# News and Stories - Winter 2019

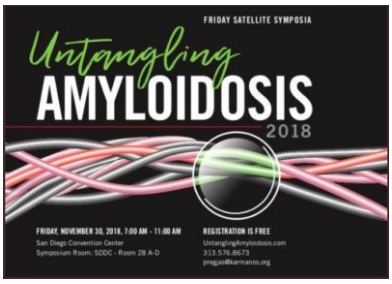
## Untangling Amyloidosis Symposium at ASH Conference

By Jeffrey Zonder, MD– Karmanos Cancer Institute

The American Society of Hematology held its annual meeting in San Diego, California the first week of December, 2018. Over 20,000 researchers from around the world convened to share the results of their research in areas spanning hematology, including amyloidosis. While there were no new "blockbuster" breakthroughs in amyloidosis presented, there is still news to share.

On Friday November 30th, there was a Satellite Symposium supported by the Amyloidosis Foundation entitled 'Untangling Amyloidosis 2018'. This well-attended program provided a state-of-the-art update on the diagnosis and treatment of both AL (immunoglobulin light chain) and ATTR (transthyretin) amyloidosis. Here is a summary of the speakers and key points from their talks:

**Dr. Giovanni Palladini (http://bit.ly/Palladini)** from Pavia, Italy, discussed initial transplant- and nontransplant-therapy of AL amyloidosis. He emphasized the different




mechanisms of organ injury seen in the disease, including direct cytotoxicity of misfolded light chains. He emphasized the importance of achieving a rapid, deep, and sustained reduction in the abnormal (misfolded) light chains by targeting the abnormal

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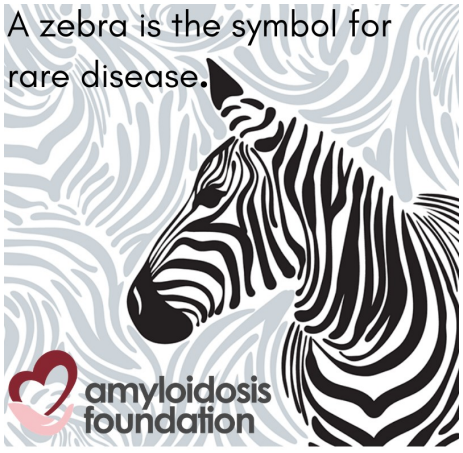
## Embrace your stripes for Rare Disease Day!




'When you hear hoofbeats, don't expect to see a Zebra.'

The above adage is especially useful in primary care as many of the conditions physicians see are common. But it is not so useful when physicians consider diagnosing and supporting patients with rare diseases like amyloidosis. They must be ready to think again and look out for the horse with stripes!

Show your support for the 7000+ rare diseases by wearing zebra print (or black and white stripes) on March 28, 2019, which is Rare Disease Day in the United States. **AF**

## 2019 Amyloidosis Foundation Research Grant Awardees

The Amyloidosis Foundation recently awarded two research grants for 2019. The Donald C. Brockman Memorial Research Grant went to Siyang Leng, MD from Columbia University Medical Center in New York City and the David Seldin, MD, PhD Memorial Research Grant recipent is Amandeep Godara, MD from Tufts Medical Center in Boston, MA.

Since 2005, the AF has funded over $1.8 million to promising clinical and scientific amyloidosis investigators from around the world. We applaud their efforts and look forward to the success this work will bring to patients.

**Siyang Leng - MD**

Quality of Care and Disparities in Myeloma Associated Amyloidosis

Amyloidosis Foundation
Donald C. Brockman Memorial Research Grant, 2019
Columbia University Medical Center, New York, NY

**Amandeep Godara - MD**

Development of Antibodies for Elimination of Serum Free Light Chains

Amyloidosis Foundation
David Seldin, MD, PhD Memorial Research Grant, 2019
Tufts Medical Center, Boston, MA

# Patient Resources

The foundation has several programs that benefit patients and their families. All of these are provided free of charge.

- Webinar recordings posted on our website
- Updated informational pamphlets
- Toll Free Number **1-877-AMYLOID**
- Listing of experienced physicians that specialize in amyloidosis. Email us anytime with questions: **info@amyloidosis.org**

Our comprehensive website has information for patients, caregivers and physicians featuring:

- Treatment Centers (US / International)
- Support Groups
- Newsletters
- Webinars
- Fundraising Toolkits

## Follow Us

amyloidosis foundation

Stay connected for all the latest information on Amyloidosis:

**Web:** **www.amyloidosis.org**
**Twitter:** **@Amyloidosisfdn**
**Facebook:** **@amyloidosisfdn**
**Instagram:** **@amyloidosisfoundation**

# President's Corner

Mary E. O'Donnell

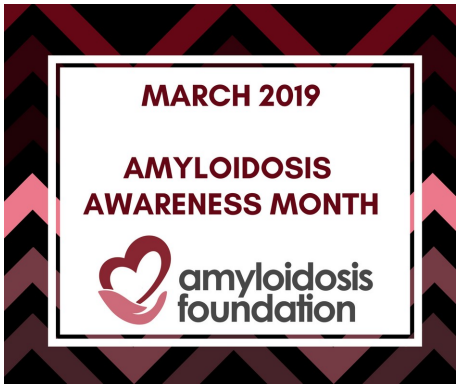
Now that the holidays are behind us, we are looking forward to the new advancements a new year can bring in the field of amyloidosis.

2018 was a stellar year for hATTR amyloidosis as there were 2 FDA approvals. In August 2018, the U.S. Food and Drug Administration approved Onpattro (patisiran) infusion for the treatment of peripheral nerve disease (polyneuropathy) caused by hATTR in adult patients.

The second treatment approved (Inotersen) inhibits production of the transthyretin (TTR) protein amyloid. The FDA announced the approval on October 5, 2018. It is administered in a subcutaneous injection once a week and will be marketed as Tegsedi.

In the near future, new therapies stand to gain FDA approval for amyloidosis. May the year 2019 be blessed with good health and more great news for amyloidosis warriors!

**March Designated as Amyloidosis Awareness Month**




Thank you to efforts of Amyloidosis Foundation Board Member Charlotte Haffner in Tennessee, Volunteers in Indiana and Louisiana, and AF employees in Michigan, we can now officially announce that Amyloidosis Awareness month is recognized by these states.

For more information, visit our website. **AF**



Our newsletter is published quarterly (Spring, Summer, Fall and Winter) by the **Amyloidosis Foundation**. We welcome letters, articles and suggestions.

Please contact us anytime at: **info@amyloidosis.org, 1-877-AMYLOID** (877-269-5643) or **7151 N. Main Street, Ste. 2, Clarkston, MI 48346**

*If you wish to receive an electronic version, please send us an email:*

**info@amyloidosis.org**

## Against All Odds

**Taken from a story by Dr. Leslie Schumaker-McKee**

My name is Dr. Leslie Schumacher-McKee and I am 39 years old. After the birth of my second son, I started experiencing increased bruising, muscle (particularly calf) pain, abdominal pain, and lethargy. In August of 2011, after 5 months of numerous appointments with multiple doctors and physical therapy (mostly attributing my symptoms to being a working mom with a 7 year old and a nursing baby), I received a phone call that forever changed my life; I was in kidney failure! It was shortly determined through kidney and bone marrow biopsies that I had AL amyloidosis. On October 1, 2013 (and against all odds), my brother Stephen donated his kidney "Lefty" to me, and I slowly began my recovery process, with minor setbacks. Today, I am leading a wonderful life. Although I will never be back to my "old normal self," I am stronger physically, emotionally, and spiritually. My husband started a charity called "Present Troubles Racing" to support amyloidosis programs as well as stem cell patients. He and I also organize an event each year, the "Hills & Hollers" Fundraiser for Amyloidosis Awareness, Patient Support and Research, which is a Half Marathon & 5K in Burwood, TN.

I cherish time with my family and friends in a whole new way. My journey has been difficult, but I strive daily to be a better person while raising awareness of, and supporting patients with, amyloidosis and organ transplants. **AF**

## GivingTuesday Update

Thank You Everyone for Your Support on #GivingTuesday!

The Amyloidosis Foundation is thankful for the generosity and love from our friends and donors on #GivingTuesday.

You helped us surpass our goal of raising $15,000 in 24 hours (in honor of our 15th anniversary), by soaring past that with over $20,000 in online donations and Facebook fundraisers.

Over the past 15 years, the Amyloidosis Foundation has been privileged to provide over $1.97 million to medical research for the disease and to support dozens of young investigators who have presented their work at international meetings.

We are so very thankful to our donors, to the patients and families and corporate sponsors who have made this effort possible and to our staff, board members and advisors who have led us in these endeavors. **AF**

# How To Make AF Part of Your Estate Plan

Even though planning your estate isn't a fun job, it's necessary so that you can efficiently take care of those you leave behind. There are many different ways to leave a charitable legacy, such as naming a Gift Fund. It's important to get legal or tax advice and think through how each asset will pass to your beneficiaries. A well planned estate avoids confusion for your loved ones.

Still, with all the advantages of estate planning, many people make many mistakes in the process. The most common mistake is not getting around to doing it at all. All of your financial papers should be in order so that it's easy for someone to find them. Make sure that one of your loved ones has information on where to find the papers necessary for planning after your death.

Here are some simple ways that you can make the Amyloidosis Foundation part of your estate plan.

**1. Name Amyloidosis Foundation as a beneficiary** if you are using a will without a trust. You can name us as a remainder beneficiary that receives all of your assets after all expenses and debts are paid.

**2. If you are using a living trust,** consider putting a revision within your trust that points your trustee in the direction of making a distribution of trust assets directly to the Amyloidosis Foundation.

**3. If you have a life insurance policy or a retirement savings plan,** the Amyloidosis Foundation can be named as a beneficiary, if you have an asset that allows for beneficiary designation.


**4. If you are worried that you will have enough assets to make a charitable gift,** you can include provisions that give the trustee flexibility when it comes to the amount of assets that should be given to the Amyloidosis Foundation.

**5. Charitable bequests are a great way to continue to support a cause that is important to you beyond your lifetime.** You can state a specific amount, a percentage of your estate or specific assets. A charitable bequest to the Amyloidosis Foundation can reduce, or even eliminate the estate tax burden.

**6. It is possible to include charitable giving while you are still living.** You can opt to make donations in order to reduce the size of your estate for federal tax purposes, while still accomplishing your philanthropic goals.

Take the time to plan for your death even if you think that you have years before it becomes an issue. The key to successful estate planning is being prepared. Setting up an estate plan, complete with key documents, such as a will and durable power of attorney, is one of the most important ways to protect your loved ones—and yourself. **AF**

## *Untangling Amyloidosis Symposium at ASH Conference*

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bone marrow plasma cells which produce the light chains.

He showed that the likelihood of organ function improving correlated with the reduction in light chains (a concept he previously established), but also showed some data from Pavia suggesting that plasma cell reduction to the lowest possible levels ("Minimal Residual Disease negative", where less than 1 in 10,000 bone marrow cells are residual abnormal plasma cells) may result in the highest chance of organ function recovery.

Finally, he discussed the importance of careful selection of patients for high dose therapy and autologous stem cell transplantation.

**Dr. Suzanne Lenzsch (http://bit.ly/Lenzsch)** from Columbia University discussed the evolving role of incorporating anti-CD38 antibodies (daratumumab and isatuximab) into the treatment of AL amyloidosis. CD38 is a protein expressed predominantly on plasma cells, so targeting it minimizes toxicity to other cells or tissues.

Daratumumab, which induces responses in approximately 30-40% of patients with relapsed multiple myeloma (a plasma cell cancer), seems to have more activity in AL amyloidosis, with response rates of 65-85% reported to date.

During the ASH 2018 meeting this year, Dr. Vaishali Sanchorawala from the Boston University Amyloidosis Program shared data from a prospective trial of Daratumumab in relapsed AL amyloidosis, in which 17 out of 21 patients exhibited a Very Good Partial Response (i.e., a high quality reduction in light chains) after just a single dose of the antibody (Abstract # 2005).

**Dr. Ashutosh Wechalekar (http://bit.ly/Wechalekar)** from London discussed the potential role of doxycycline as a supportive therapy in AL and ATTR amyloidosis. He walked the audience through some complicated basic science research showing that this antibiotic (commonly used to treat skin infections, acne, and rosacea) has a destabilizing effect on amyloid fibrils.

This is predicted to slow accumulation of amyloid in the organ and may make it easier to clear existing deposits.

He reviewed data from the Mayo Clinic originally presented at the ASH 2012 meeting and from a European registry, showing that patients with cardiac amyloidosis, in particular, may benefit from therapy with doxycycline (in addition to standard therapies such as chemotherapy or autologous stem cell transplantation).

**Dr. Jeffrey Zonder (http://bit.ly/JZonder)** from the Karmanos Cancer Institute discussed the latest available information regarding anti-fibril antibody therapies. In contrast, the traditional therapy, which targets the plasma cells making the abnormal amyloid-forming light chains, these anti-bodies target the abnormal light chains and amyloid deposits themselves, helping the immune system to clear them.

Until recently, three different programs were in development. Two of them were discontinued earlier this year

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(Prothena's NEOD001 and GSKs anti-SAP agents miridisap and dezimizumab), leaving one – CAEL101 – in development.

The results of a previously-reported Phase I trial which demonstrated evidence of heart and kidney function improvement were reviewed. Dr. Divaya Bhutani from Columbia University presented research at the meeting (Abstract # 958) demonstrating treatment with CAEL101 improved cardiac function in AL amyloidosis patients with documented cardiac involvement by amyloid, but not in patients without cardiac involvement.

**Dr. Sascha Tuchman (http://bit.ly/STuchman)** from the University of North Carolina provided an exceptional review of an exciting topic in amyloidosis therapy: RNA interference agents.

This is a hot topic as two agents have recently been approved for patients with hereditary forms of ATTR amyloidosis with disease-related neuropathy: the intravenous drug patisiran, and the subcutaneously-administered drug inotersen.

Both drugs were shown in randomized trials to effectively suppress production of the amyloid-forming transthyretin protein and thereby slow or even reverse the progressive neurologic damage which occurs in the disease.

Dr. Thomas Brannagan from Columbia University provided an update on inotersen during the meeting (Abstract # 498) which revealed sustained responses without any obvious new long-term toxicities related to ongoing transthyretin suppression.

During the Friday symposium, Dr. Tuchman also discussed the potential of a newer technology (CRISPR) to effectively permanently edit the gene responsible for the abnormal protein in patient with ATTR amyloidosis.

**Dr. John Berk (http://bit.ly/DrBerk)** from Boston discussed other emerging therapies for ATTR amyloidosis, including a class of drugs called tetramer stabilizers, which keep the transthyretin molecule in a conformation which cannot be easily incorporated into amyloid fibrils.

Tafamidis is one such agent, and Dr. Berk focused on the results of a recent randomized study which is expected to lead to FDA approval of the drug this year based on improvement in overall survival in tafamidis-treated patients compared to placebo.

He also spent some time talking about new strategies to minimize organ damage from amyloidosis, including focusing on an intracellular pathway called the Unfolded Protein Response (UPR).

Overall, the meeting should reassure amyloidosis patients, their families, and the clinicians managing this complex disease that progress in our understanding of amyloidosis disease mechanisms and the development of new therapies leveraging this knowledge continues to move forward. **AF**

***Contact us if you'd like more information on patient resources.***

***1.877.AMYLOID or***

***info@amyloidosis.org***

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FROM OUR FAMILY TO YOURS

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