



## News and Stories - Winter 2022

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### Eplontersen granted Orphan Drug Designation in the US

In an AstraZeneca press release at the end of January, it was stated that Eplontersen has been granted Orphan Drug Designation (ODD) in the US by the Food and Drug Administration (FDA) for the treatment of transthyretin-mediated amyloidosis. The press release follows:



Eplontersen, formerly known as IONIS-TTR-LRx, is a ligand-conjugated antisense (LICA) investigational medicine currently in Phase III clinical trials for amyloid transthyretin cardiomyopathy (ATTR-CM) and amyloid transthyretin polyneuropathy (ATTR-PN). It is designed to reduce the production of transthyretin (TTR protein) to treat both hereditary and non-hereditary forms of TTR amyloidosis (ATTR).

ATTR-CM is a systemic, progressive and fatal condition that leads to progressive heart failure and death within four years from diagnosis. It remains underdiagnosed and its prevalence is thought to be underestimated due to a lack of disease awareness and the heterogeneity of symptoms. Hereditary ATTR-PN is a debilitating (Continued on page 6)

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### The Journey Continues By Charlotte Haffner

As most of you know, or have read about the first part of my journey, thirteen years ago (2008), I was diagnosed with AL Cardiac Amyloidosis.

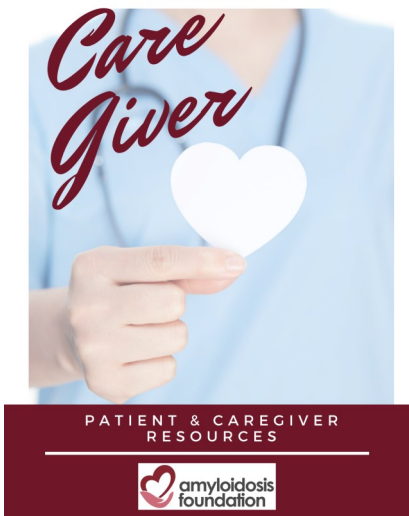
My life went along nicely until 2019. I began to notice changes and after a CT and PET scan, they revealed a tumor laying on top of my left kidney. This tumor was diagnosed as PTLD, or Post-Transplant Lymphoproliferative Disorder. This is brought on by the long-term use of immune suppressant drugs.

I was told at the time of my heart transplant in 2008 that this could happen with long term use of the drugs. I had a good run, but knew I was going to undergo a series of chemo treatments that would be grueling. So, for the next five months, I went to Vanderbilt to receive treatment. For five days, I had five different chemo's going 24 hours a day every 21 days. Then I would return home to recover from the high doses of medication and start the process all over.

Continued on page 4



## Patient/Caregiver Binder



“

Thank you for the binder. It is extremely helpful. It contained more information than I even thought to ask or think about. It actually helped prepare me for my evaluation.

Andrea M.

”

This binder was designed by the Amyloidosis Foundation staff, a team who strive to make care giving easier for caregivers and patients. Each section has links to PDF files that are designed so that you can print your own copy and keep in a 3 ring binder to take with you to doctor's appointments.

**Print your copy here:** <http://amyloidosis.org/resources/#caregiver-resources-binder>

**Email for a binder to be sent:** [info@amyloidosis.org](mailto:info@amyloidosis.org) or **call 248.922.9610** **AF**

### 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)





## President's Corner

Mary E. O'Donnell

I hope you all enjoyed your holidays with friends and family.

As we begin 2022, we are proud to announce that through the generosity of donors like you, our Research Grant Program continues to make a difference. In this issue, we are pleased to introduce the 2022 awardees. Congratulations to all!

You are the reason we are able to carry out the mission of the Amyloidosis Foundation: supporting patients and families while promoting research, education and awareness.

Have a happy and healthy new year,



## Board of Directors

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## Thank You!

Together, we have reached our goal and raised just over \$21,000 in one day on #GivingTuesday! This includes our #GivingTuesday link as well as Facebook Fundraisers that were specifically for #GivingTuesday. We have THE BEST community out there that knows the power of research!



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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**, (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**



## The Journey Continues Continued from page 1

By the end of September, the tumor was gone and I was on the mend. I won't say that it didn't take a toll on me, but for the next year I bounced back rather well. 2021 came in with a storm as my kidney started failing. This was somewhat of a shock, as they had been doing good. Then all of a sudden, my creatinine levels started going up, and my eGFR went down, down and down.



My doctors caught this trend and recommended a kidney transplant. Unfortunately, the amyloidosis that caused all of my problems and the light chains revealed that I was no longer in remission. I got turned down for the transplant until I could reduce my light chains which meant more chemo. I began to feel really bad and lost weight as well as became anemic. I knew something had to change, so my renal doctor suggested dialysis until I could get back in shape.

I chose the peritoneal catheter that you can do at home while sleeping. On September 28th, I went into surgery and had the PTD catheter put into my stomach so that I could do the treatment at home. The procedure was a little dicey, but I had a great surgeon and all turned out well. After several days in the hospital, I went home.

Unfortunately, I did not do well at home. I couldn't hold down anything that I ate, so I went back to the hospital for a week. I began on the cardiac floor as my heart needed some serious monitoring, but soon they moved me to the kidney transplant floor where the dialysis was started for the next 4 days.

The toxins were being removed from my kidneys and I was getting better. I finally was able to go home with 3 days of dialysis to get ready for my training for the PT catheter that lasted 5 days and then I was on my own. My machine was delivered and my dialysis nurse came out to make sure everything I needed was there as I prepared to do it on my own. So far, I have been doing well and all is going good with the process.

Even though the journey continues, there is so much hope because of the research and the awareness of the disease today. My life has forever changed. I am so thankful to God, my family and the Vanderbilt doctors for the last 13 years of my life.

*Charlotte Haffner became Vanderbilt University's first patient to undergo a heart transplant followed by a stem cell transplant to treat amyloidosis.*

*She now dedicates her time to raising awareness about the amyloidosis, working tirelessly to have March declared Amyloidosis Month. She has been on the Amyloidosis Foundation's Board of Directors since 2014.*

**AF**



## Clinical Trials

Did you know that clinical trials for amyloidosis are always ongoing? Patients and families can search for actively recruiting studies that you may be able to participate in or learn about new interventions/treatments that are being considered.

Each study record includes a summary of the study protocol, including the purpose, recruitment status, and eligibility criteria. Study locations and specific contact information are listed to assist with enrollment.

Clinicaltrials.gov is a free service of the National Institutes of Health (NIH) and is maintained by the National Library of Medicine (NLM).

**clinicaltrials.gov** is updated daily. Please check the site frequently for new information.

Go to **clinicaltrials.gov** and type “amyloidosis” in the search bar.

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**DID YOU  
KNOW?**

## Light The Night For Amyloidosis

“Light the night for amyloidosis” is an awareness campaign created to draw attention to amyloidosis symptoms, diagnosis, treatment and hopefully a cure!

Since ‘Amyloidosis Awareness Month’ occurs in March, we are asking everyone to light up their porch / entryway with red bulbs for the month of March.

We have also contacted many worldwide landmarks and businesses to do the same in March 2022.

The ‘Light The Night for Amyloidosis’ campaign in March 2021 was a great success! The Amyloidosis Foundation had great participation, with over 100 sites in 5 countries lighting for our cause.

We have red light bulbs available for purchase on our website!

Please join us in our ‘Light the Night for Amyloidosis’ awareness campaign.

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## Eplontersen...

(Continued from page 1)

disease that leads to peripheral nerve damage with motor disability within five years of diagnosis and, without treatment, is generally fatal within a decade. The FDA grants ODD status to medicines and potential new medicines intended for the treatment, diagnosis or prevention of rare diseases or disorders that affect fewer than 200,000 people in the US.

Mene Pangalos, Executive Vice President, BioPharmaceuticals R&D, Astra-Zeneca, said: "Eplontersen has the potential to be a best-in-class treatment to halt the progression of transthyretin-mediated amyloidosis and treat this fatal condition. The FDA designation further underscores the potential for eplontersen to offer new hope to this patient population currently faced with limited treatment options."

As part of a global development and commercialization agreement with Ionis Pharmaceuticals, Inc. (Ionis), eplontersen will be jointly developed and commercialized by both companies in the US, and will be developed and commercialized in the rest of the world, except in Latin America, by Astra-Zeneca.

Hereditary ATTR-PN is expected to be the first indication for which the companies will seek regulatory approval for eplontersen, with the potential to file a new drug application with the US Food and Drug Administration by the end of 2022.

**AF**

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## Cocktails 4 A Cause



**THANK YOU** to the Conway family for your never-ending support and awareness of amyloidosis! At their event, 'Cocktails For A Cause', they were able to raise over \$3600 at the event and in donations to the foundation in Jason's name! YOU ARE TRUE WARRIORS and we appreciate you immensely!!!

If you are interested in hosting an event to benefit the Amyloidosis Foundation, email: [kathi@amyloidosis.org](mailto:kathi@amyloidosis.org).

We have toolkits available to help you plan!

**AF**



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# Announcing our 2022 Grant Recipients

## Meet our grant awardees

We are proud to feature our annual grant recipients, whose research targets the challenges in the field of amyloidosis.



**K. H. Vincent Lau, MD**

*Evaluating Plasma Neurofilament Light Chain as an Early Biomarker for Polyneuropathy in V122I Hereditary Transthyretin Amyloidosis*

Amyloidosis Foundation Research Grant, 2022  
Boston University, MA



**Zainul S. Hasanali, MD, PhD**

*Targeting amyloidosis through study of calcium dependent endoplasmic reticulum resident protein folding chaperones and their effect on antibody production in plasma cells*

Amyloidosis Foundation Research Grant, 2022  
University of Pennsylvania, PA



**Taxiarchis Kouelis, MD**

*Characterizing the Role of the Tissue Immunome in the Pathogenesis of Renal AL Amyloidosis*

Amyloidosis Foundation Research Grant, 2022  
Mayo Clinic, Rochester, MN

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## Rare Disease Day is February 28!

### Save the date

for #RareDiseaseDay at the National Institutes of Health (NIH). The virtual event will be held on Monday, February 28, 2022.

Each year, the National Center for Advancing Translational Sciences and The NIH Clinical Center co-

sponsor this event to raise awareness about rare diseases, the people they affect and NIH collaborations to advance research for new treatments.

**Register today to attend:** <https://bit.ly/3fYUECN>

**AF**



**RARE DISEASE DAY** at NIH

Feb. 28, 2022 | #RDDNIH

Virtual Conference



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