



# amyloidosis foundation



## News and Stories - Spring 2018

### TN First State to Recognize Amyloidosis Awareness Month

Jessica Pasley, *The Reporter* magazine, Vanderbilt University Medical Center (reprinted with approval from the author)

Ten years ago, Charlotte Haffner became Vanderbilt University's first patient to undergo a heart transplant followed by a stem cell transplant to treat a plasma cell disorder called AL amyloidosis.

The groundbreaking approach used in the current management of the disease saved her life, and now she dedicates her time to raising awareness about the rare disease. For the past year,

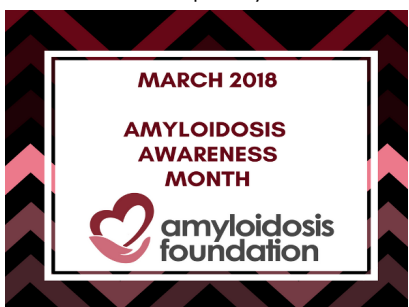
Haffner, Amyloidosis Foundation board member, has worked tirelessly to have March declared Amyloidosis Awareness Month in Tennessee. On March 14, House Joint Resolution No. 711 was signed by Gov. Bill Haslam. The declaration was adopted on Feb. 26 making Tennessee the first state to champion the disease.

"This is just such a difficult disease to have and to diagnose," said Haffner, 67. "Raising awareness among physicians is so very important so that it can be caught in time for treatment. I am hoping that this resolution will help in our efforts to bring this more to the forefront and allow more people to learn about it."



From left, Charlotte Haffner, Pat Dunn, Jonathan Wall, PhD, director, Amyloidosis and Cancer Theranostics Program at UT Medical Center, Emily Martin, PhD, Amyloidosis and Cancer Theranostics Program at UT Medical Center and Rep. Glen Casada look on as Gov. Bill Haslam signs House Joint Resolution No. 711.

(Continued on page 7)



### AF Hosts Annual Golf Outing in Michigan

The Amyloidosis Foundation is thankful for all the support from our local community in Clarkston, MI for our annual golf event on Saturday, May 12 - held at the Fountains Golf



Course. We had over 70 golfers brave the wind, rain and chilly temps to



raise funds for amyloidosis research. Most have participated in this event for many years, with a special connection to the foundation.

We had lots of basket raffles with wonderful prizes donated by local companies plus sponsors that were generous to support the outing. Join us next year in May for another great day of golf! **AF**



**Interested in organizing a golf fundraiser?** Check out our toolkit online: [www.amyloidosis.org/resources/](http://www.amyloidosis.org/resources/)

**Education • Awareness • Support • Research**

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



## Amyloidosis Foundation Celebrates 15th Anniversary!

As we look back on our past years, we asked previous board members to share their thoughts and what impact the AF has made in the amyloidosis community.

“The Amyloidosis Foundation’s mission, to support research and raise awareness of the rare disease amyloidosis, is to be commended.

The AF has raised more than \$1.9 million dollars for medical research for amyloidosis. Most importantly, it has supported dozens of young investigators to present their work at international amyloid meetings.

This support is of great importance in enticing

investigators to enter the field of amyloidosis.”

**Robert A. Kyle, MD**  
Mayo Clinic



“The Amyloidosis Foundation is unique in its approach, providing a global resource to patients, caregivers, physicians and scientists.

It supports education and research by providing grants and resources to young scientists. Its mission is to

serve as a resource for patients, to obtain evaluations and treatment with amyloid diseases.

Established in 2003 by Mary O’Donnell and her late husband Don Brockman, a patient with AL amyloidosis, the AF was the first patient/caregiver led network to increase awareness and impact the outcome of patients with amyloidosis.”

**Vaishali Sancharawala, MD**  
Boston University School of Medicine, Amyloid Center

### Anniversary Open House

September 20, 2018

Fountains Golf & Banquet Center

Clarkston, MI

**\*\*Details to follow\*\***

## 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](mailto:info@amyloidosis.org)
- Treatment Centers (US / International)
- Support Groups
- Newsletters
- Webinars
- 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)

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## President's Corner

In 2018, the Amyloidosis Foundation is proud to be celebrating our 15th anniversary with all of you. It's hard to believe the AF has come so far - what began as a small idea has become an international resource for patients, families, physicians and more. We look forward to the next years with hope for a cure. So much is possible when we all come together, I know we can achieve it.

Spring is always a busy month for the foundation. We hosted our booth at the American College of Cardiology (ACC) conference, attended Rare Disease Week in Washington, DC, held awareness events in Michigan and Tennessee for Rare Disease Day and most importantly, had Amyloidosis Awareness month officially recognized in these two states. Many of you have contacted us for help to do something similar in your state. If you are interested, please call or send us an email ([info@amyloidosis.org](mailto:info@amyloidosis.org)) so we can assist you.

With grateful appreciation,  
Mary E. O'Donnell

### **Save the date!**

**3rd Annual Pittsburgh Amyloidosis Research Benefit**

**Friday, October 26, 2018**

**Join us and enjoy a gourmet dinner, live music, wine raffle, silent auction, and more—all in the name of raising funds for amyloidosis research.**

**Montour Heights Country Club**

**Tickets \$175**

**For tickets & further details visit: [www.amyloidosis.org](http://www.amyloidosis.org)**

## **Board of Directors**

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Jonathan Wall, PhD

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), **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](mailto:info@amyloidosis.org)**



## Michigan Rare Disease Day a Success

The Amyloidosis Foundation co-chaired the annual Rare Disease Day event in Lansing on March 8, bringing together over 75 patients, caregivers, family and friends on this special day.

This was held in the Speakers Library at the Capitol and the agenda included Rep. Jim Tedder and State Senator Margaret O'Brien. Both of these legislators are supporting a bill to form a Rare Disease Advisory Council in Michigan, to give the rare disease community in a voice in state government.

The foundation was grateful to have our dear friend Nancy Sullivan speak about her late husband, Kevin, and share his amyloidosis journey. Her son,



Connor, (both in the photo above) was there to show his love and support. Nancy's pledge to Kevin was to spread awareness of this rare disease and work to educate physicians for an increase in early diagnosis.

We appreciate Alnylam for sponsoring the lunch for all attendees and speakers and grateful for their continued support for amyloidosis patients. **AF**



Some of the attendees at Michigan Rare Disease Day

## Cardiac Amyloidosis Webinar- Often Overlooked, Not Uncommon & Manageable



Recorded in February and hosted by Mat Maurer, MD from New York-Presbyterian Hospital and Columbia University Medical Center, this webinar defines wild type cardiac amyloidosis in this easy-to-understand program. If you missed the live version or would like to listen again, use the links below.

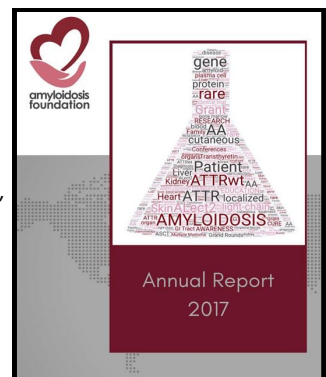
Dr. Maurer describes common symptoms, tips for diagnosis and emerging treatment strategies. Watch this informative webinar to learn more about this overlooked type of cardiac disease.

Find the webinar here online at [www.amyloidosis.org/resources](http://www.amyloidosis.org/resources) or use this link on our **Amyloidosis Foundation YouTube page**: <http://bit.ly/CardiacAmyloidosis>

## 2017 Annual Report

Celebrating an amazing year at the Amyloidosis Foundation, our 2017 report is now available online. We share details on our finances, research grant program, fundraising events, donors and more.

**Check it out today!**





## Rare Disease Week on Capitol Hill—Washington, DC

*Kathi Luis, Amyloidosis Foundation - Special Projects Director*

### Sunday, February 25

I attended a Rare Disease Documentary Screening of 'The Ataxian', which details the journey Kyle Bryant, who has neuromuscular disorder Friedreich's ataxia (FA). He is unable to walk due to the disease but he and a team of friends attempt the most grueling bicycle race in the world. Tears were wept throughout the theatre, which inspired everyone about their own advocacy.

### Monday, February 26

Legislative Conference Day. I spent the day learning the proper protocol of lobbying, learning to prepare for successful meetings, practicing my speech, meeting the other Michigan delegates and learning about the legislative "asks" in depth (OPEN ACT, Advancing Access to Precision Medicine Act and the Rare Disease Congressional Caucus).

### Tuesday, February 27

Lobby Day Meetings with members of Congress. I started my day with coffee with Gary Peters (MI-S-D). He seemed genuinely interested in learning about amyloidosis. I gave my speech and he asked me a few questions about who it affects, what organs are involved and the symptoms. At Debbie Stabenow's (MI-S-D) office, I met with a legislative aide by the name of Lorenzo A. Rubalcava. He was great and told that he would relay our information. In Mike Bishop's (MI-08-R) office, I met with Allie Esau, a legislative correspondent. I told her of

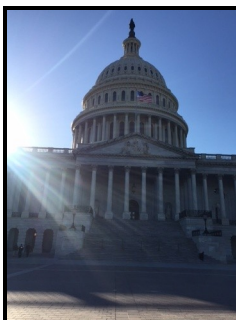


**Kathi Luis (far left) and the Michigan Rare Disease Day delegates in Washington, DC, with Senator Gary Peters.**

Congressman Bishop's strong voting record for those with rare diseases and urged that he cosponsor the OPEN ACT. It was also asked that he join the Congressional Caucus. Although not in our district, I also met with Dave Trott's (MI-11-R) legislative director, Bridget Sobek Dobyen. She has a family member with a rare disease, so we connected on a completely different level. She seemed very open to getting things accomplished as a team dedicated to rare diseases.

### Wednesday, February 28

Attended the Congressional Caucus Briefing, which is a group of over 100 Members of Congress who seek to raise awareness on Capitol Hill.



They gave us updates on the current legislative initiatives and policy issues for rare disease patients, giving them a voice in Washington DC. In the evening, I attended the 'Rare Artist' reception, where art from rare patients was displayed. It was a great networking opportunity.

### Thursday, March 1

Rare Disease Day at the NIH in Maryland. This day was jam packed with speakers. Some of the highlights were an update from the FDA on orphan drugs, groundbreaking work in gene editing, gene therapy and engaging the next generation of the rare disease community. It was amazing what these young individuals have accomplished.

I left DC renewed, filled with the powerful stories I heard and ready to continue advocating for amyloidosis patients and families. **AF**



## Donations from the Heart

When Paige and Juan Sanchez decided to renew their vows and host a formal wedding on October 7, 2017, they opted to do something special in lieu of receiving gifts. They included this poem (written by Paige) in their wedding invitation:

*Since this marriage is not brand new,  
we already have a thing or two.*

*We're in no need of pots or pans,  
we just want to lend a helping hand.*

*We picked an organization close to our  
heart, in hopes that your donation will give  
the cure a start.*

*Your company on our big day is the best  
gift of all, and each donation makes a  
difference, big or small.*



Paige's mother, Jennifer Brink, has ATTR amyloidosis. This is a rare, hereditary disease that has affected many members of her family. The TTR amyloid protein is mainly manufactured in the liver. Jennifer had a liver transplant early in 2017 and wasn't sure if she would be able to attend the wedding.

We are thankful to say she was there to celebrate this happy occasion with her daughter and extended family, as seen in these beautiful photos. Paige was also diagnosed with ATTR in April 2018.

The Amyloidosis Foundation awards research grants annually to those who target the challenges in the field of amyloidosis. In total, Paige and Juan raised over \$1,500 for the Amyloidosis Foundation Research Program. We are grateful for their generosity and support, and wish them a lifetime of happiness.

**AF**



## Amyloidosis Foundation Announces Passing of Board Member

It is with regret that we have lost our good friend and Amyloidosis Foundation Board Member, Darcy Tannehill, who passed away on Saturday, April 21, 2018 after courageously and passionately battling amyloidosis.

She served as the chair of the annual Pittsburgh Amyloidosis Research Benefit (which began in 2016) and her daughter Courtney Sullivan will continue this event in her memory on **Friday, October 26, 2018**. You can donate or purchase tickets online on our website.

She will be greatly missed and we send our heartfelt condolences out to her family and friends for their loss.

Darcy was the Associate Professor of Education at Robert Morris University in Pittsburgh, PA.

Most recently she made a generous gift to the Department of Medicine at the University of Pittsburgh's School of Medicine, establishing the **Dr. Darcy B. Tannehill Amyloidosis Research and Education Fund**.

Darcy made a planned gift to ensure that her hard work will continue.

She hoped that her gift helps Pitt to become a powerhouse in amyloidosis research, ultimately helping to save many lives. She was an inspiration to patients and selfless in spreading amyloidosis awareness. Her legacy will continue as we raise funds and support research for a cure. **AF**





## Richard and Ruth Shapiro Share Their Amyloidosis Foundation Memories

We wish the Amyloidosis Foundation congratulations for their 15th Anniversary. We have been privileged to have had a connection with the foundation since the beginning. Though research is a major force of the AF, we have been very connected to the important efforts of spreading awareness of this rare, serious disease.

When Richard was diagnosed in 1998, there was little information about Amyloidosis. We met Mary on an online support group when her husband, Don, was diagnosed. Mary is a true champion and has taken the leadership of the AF in honor of her amazing husband, Don, who unfortunately

passed away from this disease. Mary has been so true to her promise to Don, to help find a cure and save others.

We are grateful for the continued effort of the foundation to exhibit at global medical conventions to promote education and awareness to physicians and health care providers from around the world. We've enjoyed volunteering at these events. The noticeable increased awareness and knowledge of amyloidosis over the last 15 years has saved lives through



early diagnosis and treatment.

We wish the foundation many more years of success and hope to be a continual part of volunteering for many years to come.

*Richard and Ruth Shapiro*

## TN First State to Recognize Amyloidosis Awareness Month

*(Continued from page 1)*

AL amyloidosis originates in the bone marrow and results when amyloid protein builds up in one or more organs, causing them to malfunction. It is a rare disease with only 1,200 to 3,200 new cases reported each year in the United States.

Amyloid proteins can deposit in the kidney, liver, spleen, heart and bone marrow.



*Rep. Casada signs the bill*

Patients often undergo chemotherapy and, if eligible, a stem cell transplant to help control the disease and ultimately rid the body of amyloid deposits and help improve the function of affected organs.

Many of the patients first present to cardiology when they begin to experience signs of heart failure, a result of gradual buildup of the amyloid protein in the heart. Haffner underwent a heart transplant in November 2008, followed by a stem cell transplant in February 2009.

Once she recovered, she formed a support group for other amyloidosis patients in Nashville, coordinated support group meetings in neighboring counties and was instrumental in the creation of the Vanderbilt Amyloid Multidisciplinary Program (VAMP), which is poised to advance the understanding, treatment and research of amyloidosis.

Haffner's physicians say her dedication to raising awareness about the disease is formidable. "She is motivated to help other affected patients and her ultimate goal is to give some visibility to the challenges faced by patients," said Adetola Kassim, MD, professor of Medicine at Vanderbilt and Haffner's hematologist.

"She is absolutely remarkable in her efforts and passion to move awareness and support of this disease forward," said Mark Wigger, MD, assistant professor of Medicine and Haffner's transplant cardiologist. "She has pulled together folks on the state and national level and brought awareness to many healthcare providers."

Haffner is already busy working with other states to have similar resolutions passed with the overall goal of having a national presence in the coming years. "What I am doing is going to save lives," said Haffner. "It's a good feeling being an advocate." **AF**



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