



News and Stories - Winter 2016

2016 Pittsburgh Amyloidosis Benefit was an Incredible Success!

The Amyloidosis Foundation hosted a benefit at the Montour Heights Country Club in Pittsburgh, PA on Friday, October 28, 2016 and what an amazing night it was! Over 120 people attended this



event to raise funds for our research grant program. The benefit raised over \$41,000.

Darcy Tannehill, Ed.D., who has amyloidosis, and her daughter Courtney Sullivan were Chair and Co-Chairs of the benefit (in the photo to the right). They gathered donations, silent auction items, cases of wine for a "raffle pull" and more.

Live music, fabulous food and a beautiful venue made this evening very special. We are truly grateful for their hard work to make this a success!

Darcy said the goal of the

benefit was to raise amyloidosis awareness and to help researchers develop treatments specifically for this rare disease.



Please save the date, next year the benefit will be on **Friday, October 27, 2017,**

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Amyloidosis Foundation Spreads Awareness at Hematology Conference

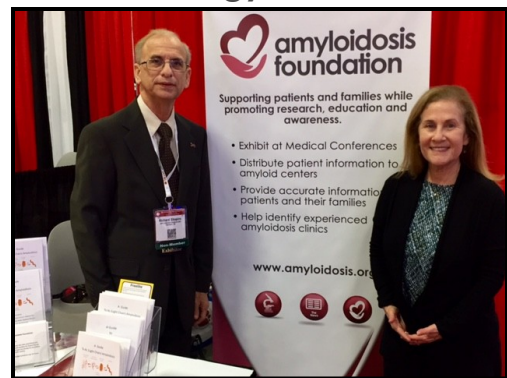
The American Society of Hematology (ASH) held its 58th annual meeting in San Diego, CA on December 3-6, with more than 20,000 hematology professionals in attendance. The ASH Annual Meeting is the world's premier event in malignant and non-malignant hematology.

One of our primary goals is raising awareness of amyloidosis. The Amyloidosis Foundation exhibits at several major medical conferences throughout each year. Thanks to our volunteers, Richard and Ruth Shapiro (see photo to the right),

and AF BOD Dena Heath who attended the ASH event this year. The number of physicians who visited our booth to learn more about amyloidosis exceeded any previous years attendance. Our team distributed

information on the various types of amyloidosis through journal articles and our updated brochures.

There were over 25 presentations from physicians and researchers around the world focusing on amyloidosis treatment.



Over 70 oral and poster abstracts about amyloidosis were featured this year. There was also an educational session that included details on current clinical trials.

Thank you to our volunteers for your support! **AF**

Education • Awareness • Support • Research

www.amyloidosis.org



Amyloidosis Information & Rare Disease Day 2017

Our website has many resources for patients and families: www.amyloidosis.org.

We have links for veterans who were exposed to Agent Orange or other herbicides during military service. Other links have information on Veterans Choice, easy to understand videos about amyloidosis plus options for medically-related transportation.

Our comprehensive list of U.S. and international treatment centers is helpful when newly diagnosed, plus the updated brochures and information on our AF support groups is easy to find.

Did you know the Amyloidosis Foundation has its very own YouTube channel? Here you can also find links to our webinars and the trailer for our upcoming documentary. The link is: www.youtube.com and then type in Amyloidosis Foundation in the search field.



Please share with your friends and family to educate them on this disease and create awareness.

February 28, 2017 is Rare Disease Day, which occurs on the last day of February each year.



Millions of patients and their families around the world share their stories to promote awareness of the challenges, hopes and needs of those living with rare diseases.

Almost every state participates in this important day. Share your story & get involved in your community, use this link and search by your state:

www.rarediseaseday.us/. **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
- Toll Free Number **1-877-AMYLOID**
- Listing of experienced physicians that specialize in amyloidosis. Email us anytime with questions: info@amyloidosis.org
- Treatment Centers (US / International)
- Support Groups
- Newsletters
- Webinars
- Fundraising Toolkits

www.amyloidosis.org

Follow Us



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President's Corner

Through the generosity of donors like you, our Amyloidosis Foundation research grant program continues to grow. We are happy to introduce the 2017 awardees to you in this issue. *Congratulations to all!*

We have new merchandise available on our website: t-shirts, bracelets, lapel pins and our standard note cards. You can order online or call the foundation anytime.

In this newsletter we've also featured some of our fundraising events that were held across the country this Fall. You can help us to find a cure for amyloidosis and continue to fund much-needed research by signing up now for the 2017 events below.

From everyone here at the AF, we wish you all a happy and healthy new year,

Mary O'Donnell

2017 AF Fundraising Events

- **May 7:** Pittsburgh Half Marathon
- **May 13:** AF Annual Golf Event in MI
- **May 21:** Half Ironman in TN
- **August 12:** Prieber Golf Event in MI
- **October 19:** Bike Race/Fun Ride in TN
- **October 21:** AF 5K in MI
- **October 27:** Annual Pittsburgh Benefit

Contact the Amyloidosis Foundation if you would like to participate in these events or if we can help you start your own fundraiser in 2017!



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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**, **1-877-AMYLOID** (877-269-5643) or **7151 North Main Street, Ste. 2, Clarkston, MI 48346**

If you no longer wish to receive this newsletter, please send us an email:

info@amyloidosis.org



Amyloidosis Foundation Awards 2017 Research Grants

The Amyloidosis Foundation awarded four research grants in December 2016. Since 2005, the AF has funded over \$1 million to promising clinical amyloidosis investigators from around the world. We applaud their efforts and look forward to the success this work will bring to patients.

Emily Martin, PhD



A Method to Predict Amyloid Risk in Patients

Amyloidosis Foundation
Donald C. Brockman
Memorial Research Grant,
2017

University of Tennessee Medical Center, Medicine Division:
Amyloidosis

It is estimated that approximately 15% of patients with multiple myeloma will also develop symptomatic light chain amyloidosis in the course of their disease. Since there are now several new amyloid-clearing and preventing drugs in development, it would be beneficial to identify these patients before severe amyloid forms and organ failure occurs so that they can benefit from these new treatments.

This project will evaluate a

novel test for identifying patients with a diagnosis of multiple myeloma that are at risk of developing light chain amyloidosis. We will study light chain proteins, which have been isolated from patients' urine, for their ability to bind to synthetic amyloid fibrils. Our data suggest that light chain proteins from amyloidosis patients show enhanced binding to these fibrils as compared to those from patients with multiple myeloma.

With the help of the Amyloidosis Foundation, we will now focus on further exploring this observation and developing a simple test that can measure the amyloidogenic potential of light chain proteins and, thus, the risk of developing amyloid before symptoms of the disease appear in patients.

Lorena Saelices, PhD



A Novel Strategy for the Treatment of ATTR

Amyloidosis Foundation
David Seldin, MD, PhD
Memorial Research Grant,
2017

Regents of the University of California, Los Angeles

Transthyretin (TTR) is a plasma protein whose abnormal *amyloid* aggregation causes wild-type amyloidosis and some cases of hereditary amyloidosis, referred as ATTR. TTR amyloid deposits are found in virtually every organ of ATTR patients and are made of unbranched fibrils that accumulate mostly between cells.

In our previous work, we identified the two segments of TTR that are crucial for amyloid fibril formation. We designed and characterized two specific small peptides that block TTR fibril formation by interacting with these two segments *in vitro*.

The 2016 Amyloidosis Foundation Research Grant allowed us to evaluate our peptide inhibitors in a ATTR model of the fruit fly, *Drosophila melanogaster*.

The results show that the treatment with our peptide inhibitors improves motor skills and reduces TTR insoluble deposition.

With the support of the 2017 Amyloidosis Foundation Grant, we aim to assess the combinatory effect of our peptide inhibitors and diflunisal, a compound that was tested in clinical trials for the treatment of TTR amyloidosis. Our research may represent a novel strategy for the treatment of ATTR.

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Amyloidosis Foundation Awards 2017 Research Grants

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Petros Moustardas, PhD



Novel Therapy for Renal amyloidosis

Amyloidosis Foundation
Research Grant, 2017

Linköping University, Sweden
Division: Microbiology &
Molecular Medicine

Living cells have a sophisticated quality control system in the Endoplasmic Reticulum (ER) to ensure that the proteins they produce have proper shape and function. When the ER folding capacity is overwhelmed, homeostasis is disturbed, a condition known as ER stress.

Amyloidosis involves the accumulation of insoluble, misfolded proteins in the afflicted tissues. Thus, we believe that the deregulated function of ER components is involved in the underlying pathology. ERdj5 is a chaperone protein resident in the ER and an essential component of the protein quality control system.

Our research has revealed that mice lacking ERdj5 develop spontaneous renal amyloidosis.

We are thankful for the generous support from the Amyloidosis Foundation, this will allow us further exploration of this finding and investigate the pathogenetic mechanisms involved in the development of the observed phenotype. This will also lead to a better understanding of the relation between ER function and amyloidosis and has the potential to generate novel, not suspected thus far, therapeutic targets of clinical significance.



Carolina Lemos, PhD



New Biomarkers for FAP

Amyloidosis Foundation
Research Grant, 2017

University of Porto, Portugal
Division: Molecular Biology

Phenotypic variability on Familial amyloid polyneuropathy (FAP ATTRV30M), a hereditary systemic amyloidosis due to a gene mutation, has been a most intriguing feature and



the object of several studies from our group and others.

Our research has been centered mainly in the study of age-at-onset anticipation mechanisms and the search for genetic modifiers of the disease, in a large group of Portuguese families.

Although some genetic factors associated with phenotypic variability have been found, age-at-onset variability is still not totally understood.

Our aim is to assess differences in gene expression levels, transcription and regulation in discordant relative-pairs for age-at-onset, to identify potential epigenetic modifiers of age-at-onset.

We expect that this project, with the support of Amyloidosis Foundation, may contribute to elucidate some of these mechanisms, which may lead to the development of new biomarkers and to a better follow-up of families at risk. **AF**



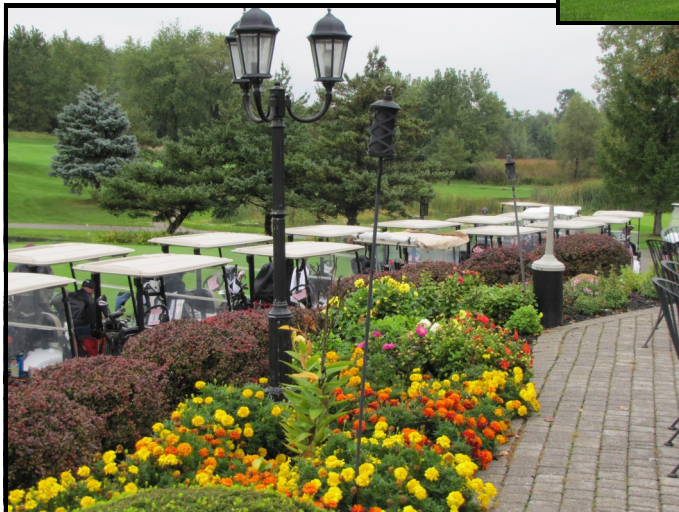
Amyloidosis Foundation Golf Outing - A Hole in One!

Thank you to everyone who participated in our annual AF golf outing at The Fountains in Clarkston, MI on Friday, September 30, 2016. We were lucky enough to have most of the rain hold off until we finished!



Still, a great day was had by all – our raffle had over 40 baskets and gift certificates for our participants, including many families affected by amyloidosis.

This was our most successful golf outing to date, raising almost \$7,200! Special thanks to Prothena for their sponsorship and support. Join us next year on **May 13, 2017! AF**

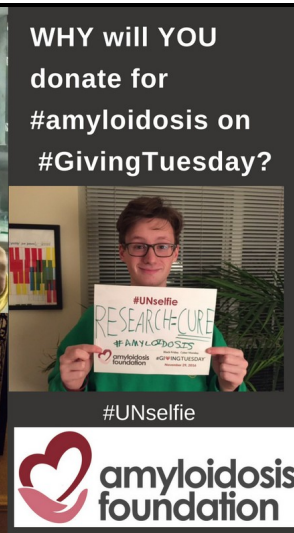
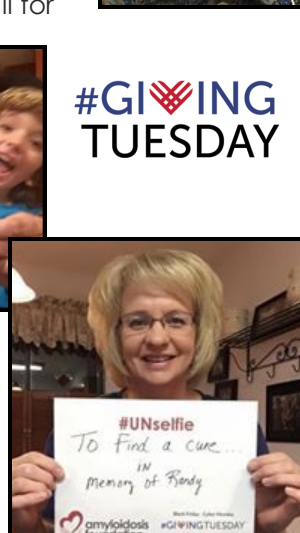
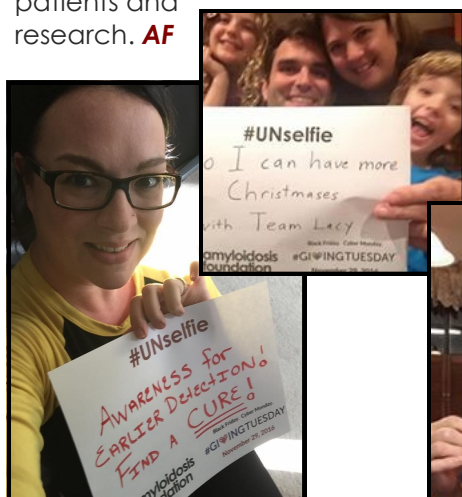
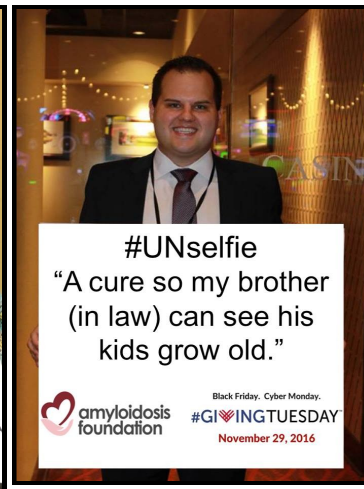
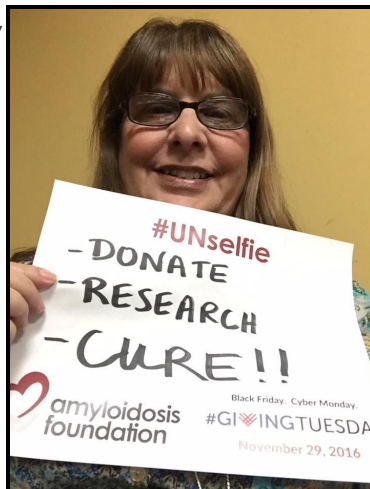




2016 #GivingTuesday—Annual Day of Philanthropy

#GivingTuesday is an international day of philanthropy. This event redefines the giving spirit this holiday season. Thank you to everyone who donated to the Amyloidosis Foundation on November 29th, we appreciate your support.

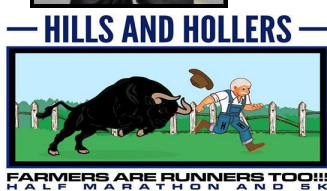
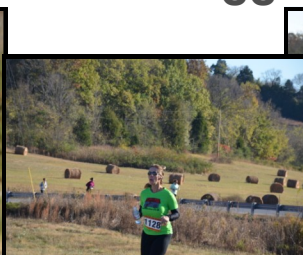
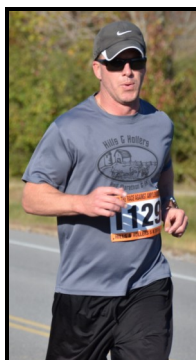
We are thankful for all the #UNselfies that were posted on our Facebook and Twitter pages, explaining why they donated. What an amazing 24 hours—we raised over \$4300 with donations from 53 supporters, up from \$3550 and 38 donors in 2015—all for patients and research. **AF**



Hills & Hollers Half Marathon and 5K— Their Biggest Year Ever!

The 2016 Hills & Hollers Half Marathon and 5K events were a big success this year in Tennessee!

They had 359 runners and raised \$25,000 between the races, their silent auction and donations. The funds will be split between Vanderbilt University Medical Center Stem Cell patients, the Amyloidosis Foundation support group in Nashville, the Amyloidosis Foundation and Secure Harbor. Congratulations runners! **AF**



2016 Pittsburgh Amyloidosis Benefit was an Incredible Success!

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once again at the Montour Heights Country Club in Pittsburgh, PA.

Santhosh Sadashiv, MD, who is Darcy's hematologist/oncologist, spoke about amyloidosis treatments and current clinical trials. Many patients, family members and caregivers were in attendance and everyone felt a sense of community in the room.

Two Pennsylvania public officials have battled amyloidosis, including the late Pittsburgh Mayor Richard Caliguri and Pennsylvania Governor Bob Casey.

We appreciate everyone in the greater Pittsburgh area for attending our event and appreciate your support.

Thank you to Alnylam and Takeda for sponsoring our research benefit. We look forward to seeing everyone next year! **AF**



The Amyloidosis Foundation appreciates your continued support.

If you would like to become more involved in the foundation, interested in starting a fundraiser or becoming an amyloidosis ambassador—we would enjoy speaking with you and helping in anyway we can.

Please call our office today **1-877-AMYLOID** (877-269-5643) or send us an email at **info@amyloidosis.org**.

Thank you!

