to help protect against common but serious viral, bacterial, and fungal infections. G-CSF may be given to patients to help white blood cell recovery.
- **Platelet Count:** A low platelet count increases the risk of bleeding. Platelet counts may be increased via a transfusion, if necessary. For patients on anticoagulation (or blood thinners), these medications may need to be temporarily held to reduce risk of serious bleeding.

- **Fertility:** HDC can affect fertility. Patients are encouraged to speak with their care team to discuss family planning options including the preservation of egg or sperm cells prior to proceeding with stem cell transplantation.

Prior to discharge after an inpatient transplant, patients must demonstrate improved side effects and adequate oral intake. In addition, patients need to show signs of early engraftment, such as increase in white blood cell count and stability in platelet count.

ASCT can be associated with secondary malignancies, such as, **myelodysplastic syndromes** (MDS) and/or **acute myeloid leukemia** (AML) within 25 years after the transplant.

MENTAL AND-EMOTIONAL SIDE EFFECTS

Undergoing an ASCT is taxing not only on the body but can also affect the mental health. It can be normal for patients to experience anxiety surrounding the whole transplant process. The recovery phase can be isolating and frustrating at times. If feelings of sadness or depression persist, it is important to connect with mental health support services for help. This could include organizations such as support groups, therapists, or spiritual/community-based groups. The social worker on the care team can also help to address any potential stressors associated with financial support, disability, caregiver accessibility, housing assistance, transportation, or psychological needs.

RETURNING TO YOUR REGULAR ROUTINE

In the post-transplant setting, it is important to closely follow the care team's recommendations and to maintain close communication with the health care team.

During the recovery period, the immune system needs time to rebuild and strengthen. It could take three to six months to regain a sense of wellness and resume a typical daily routine and other activities.

During the early post-transplant phase (<30 days), the health care team may require patients to be seen weekly for visits. The care team may include the following components in the treatment plan during this period:

- Medicines to prevent common illnesses.
- Education related to diet.
- Exercise limitations .
- Safety measures when interacting with others in close quarters.
- Sexual health and intimacy practices.
- Hygiene practices.
- When is it safe to drive on your own.

During the later post-transplant recovery phase (>30 days), the health care team can review the following:

- The optimum time to receive or update immunizations to reduce the risk of contracting preventable diseases like shingles, tetanus, diphtheria, pneumonia, covid, hepatitis B, influenza and MMR.
- When can the patient return to work and certain restrictions.

To allow for recuperation, patients should consider taking leave from work for the initial 2-3 months following the transplant. Resting should be prioritized during the recovery period to avoid any unnecessary exertion on the body.

Patients are also encouraged to notify their care team if they notice any symptoms of concern, such as a fever, cold or flu like symptoms, irregular bleeding or bruising, rash, or uncontrollable nausea or diarrhea. A symptom diary may be helpful in keeping track of symptom occurrence. If exposed to any viruses such as COVID, shingles, chicken pox, measles, rubella, patients should contact their care team immediately. To help limit this risk, patients may be instructed to limit social outings and the number of visitors they come in contact with for a period of time post-transplant.

HOW IS TREATMENT RESPONSE ASSESSED?

To observe a patient's response to treatment, regular monitoring and follow-up testing will be conducted. Tests vary depending on the patient, but typically include urine and blood testing, bone marrow biopsy, and radiographic imaging.

A patient's response to ASCT is determined by observing changes in light chain levels. This is done via blood testing to measure the associated levels:

1. **Serum free light chains** - this test measures the quantity of kappa and lambda light chains and the ratio to see if one is "dominating" and making amyloid fibrils. When the ratio is normal, that is a good sign.
2. **Serum immunofixation electrophoresis** testing - this test looks specifically for the abnormal (involved) light chain or light chain clones in the blood. These can either be kappa or lambda light chains.
3. **Urine immunofixation electrophoresis** testing - this test looks for the bad light chain or clones in the urine.

Trending free light chain results can help measure treatment response. Clinicians will often measure the dFLC (difference between involved and uninvolved Free Light Chains). dFLC is calculated by subtracting the concentration of the normal (uninvolved) light chain type from the concentration of the abnormal (involved) light chain type.

LEVELS OF HEMATOLOGICAL RESPONSE		
Complete response (CR)	**Very good partial response (VGPR)**	**Partial response (PR)**
Normal FLC ratio (kappa:lambda) and negative urine and serum immunofixation testing	dFLC under 40 mg/L	50% reduction of dFLC from baseline

A repeat bone marrow biopsy can measure diseased plasma cell percentage in the bone marrow. Minimal residual disease testing is becoming more of a common practice to measure depth of response.

The goal of chemotherapy is to achieve a complete response (CR) or very good partial response (VGPR). If you have a partial response (PR), your doctors may consider a change in your follow-up care.

Additionally, organ response will be monitored. The aim of ASCT is to stop the production of abnormal light chains and allow the organs to recover. Over time organ function may improve.

RELAPSED AL AMYLOIDOSIS AND YOUR OPTIONS

The goal of an ASCT is for the amyloidosis to be put into a state of **remission**, which occurs when a patient experiences a significant decrease in or disappearance of abnormal light chains. In AL amyloidosis patients, this can correlate with obtaining a complete response (CR) or a very good partial response (VGPR).

While many AL amyloidosis patients can remain in remission for long periods of time, even years post-transplant, there remains a chance for the disease to re-occur. When the AL amyloidosis returns after a period of remission, it is referred to as a relapse. Therapeutic options are available upon disease **relapse**.

Previous treatments may be repeated if a patient has previously demonstrated a positive response to it, or a different regimen may be prescribed. If the response to the ASCT was good and lasted for a long period of time a second ASCT could also be considered, depending on the patient's health. Clinical providers may discuss clinical trial options. Each patient's situation is unique and is assessed by doctors on a case-by-case basis.

MAP | My Amyloidosis Pathfinder

New trials are always in development to help expand treatment options and improve quality of life. Join MAP to receive notifications as new clinical trials and treatment centers are posted.

Treatment Center Selector

Clinical Trial Finder

www.myamyloidosispathfinder.org

EVOLVING ROLE OF ASCT IN TREATING AL AMYLOIDOSIS

Treatments for AL amyloidosis have markedly improved over time, and there are increasing number of treatment options. The role of ASCT has evolved over time and it is important to discuss with the care team whether this is a suitable treatment option. Clinical trials assessing different approaches to treatment are underway.

CAREGIVER SUPPORT

Caregivers are responsible for providing constant support throughout the transplant process. The caregiver role is vital to a patient's recovery; therefore, it is important for caregivers to also prioritize their health and well-being. Caregivers may experience emotional, financial, and mental challenges while helping their loved ones through recovery. Many foundations provide direct assistance for caregivers who are supporting loved ones through the transplant process. Examples of caregiver assistance include telephone support lines, counseling, caregiver support groups and financial assistance. The purpose of support programs is to equip caregivers with the tools needed to walk though emotional changes, empower caregivers to speak up for themselves, while ensuring their health and wellness is not being overlooked. Caregivers may consult with the transplant social worker for support resources.

ARC is also here to support caregivers and connect them to resources. Email us at arc@arci.org.

GLOSSARY

Acute myeloid leukemia. A cancer of the bone marrow.

AL amyloidosis. A progressive form of systemic amyloidosis caused by abnormal plasma cells that produce light chain proteins; these misfold into amyloid and circulate in the blood, building up deposits in multiple organs and tissues.

Allogenic stem cell transplant. The transferring of stem cells from a donor to a patient, after high-intensity chemotherapy or radiation.

Amyloid fibrils. Insoluble starch-like deposits formed from misfolded proteins.

Antiemetic. A drug used to combat nausea and vomiting.

Apheresis. A procedure that draws blood into a machine that separates and collects stem cells and returns the rest of the blood components to the donor or patient.

Attending Physician. Director of care throughout treatment.

Autologous stem cell transplant (ASCT). Procedure in which a patient's own stem cells are collected, stored, and then given back following high-dose chemotherapy.

Bone marrow. The soft-spongy tissue located in the center of the bone, where stem cells are produced. Stem cells are very important, as they are the body's master cells.

Catheter. A thin and flexible tube designed to insert or remover substances from the body.

dFLC. The difference between involved and uninvolved Free Light Chains; calculated by subtracting the concentration of the normal (uninvolved) light chain type from the concentration of the abnormal (involved) light chain type.

Echocardiogram. An ultrasound providing imaging of the heart.
Engraftment: The process in which stem cells begin to form new

cells in the bone marrow.

Electrocardiogram (EKG). Measures the electrical activity of the heartbeat.

Free light chain (FLC). Part of an immunoglobulin (antibody) that circulates freely in the bloodstream.

Graft versus host disease. A disorder that occurs when donor cells attack the recipient.

High-Dose Chemotherapy (HDC). An intense treatment given to destroy cancer cells; it also destroys the bone marrow and can cause other severe side effects.

Immunoglobulins. Proteins in the blood produced by specialized white blood cells (plasma cells) that fight infection and disease.

IV Infusion. The process of administering fluid directly into the vein.

Melphalan. An anti-cancer chemotherapy drug.

Myelodysplastic syndromes (MDS). A disorder in which blood cells are poorly formed and malfunction.

Neutropenia. A condition in which there are a low count of white blood cells (Neutrophils) in the blood.

Neutropenic. When the immune system is in a weakened state due to neutropenia.

Nurse practitioner or Physician's Assistant. Works with the attending physician to provide primary care through the transplant process.

Nursing Staff. Provides frequent monitoring of patients, managing care.

Platelets. Stop bleeding by forming clots.

Pulmonary function test. An invasive lung test.

Red blood cells. Responsible for carrying oxygen throughout the body.

Relapse. AL amyloidosis that responded to a previous treatment but shows signs of returning.

Remission. A significant decrease in or disappearance of signs and symptoms of disease.

Serum free light chain. Immunoglobulin light chains that are circulating in a free state.

Serum immunofixation electrophoresis testing. Looks specifically for the abnormal (involved) light chain or light chain clones in the blood. These can either be kappa or lambda light chains.

Social Worker. Offers social-emotional support while providing resources to fulfill patient needs.

Stem cells. The cells from which blood cells develop; normally found in the bone marrow.

Stem cell harvesting. The process of collecting stem cells from the blood, prior to the transplant.

Stem cell mobilization. The process of stimulating stem cells out of the bone marrow and into the bloodstream, for easy collection.

Transplant Care Coordinator. Coordinates the education, evaluation, and preparation of patients for the ASCT. Organizes appointment logistics and is the main point of contact for patients and family members to address any inquiries.

Urine immunofixation electrophoresis testing. This test looks for the bad light chain or clones in the urine.

White blood cells. A major cell type produced in bone marrow that attacks infection and cancer cells; they are part of the immune system.

FREQUENTLY ASKED QUESTIONS

What can be done to treat side effects?

Common side effects of Melphalan (used for transplant prep) are mucositis, nausea, vomiting, diarrhea, low blood counts, infections. Chewing on ice chips during the Melphalan infusion can help reduce risk of mucositis/mouth sores. Antiemetics are given to mitigate nausea and vomiting. It can be given as needed or around the clock depending on severity. A growth factor, such as Zarxio is given to support neutrophil recovery and shorten the number of days of having severely low neutrophil counts. Empiric broad spectrum antimicrobials are given to reduce infection risk.

Is a special diet needed?

A heart healthy diet is recommended, which includes lean proteins, fresh fruits and vegetables, low sodium, healthy grains, while limiting processed foods. Avoid dramatic dietary changes right before transplant. It can be a shock to the body.

Are there special things that I should or should not do during or after stem cell transplant?

To prevent infection risk, avoid people with obvious infection symptoms. Try to stay mentally and physically active. When in the hospital, take walks around the transplant unit. Try to maintain good sleep habits, by doing activities during the day (walking, light exercise, reading, puzzles) and sleeping at night. Avoid activities that have a risk of bleeding, especially when platelets are low, such as shaving or exercising with machinery. Hydrate well always!

Will stem cell transplant affect usual activities? If so, for how long?

After discharge from the hospital, the focus is on resting and recovery. It can take about 3 months to recover to baseline (regards to energy level, eating habits, and generalized well-

being). Patients gradually feel better with each week with minor ups and downs. Walks and light exercise as tolerated are recommended. It can take several weeks before a patient feels well enough to help with chores.

When are follow-up visits scheduled? Who is responsible for follow-up after stem cell transplant?

Patients should be seen in clinic within a week from discharge. Patients are typically followed on a weekly basis for 3-4 weeks post discharge. Patients will have weekly labs, transfusions as needed, and weekly provider visits with APP or MD. Caregivers are expected to drive patients to and from the clinic. especially during the 1st month post-transplant.

Can a support person (such as a partner, parent, or friend) stay during stem cell transplant? What about visitors while I am in the hospital?

Visitors are allowed, but it depends on the institution.

ARC PATIENT & CAREGIVER RESOURCES

The Amyloidosis Research Consortium (ARC) is a global organization with a mission to advance scientific discovery, improve access to state-of-the-art care, and empower patients with innovative educational tools and support. Companion booklets in our information series have been developed to inform and guide you: **arci.org/booklets**

Questions?
Email: **arc@arci.org**

SUPPORT ARC

Help support ARC's effort to extend and improve the lives of patients with amyloidosis. Contact Laura Duvelius, Executive Director of Development, to donate today.

Telephone: **406.595.6223**
Email: **Lduvelius@arci.org**

This booklet is supported by grants from:

» *Alnylam Pharmaceuticals*
» *AstraZeneca*
» *Intellia Therapeutics*
» *Ionis Pharmaceuticals*
» *Prothena*

CONTACT ARC

Amyloidosis Research Consortium (ARC)
320 Nevada Street, Suite 210
Newton, MA 02460

Email: **arc@arci.org**
Telephone: **(617) 467-5170**
Mon-Fri 9:00 am-5:00 pm EST

Learn more at **ARCI.ORG**

Amyloidosis
Research
Consortium