



News and Stories - Spring 2017

Spreading Amyloidosis Awareness on Rare Disease Day

by Emily Martin, PhD, University of Tennessee Medical Center

On Tuesday, February 28, 2017, the University of Tennessee Medical Center was thrilled to be able to participate in Rare Disease Day for the first time. With the help of Charlotte Haffner and the Amyloidosis Foundation, in addition to members of the Amyloidosis and Cancer Theranostics Program directed by Dr. Jonathan Wall.

Our research program has studied amyloidosis for more

than 40 years, and as a result, we understand the need to raise awareness and assist in educating both the general population and the medical community on the disease.

Needless to say, we were quite excited to participate in the event!

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Expanding the Screening of Amyloidosis to Larger Populations

by Jose N. Nativi-Nicolau, MD, Jared Cowley, RN, Jo Abraham, MD and Tibor Kovacs, MD
Utah Amyloidosis Program, Huntsman Cancer Institute, University of Utah Health Science Center

Amyloidosis is a rare disease that remains largely under diagnosed. Early diagnosis is limited by multiple factors including lack of awareness of its primary symptoms and ambiguous clinical scenarios that are frequently attributed to other conditions. However, one of the critical challenges for screening is the necessity for sophisticated



evaluations including biopsies, special stains or

even genetic testing which are limited for a majority of primary health centers.

Because of the complexity of the evaluation many patients are never diagnosed or are referred to amyloidosis specialty centers very late in the disease's progression.

Recent advances in the amyloidosis field are providing hope for earlier diagnosis and screening. In 2016, an international study proposed an algorithm for patients with suspected cardiac amyloidosis utilizing bone

scintigraphy and evaluation for monoclonal proteins in blood and urine (Gillmore 2016).¹

In the last two years, the University of Utah Amyloidosis Program has been utilizing the proposed algorithm to screen patients. We have observed several benefits, the key one being that the initial evaluation is non-invasive, making it more attractive to general providers and patients.

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Education • Awareness • Support • Research

www.amyloidosis.org



AF Welcomes New Board Member and Patient

The Amyloidosis Foundation is honored to announce that Darcy B. Tannehill, Ed.D. is a new member of our Board of Directors.

Dr. Tannehill has spent the majority of her career in higher education working in academic affairs, student affairs, enrollment management, international admissions, and campus management. She has held a number of academic and administrative positions in both Pittsburgh and Chicago, including Campus President and Vice President of Online and Off-Campus Programs.

She is currently an Associate Professor of Education at Robert Morris University in Pittsburgh, PA. Her undergraduate degree in psychology and sociology and her master's degree in education are from Duquesne University. Her doctorate in administrative and policy studies—higher education management is from the University of Pittsburgh.

Darcy was diagnosed with light chain amyloidosis in May of 2012, undergoing multiple rounds of chemotherapy and a stem cell transplant. While it took more than six years and visits to eight specialists to get a diagnosis, she is fortunate in that she has no major organ damage.

Her hobbies include Shetland sheepdog rescue, playing the piano, adult learning, leadership, and, of course, amyloidosis.

She is lucky to live less than a mile from her daughter, son-in-law and granddaughter Alaina.

Darcy served as the chair of the Pittsburgh Amyloidosis Research Benefit in 2016. Its success warrants it to become an annual event—mark your calendar and join us on **October 27, 2017. 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

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

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

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Stay connected for all the latest information on Amyloidosis:

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President's Corner

Thank you to everyone who participated in various Rare Disease Day events this Spring, celebrating the 10th anniversary of this important day by spreading awareness and advocating for amyloidosis. The theme was "With research, possibilities are limitless." We know all too well that without strong research we won't have a cure. We appreciate your support.

Our 2016 Annual Report is now online, giving you a snapshot of the work the foundation did last year, with your help. You can find it on our website, www.amyloidosis.org/us. It's an easy way to understand what we do and why we do it: our accomplishments and fundraisers, plus our commitment to research grants.

We are thrilled to announce that Darcy Tannehill has joined our Board of Directors. We know she will be an asset to us and bring a strong voice from the patient perspective.
Welcome, Darcy!

Thanks as always,

Mary O'Donnell



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Upcoming AF Support Group Meetings

Tennessee

Nashville

- April 26
- May 25
- June 28
- July 26

Knoxville

- April 29

Northern California

- April 22
- July 15



For more details and to RSVP, please visit the Resources page on our website: <http://www.amyloidosis.org/resources>.

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 OR if you wish to receive a printed version, please send us an email:

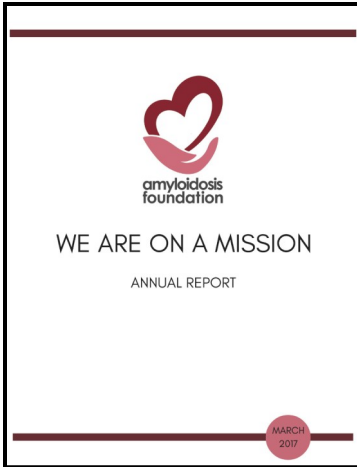
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Our Year in Review—The AF 2016 Annual Report

We are proud to share our 2016 Amyloidosis Foundation Annual Report. We are committed to supporting patients and families while promoting research, education and awareness.



Our report gives details on our finances, research grant program, fundraising events, donors and more. With your help, we will continue to be on a mission for a cure.

2017 Research Grant Recipients

We invest in outstanding scientific research and innovative research models to expedite promising therapies to patients. Through our research program we encourage, promote and invest in the medical study and exploration of the amyloidosis diseases.



Emily Martin - PhD
A Method to Predict Amyloid Risk in Patients
Amyloidosis Foundation Donald C. Brackman Memorial Research Grant
University of Tennessee Medical Center
Medicine Division: Amyloidosis



Lorena Seelices - PhD
A Novel Strategy for the Treatment of ATTR
Amyloidosis Foundation David Seidlin, MD, PhD Memorial Research Grant
Regents of the University of California, Los Angeles



Felice Rowlandson - PhD
Novel Therapy for Renal Amyloidosis
Amyloidosis Foundation Research Grant
Linköping University, Sweden
Medicine Division: Microbiology & Molecular Medicine



Carolina Lemos - PhD
New Biomarkers for FAP
Amyloidosis Foundation Research Grant
University of Porto, Portugal
Medicine Division: Molecular Biology

Awareness in Action



Our year was strong because of YOU, those who raised donations at various fundraisers, those who generously donated, our supporters who put their time and energy into raising awareness while staging events to deliver funds for the Amyloidosis Foundations.

As you can see below, we have many events already planned for this year.

You can find our annual report online at www.amyloidosis.org/us.

If you would like a printed version, please contact the foundation and we would be happy to mail you a copy.

We are forever grateful and look forward to an incredible 2017. Thank you.
AF

2017 Amyloidosis Foundation Fundraising Events

- **May 7:** Pittsburgh Half Marathon (PA)
- **May 13:** AF Annual Golf Event (MI)
- **May 21:** Half Ironman (TN)
- **July 22:** "I Ran for Joann" (MI)
- **August 12:** Prieber Golf Event (MI)
- **October 19:** Bike Race/Fun Ride (TN)
- **October 21:** AF "Run for Your Life" 5K (MI)
- **October 27:** Annual Pittsburgh Research Benefit (PA)

Contact the Amyloidosis Foundation if you would like to participate in these events OR you can donate online to show your support.



Michigan Hosts 3rd Annual Rare Disease Day Event

The Amyloidosis Foundation was proud to co-chair the 2017 Michigan Rare Disease Day event at the State Capitol in Lansing. The weather co-operated and we didn't have to reschedule like last year!

It was an amazing day of advocacy for many rare diseases. We had nine speakers tell their personal stories and why they felt it was important to share their struggles to advance awareness and research.

Participating organizations included: Mowat-Wilson Syndrome, Primary Ciliary Dyskinesia (PCD), Gorham's Stout Disease, Complex Regional Pain Syndrome (CRPS), Von Willebrand Disease, Hemophilia Foundation of Michigan, Dystonia, Shwachman-Diamond Syndrome, SWAN USA, Phelan-McDermid Syndrome, Barth Syndrome and the Amyloidosis Foundation.

One of the goals this year was to work with legislators

Advisory Council (RDAC) in MI. This would enable

the rare community to have a voice in state government. There are currently three states that have successfully implemented a RDAC: Connecticut, Illinois and North Carolina. Others, like Michigan, are working towards this in the 2017 legislative session.



Rep. Adam Zemke, Rare Action Network Ambassador Jen O'Connor, Senator O'Brien who also spoke about her daughters rare disease.



to pass a bill to form a Rare Disease

Thanks to everyone who participated in our event (and others across the US) this year. We look forward to making a difference again in 2018! **AF**



Devin Derusha, 13, who had a heart transplant at 9 weeks old, due to complications from Barth Syndrome. He is a joy!



2017 Rare Disease Day Speakers



Will You Help Us Plan for the Future?

People give their time, talents, and treasures to non-profit organizations because they believe in the mission of the organization and they understand the importance of preserving the programs and services offered.

In the case of the Amyloidosis Foundation, the mission is to fund research, create awareness in the medical community and to provide support through patient education and advocacy programs.

By making us a beneficiary of your will or revocable trust, you'll help guarantee our financial strength tomorrow, without affecting your cash

flow or your family's financial stability. The idea of making a gift may feel intimidating. Actually, a charitable gift is simpler and easy to set up than many people realize.

By making a bequest to the Amyloidosis Foundation, you will join many who have decided that our mission is important and that supporting our cause in the future is imperative.

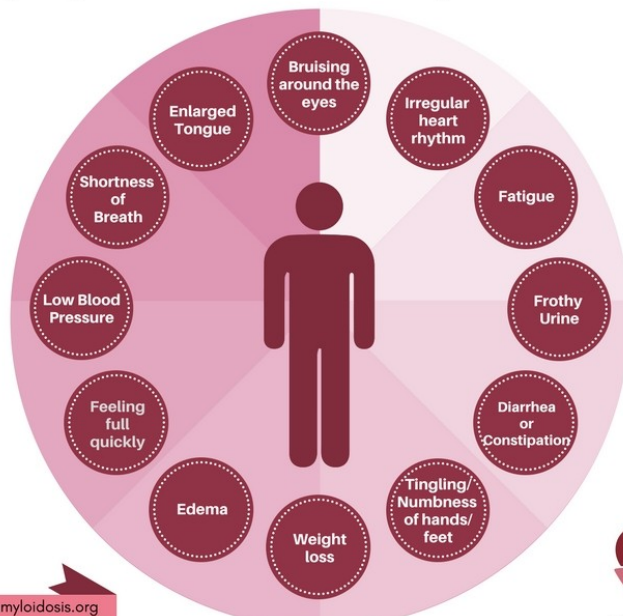
What is a BEQUEST?

Will bequests are the most popular and personal way to making a difference beyond your lifetime.

A bequest is a charitable gift of property to be delivered at the donor's passing. You can create a bequest simply by directing in your will that certain money or property be transferred from your estate to the Amyloidosis Foundation. Bequests from wills vary widely...some are a few hundred while others can range from a few hundred thousand to millions of dollars. Every bequest is equally appreciated.



Do you know some of the symptoms of AL Amyloidosis?



www.amyloidosis.org

1-877-AMYLOID



Why make a BEQUEST?

Charitable bequests are an excellent way to pass on assets that may otherwise be substantially taxed. The donor's estate may be entitled to an estate tax charitable deduction for the full, fair market value of the bequest. Also, wills are fully revocable before death.

We encourage you to consult with your attorney, legal advisor or counsel when making decisions about your estate.

There are many creative, long-term giving opportunities and we would be happy to talk through the options with you. We are here to help every step of the way. **AF**



Expanding the Screening of Amyloidosis to Larger Populations

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Also, compared to cardiac magnetic resonance, bone scintigraphy can be applied to larger populations including patients with pacemakers and with renal insufficiency which are common in this population.

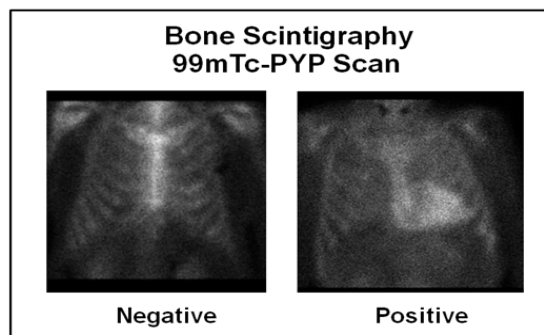
Moreover, compared to cardiac magnetic resonance the bone scintigraphy provides insights about the possible type of amyloidosis (transthyretin vs lightchain), facilitating the disease workup and consequent discussions with the patient.

Because of the positive experience with the algorithm using bone scintigraphy and the evaluation for monoclonal proteins, we are now expanding the screening for amyloidosis to larger populations.

We are making more diagnoses of amyloidosis in heart failure patients with preserved ejection fraction, patients with refractory atrial fibrillation, patients with aortic stenosis and older adults with left ventricular hypertrophy.

The screening algorithm combining bone scintigraphy with the evaluation for monoclonal proteins in blood and urine is a game changer for the amyloidosis field because it is simple to implement in institutions and it can be applied to larger populations.

The future will provide even further advances for screening and diagnosis.



**Bone Scintigraphy
99mTc-PYP Scan**

Negative

Positive

The hope is to increase the awareness and provide early diagnosis in order to improve the outcomes and quality of life of these patients. **AF**

References:

1. Julian D. Gillmore, MD; Mathew S. Maurer, MD; Rodney H. Falk, MD, et al. Nonbiopsy Diagnosis of Cardiac Transthyretin Amyloidosis. *Circulation*. 2016; 133:2404-2412.

Utah Amyloidosis Symposium for Physicians and Patients - June 2017



The Utah Amyloidosis Program in collaboration with the Amyloidosis Foundation will be hosting the **1st Utah Amyloidosis Symposium on Friday, June, 9, 2017.**

This event is open to patients and physicians and will review the latest advances in the amyloidosis field.

Keynote speakers will include Raymond Comenzo, MD from Tufts University, Daniel Lenihan, MD from Vanderbilt University and Charlotte Haffner from the Amyloidosis Foundation to lead the patient panel.

Topics include: Insights into molecular pathways,

Emergent disease modifying therapies, Multidisciplinary care, patient perspectives and more.

Join us for this exciting event in June. For more information use this link: <http://bit.ly/1lyPAfQ> or send an email to:

amyloidosis@hci.utah.edu. **AF**

www.amyloidosis.org

Spreading Amyloidosis Awareness on Rare Disease Day

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We received much interest from physicians, nurses, laboratory technicians, medical students and visitors with several people leaving with reading materials provided by the Amyloidosis Foundation. Most had never heard of amyloidosis, and others new very little about the disease, so there were abundant questions which we were able to address.

Among the interested individuals was a gentleman who had been diagnosed with multiple myeloma; he was quite knowledgeable about amyloidosis and was eager to help in our research if the opportunity were to arise. Additionally, we were fortunate to meet a lady who informed us that she had been reading on her own time about amyloidosis, and she plans to speak with her personal physician about the disease and some symptoms she has been experiencing—she took every piece of reading material we offered!



Dr. Emily Martin; Charlotte Haffner, member of the Amyloidosis Foundation Board of Directors; and Tina Richey were discussing Amyloidosis as part of Rare Disease Day at UT Medical Center.

Dr. Jonathan Wall, Dr. Stephen Kennel, and Sallie Macy, who have lead and conducted Amyloidosis research, also participated in the event.

We also had a physician stop by and ask specific questions about fat pad biopsies for Congo red staining which, as we know, is one of the first steps to take in detecting this disease. Each of those conversations alone would have made the event well worth our time and effort, but the experience was above and beyond what we could have anticipated. We gave away hundreds of items, including pins, wristbands and writing pens from the foundation as well as other items designed by our research team, in an attempt to raise awareness.

Many thanks to the Amyloidosis Foundation and Ms. Charlotte Haffner for helping to make Rare Disease Day a success at UTM. We are eager to participate again in 2018! **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!

