



## News and Stories - Winter 2024

### Nashville Support Group Meeting

There were 45 people in attendance for this meeting and it was discussed about starting a support group for caregivers. Dr. Wall presented on the ongoing CT using PET/CT with a radioisotopes designed to help identify amyloid fibrils for diagnosis, staging, organ and tissue involvement and to monitor treatment response.

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**Amyloidosis and Cancer Theranostics Program**

**ACTP**  
Dr. Steve Kennel  
Dr. Manasi Balachandran  
Dr. Trevor Hancock  
Alan Stuckey  
Tina Richey  
Sallie Macy

**UTM**  
Dr. Ron Lands  
Dr. "Woody" Besozzi  
Dr. Dustin Osborne  
Dr. Yitong Fu  
Dr. Dustin Powell  
Bryan Whittle  
Barbara Marine

**UTM**  
Jennifer Ferris  
Dr. John Bell  
Dr. Rod Ramchandren  
Dr. Muddassar Mehmood  
Dr. Renju Raj  
Brett Hines  
Dr. Eric Heidel

**NIH** National Institute of Diabetes and Digestive and Kidney Diseases  
**NIH** National Heart, Lung, and Blood Institute  
**attralus**  
**amyloidosis foundation**  
**AMYLOIDOSIS SUPPORT GROUPS**  
**UT THE UNIVERSITY OF TENNESSEE MEDICAL CENTER** Wisdom for Your Life.

## SAVE THE DATE!



"Light The Night For Amyloidosis" is our annual amyloidosis awareness campaign. It was created to draw attention to symptoms, diagnosis, treatment, and hopefully someday, a cure!

Since 'Amyloidosis Awareness Month' also occurs in March, we are asking everyone to light their entryway, home or business with red bulbs for the month of March.

We have also contacted sites across the globe to do the same. Last year we had over 100 sites in 5 countries light for our cause! We have red bulbs available for purchase on our website at: <https://amyloidosis.org/node/124>

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Education • Awareness • Support • Research

[www.amyloidosis.org](http://www.amyloidosis.org)



## Are You Using a Matching Gift Program?

Matching gift programs offer a way for companies to support the charitable giving of their employees by matching the donations they make to nonprofits. A matching gift program allows employers to support employee donations to the Amyloidosis Foundation and help make an impactful gift even more so by doubling or tripling the gift—at no additional cost to the employee. If you aren't utilizing a matching gift program, you could be passing up easy money!

How do you ask for a matching gift?

Here's what you can do: Step 1: Contact your employer's HR head to see if they offer a matching gift program to increase your donation. Step 2: Your HR head will point you in the right direction and let you know if you need to fill out any necessary forms and be aware of submission deadlines.



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## 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
- Listing of experienced physicians that specialize in amyloidosis. Email us anytime with questions: [info@amyloidosis.org](mailto:info@amyloidosis.org)
- Treatment Centers (US / International)
- Support Groups
- Newsletters
- Webinars
- Caregiver/Patient Binder
- Fundraising Toolkits

## Follow Us!



Stay connected for all the latest information on Amyloidosis:

Web: [www.amyloidosis.org](http://www.amyloidosis.org)  
 Twitter: [@Amyloidosisfdn](https://twitter.com/Amyloidosisfdn)  
 Facebook: [@amyloidosisfdn](https://www.facebook.com/amyloidosisfdn)  
 Instagram: [@amyloidosisfoundation](https://www.instagram.com/amyloidosisfoundation)





## Share Your Journey!

It's often helpful to those newly diagnosed to hear from a patient who has been dealing with amyloidosis. A diagnostic journey begins when a patient first seeks medical care for his or her symptoms and ends when a correct diagnosis is given for these symptoms.

An expectation of modern medicine is that if one becomes sick, one can go to the doctor and quickly receive a diagnosis and, hopefully, treatment. Was this your case?

Share your journey with [kathi@amyloidosis.org](mailto:kathi@amyloidosis.org) for use on our website!

Use the link below to read our Patient Stories:

<https://amyloidosis.org/category/stories/>



**Share Your  
Story**

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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](mailto:info@amyloidosis.org), (248) 922-9610  
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](mailto:info@amyloidosis.org)



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## WAINUA™ (eplontersen) granted first-ever regulatory approval in the US for self-administered treatment

US FDA approval was based on a 35-week interim analysis from the NEURO-TTRansform Phase III results, and showed WAINUA™ demonstrated consistent and sustained benefit improving neuropathy impairment and quality of life. Additional regulatory reviews underway in the rest of the world.

AstraZeneca and Ionis' WAINUA™ (eplontersen) has been approved in the US for the treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis in adults, commonly referred to as hATTR-PN or ATTRv-PN. WAINUA™ is the only approved medicine for the treatment of ATTRv-PN that can be self-administered via an auto-injector.

Michael J. Polydefkis, M.D., Professor of Neurology at Johns Hopkins University School of Medicine and an investigator in the NEURO-TTRansform study, said: "Many people living with hereditary transthyretin-mediated amyloid polyneuropathy are unable to fully enjoy their lives because of the relentless, progressive and debilitating effects of the disease. Approval of WAINUA™ represents a meaningful advancement in treatment, one that gives those who are living with transthyretin-mediated amyloid polyneuropathy help managing the disease."

Ruud Dobber, Executive Vice-President, BioPharmaceuticals Business Unit, AstraZeneca, said: "There is an urgent medical need for new therapies for people living with hereditary transthyretin-mediated amyloid polyneuropathy. The US approval of WAINUA™ offers a new treatment option that provides consistent and sustained reduction in serum TTR concentration compared to baseline while halting disease progression and improving quality of life for people living with this debilitating condition."

ATTRv-PN is a debilitating disease that leads to peripheral nerve damage with motor disability within five years of diagnosis and, without treatment, is generally fatal within a decade. WAINUA™ is a ligand-conjugated antisense oligonucleotide (LICA) medicine designed to reduce the production of TTR protein at its source to treat both hereditary and non-hereditary forms of transthyretin-mediated amyloidosis (ATTR).

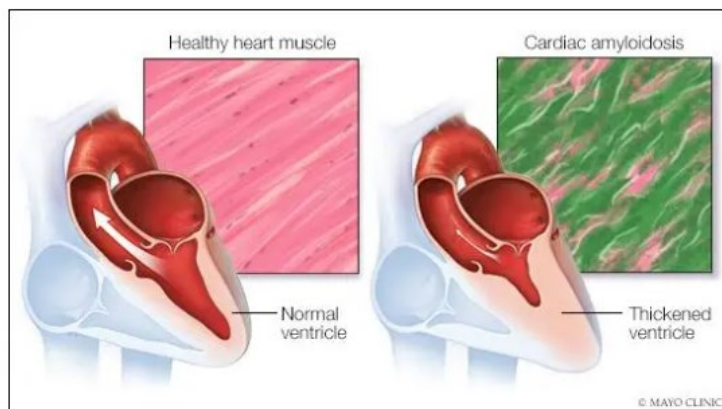
Eplontersen is currently being evaluated in the CARDIO-TTRansform Phase III trial for treatment of transthyretin-mediated amyloid cardiomyopathy (ATTR-CM), a systemic, condition that typically leads to progressive heart failure and often death within three-to-five years from disease onset.

Learn more here: <https://bit.ly/48xpFa2>

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## Cardiac Amyloidosis: Is it AL, hATTR or ATTRwt?



Cardiac amyloidosis is one of the leading causes of restrictive cardiomyopathy. It typically presents with rapidly progressive diastolic dysfunction in a non-dilated ventricle. It is one of the under-diagnosed disease entities, according to the National Institute of Health (NIH). The diagnosis of cardiac amyloidosis requires a high degree of suspicion, with cardiovascular imaging being pivotal in reaching the diagnosis.

There have been many great advances in imaging modalities to diagnose cardiac amyloidosis sooner, but what is it and what types of amyloidosis can it be? Cardiac amyloidosis is the term used when amyloid protein deposits are found in the heart. Cardiac amyloidosis can be one of three types; AL (light chain), hATTR (hereditary amyloidosis, also called familial amyloidosis and ATTRv), or ATTRwt (wild-type).

Symptoms of AL and both types of ATTR amyloidosis are determined by the organs involved in the disease. In cardiac amyloidosis, amyloid protein deposits in the heart muscle causes it to become thick and stiffened. Over time, this can cause the heart to become weak. Amyloid protein deposits in the heart can lead to:

- Congestive heart failure. Symptoms include shortness of breath during activity or while at rest, fatigue, fluid buildup in the abdomen and legs, and difficulty lying flat at night.
- Heart rhythm abnormalities. Symptoms include lightheadedness, dizziness, palpitations, shortness of breath, and fatigue.
- Valvular disease. If amyloid deposits in the valves in the heart, this can lead to leaky (regurgitant) or narrowed (stenotic) valvular disease. Specifically, ATTR amyloidosis has been found in some patients being treated for severe aortic stenosis. Symptoms include shortness of breath, exercise intolerance, lightheadedness and dizziness.

Treatment of cardiac amyloidosis requires a two-pronged approach. Some therapies aim to alleviate cardiac symptoms and complications, while others treat the underlying condition. Treatments can include medications, dietary modifications, and, in some cases, heart and/or liver transplantation.

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## American Society of Hematology Meeting

Amyloidosis Foundation volunteers, Lori Lawter, Adrienne Molteni, and Leslie Schumacher joined over 30,000 attendees for the 2023 American Society of Hematology (ASH) meeting in San Diego, CA December 8-12. There were over 7,400 abstracts accepted, making this a very busy weekend. It was a fantastic meeting with lots to learn and some very exciting advances in the field of hematology. There was heavy emphasis on equity and inclusion in trials and treatments, CAR-T Therapy advancement, and the use of AI in diagnostic and treatment algorithms.

The Amyloidosis Foundation had a booth in the main section of the conference with hundreds of interested individuals stopping by to ask questions or request information on amyloidosis and the role of the Foundation in supporting patients and physicians.

(Continued on page 7)

## Meet Our Grant Awardees

We are proud to feature our grant recipients, whose research targets the challenges in the field of amyloidosis. Congratulations to them both!



### **Joban Vaishnav, MD**

*Deep Clinical and Molecular Phenotyping of Transthyretin Amyloid Cardiomyopathy*

Amyloidosis Foundation Research Grant, 2024

Johns Hopkins University, Baltimore, MD



### **Emre Karayol, MD**

*Identification of Novel Therapeutic Targets within the Proteostasis Network in AL Amyloidosis*

Amyloidosis Foundation Research Grant, 2024

Brigham and Womens Hospital, Boston, MA

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## American Society...

(Continued from page 6)

Notably, Oshrat Rokah, one of our grant recipients stopped by to thank the Foundation and say hello. She presented last year on AL "drug repurposing" and hopes to present again at ISA 2024.



Several attendees asked about patient education materials in Spanish, French, and Dutch, demonstrating the felt need for education in all areas of the globe.

There were several amyloidosis sessions covering ongoing clinical trials, current treatments, diagnostic tools in use today, prognostic indicators, and imaging.

Dr. Jeffrey Zonder presented UNTANGLING AMYLOIDOSIS 2023: DIAGNOSIS, PROGNOSIS, SELECTION OF INDIVIDUALIZED THERAPY, AND MANAGEMENT OF DIFFICULT CLINICAL COMPLICATIONS in a satellite symposium on Friday, December 8th to a standing-room-only crowd. The Amyloidosis Foundation supported this symposium, it was so exciting to see the interest in amyloidosis and hear the many questions posed at the end.

Drs. Jon Wall, Emily Martin, and Eric Heidel presented a poster on Structural and Functional Cardiac Parameters in Patients with AL Amyloidosis Correlate with Uptake of the Amyloidophilic Radiotracer Iodine (124I) Evuzamitide. They also stopped by the booth and updated us on ongoing clinical trials at UT Knoxville.

We met with other amyloidosis patient advocates for dinner and some industry representatives where we shared good conversation and ideas on increasing support group attendance. All in all, it was a great conference and while the understanding of amyloidosis has come a long way, there are still some gaps in knowledge, especially outside of centers of excellence. This conference provided lots of hope that the field is advancing and that patients will continue to benefit from new therapies and a greater global understanding of this disease.

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