News and Stories - Spring 2019

FDA approves Vyndaqel® and Vyndamax™

On May 6, 2019, the U.S. Food and Drug Administration (FDA) approved both VYNDAQEL® (tafamidis meglumine) and VYNDAMAX™ (tafamidis) for the treatment of the cardiomyopathy of wild-type or hereditary transthyretin-CM) in adults to reduce cardiovascular mortality and cardiovascular-related hospitalization. VYNDAQEL and VYNDAMAX are two oral formulations of the first-in-class transthyretin stabilizer tafamidis, and the first and only medicines approved by the FDA to treat ATTR-CM.

Transthyretin amyloid cardiomyopathy is a rare, life-threatening disease characterized by the buildup of abnormal deposits of misfolded protein called amyloid in the heart and is defined by restrictive cardiomyopathy and progressive heart failure. Previously, there were no medicines approved to treat ATTR-CM; the only available options included symptom management, and, in rare cases, heart (or heart and liver) transplant. It is estimated that the prevalence of ATTR-CM is approximately 100,000 people in the U.S and only one to two percent of those patients are diagnosed today.

For more information, visit: www.vyndalink.com **AF**

Are you on Facebook?

We have a large community on Facebook with our account and our business page. We have found that we reach more people by sharing stories, articles, webinars, events and news. We try to share something new every day!

Last year, we were approved as an official charity through Facebook. This means that anyone who has an account can start a Facebook Fundraiser on our behalf. The funds raised are dispersed to us twice a month and Facebook doesn't charge fees on fundraisers for nonprofits.

Starting is easy. They'll help you get started and give you tips to reach your goal. **Share with friends.** Connect friends to your cause and gain supporters across Facebook. **Get donations.** People can donate to your fundraiser in just a few clicks without leaving Facebook. For more info, find us on Facebook! **AF**

AF Board Member integral in Tennessee legislation

In 2008, Charlotte was a healthy, active 55-year-old who loved the outdoors, especially taking care of her horses. Then, one day she started feeling different: fatigued, retaining fluid, shortness of breath.

The following months dragged on. Routine testing offered no explanation for her heart failure. Her condition deteriorated. "I sat on the hillside behind my house and prayed. I was dying, and I knew it," Charlotte says.

Then she was referred to cardiologist Joseph Fredi, MD. He had an idea what was wrong. Tests confirmed it. Charlotte has primary AL amyloidosis.

"I asked Dr. Fredi if it was fatal, and he held back for a minute, and he said, 'I'm afraid it could be,'" Charlotte remembers. "I said to him, 'Well, it won't be this time.'"

Eleven years later, Charlotte has undergone heart transplant and peripheral blood stem cell transplant. She helped found VAMP (Vanderbilt Amyloidosis Multidisciplinary Program); joined the Board of the Amyloidosis Foundation (AF), where she has "a platform to help other patients"; and became the first patient enrolled in a first-in-human, phase 1 clinical trial testing a new amyloid-binding peptide for detecting amyloid.

She also convinced Tennessee State Representative Glen Casada to sponsor a resolution that led to Tennessee becoming the first state to declare March Amyloidosis Awareness Month. **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
- Caregiver/Patient Binder
- Fundraising Toolkits

Follow Us

amyloidosis foundation

Stay connected for all the latest information on Amyloidosis:

Web: **www.amyloidosis.org**
Twitter: **@Amyloidosisfdn**
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Instagram: **@amyloidosisfoundation**

President's Corner

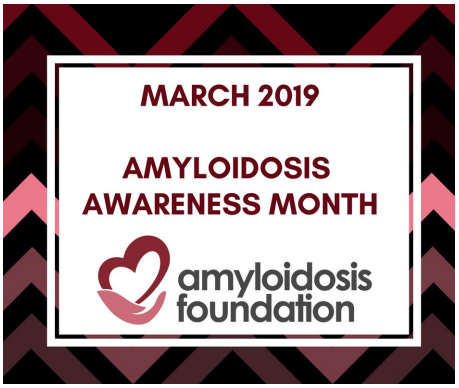
Mary E. O'Donnell

I have personally been a part of the amyloid world for over 17 years. Although we still need great strides and advances, the picture is completely different than in 2002. In the past year, 4 drugs have been approved for hATTR; 2 for hATTR polyneuropathy and 2 for hATTR cardiomyopathy and ATTR wild type. Trials are in progress for AL amyloidosis that show promise, and hopefully will progress to FDA approval.

Early diagnosis is still critical, so we continue to work at raising awareness of all types of systemic amyloidosis in the medical community. Your generosity not only supports innovative research but helps our awareness activities.

Mary

March was Amyloidosis Awareness Month.



We began in 2018/2019 by passing state resolutions to recognize Amyloidosis Awareness Month in Tennessee, Michigan, Indiana, Florida and Louisiana. We have advocates working on similar legislation in the following states for 2019: AK, CA, GA, KY, NE, NY, PA, UT, VA and WI. If you're interested in helping to pass this legislation in your state, please contact **kathi@amyloidosis.org** **AF**

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 N. Main Street, Ste. 2, Clarkston, MI 48346**

If you wish to receive an electronic version, please send us an email:

info@amyloidosis.org

Rare Disease Week on Capitol Hill

In February, one of our staff members traveled to Washington DC for Rare Disease Week on Capitol Hill. She participated in many activities and events advocating and giving a voice to amyloidosis patients, caregivers, family and friends. As you know, Washington DC is often gridlocked with partisan bickering. However, amyloidosis isn't a partisan issue, it doesn't discriminate. It affects both Republicans and Democrats alike.

The festivities began on Sunday evening with a documentary screening. The film, 'My Turn', was about a former professional hockey player, and his first-hand perspective on how his life has changed since his rare disease diagnosis. His wife was on a panel for a discussion after the film because her husband lost his battle 2 months prior. There were many questions from viewers regarding everything from those diagnosed telling their stories to navigating insurance.

Monday was a Legislative Conference. It was a full day packed with many speakers and breakout sessions. Some things

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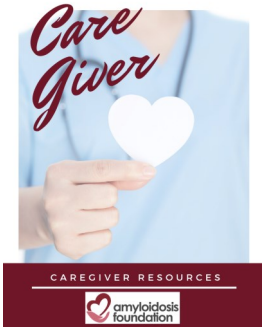
NEW Caregiver Binder and Resources!

The AF is pleased to announce that we have a new resource for caregivers and patients.

The binder was designed by the Amyloidosis Foundation staff, a team who strive to make caregiving 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.

Here's how it works: Keep documents in one location



Medical History Lab Results Appointments Questions Notes from the doctor

Information, business cards and handouts.

Take It, Share It, Use It

 Take it with you to doctor's appointments Share it with other caregivers and loved ones Use it to make caregiving easier and more effective

Suggestions From Other Caregivers Move pages and sections to fit your needs Use pockets in the 'Medications' section for information you receive from the pharmacy Use the calendar to keep track of appointments and when to order medications and medical supplies Use a plastic business card sleeve to hold business cards. You may also write notes on the front/back of the cards

Download yours today! If you don't have internet access, we can even mail you a copy! Contact us at info@amyloidosis.org **AF**

Rare Disease Week on Capitol Hill

(Continued from page 4)

covered were the Health Policy Forecast, Healthcare's challenging issues, how the new Congress will impact health policy in 2019, how to advocate effectively, and building effective partnerships. One breakout session was a deep dive into policy of NIH and FDA Appropriations. An investment is needed in rare disease biomedical research conducted at NIH and FDA, which ultimately leads to treatments and facilitates patient access to treatments.

Another session was 'Access to Treatment of Rare Diseases - A spotlight on Rare Cancers'. This was an excellent session as Multiple Myeloma was discussed. Treatments for MM have increased significantly, yet it still isn't curable.

On Tuesday, she proceeded to meetings with Michigan legislators. While there, Kathi met with 2 Senators (and/or Legislative Aides), and 2 House Representative Aides. The two main areas discussed were the Rare Disease Congressional Caucus that works to raise awareness of rare diseases, namely amyloidosis, and asking for their support for robust funding for the NIH and FDA for the annual discretionary funding in the Fiscal Year 2020. Many personal stories were shared, tears were shed, and our impact on Capitol Hill was felt. Tuesday evening ended with a meeting on 'Rare Diseases: Perspectives on Progress'. This meeting focused on advancements in rare disease and the current development landscape. The program provided discussions about the challenges and opportunities in rare disease research and the needs of the patient community.

Wednesday was a Rare Disease Congressional Caucus Briefing where many legislators and directors from NIH and FDA discussed how they are fighting rare disease, tools and techniques in diagnosing rare diseases and challenges for caregivers.

This is an area that the Amyloidosis Foundation is working on. We want to support the caregiver with ideas and resources. In the evening, there was a 'Rare Artist Reception' where artwork was displayed from the rare disease community. This was a wonderful event to view beautiful pieces while supporting a cause we all care so deeply about.

Thursday, February 28 was Rare Disease Day. Naturally, it was also 'Rare Disease Day at the National Institute of Health' (NIH). It featured interactive panel discussions on collective research models for rare diseases, patient registries, and rare cancer initiatives. A common theme was that "Rare diseases are not rare", considering there are over 7,000 rare diseases. 30 million people in the US are living with a rare disease, that's 1 in 10 Americans, or 10% of the US population. In addition, 95% of these diseases do not have a FDA approved drug treatment. We are trying to change that...all of the rare diseases together, including amyloidosis. **AF**

Utah Amyloidosis Symposium 2019

On May 17, one of our staff members attended the 2nd Annual Utah Amyloidosis Symposium at the University of Utah in Salt Lake City.

The event was sponsored by the Amyloidosis Foundation, University of Utah Healthcare, the Huntsman Cancer Institute, Amyloidosis Support Groups and Amyloidosis Research Consortium.

The objective of the Utah Amyloidosis Symposium was to develop a forum that will catalyze, advocate, and provide education that promotes change, development and improvement for the early diagnosis and management of amyloidosis.

Speakers included many top physicians and researchers in the field of amyloidosis. The first speaker was Giovanni Palladini, Acting Director, Amyloidosis Research and Treatment Center, Foundation "Istituto di Ricovero e Cura a Carattere Scientifico (IRCCS) Policlinico San Matteo" and Department of Molecular Medicine, University of Pavia, Italy. He spoke on median survival and mentioned that amyloidosis is the "great imitator". His slides included one that had some discouraging statistics that 32% of patients need to see 5 or more physicians to reach a diagnosis. All agreed that this needs to change.

Another point that he made was that AL should be searched for by physicians before the onset of symptoms. Once there is symptomatic organ involvement, it can portend a poor outcome.

Tibor Kovascsovics, Director, Hematology, Utah Amyloidosis Program, University of Utah Health/ Huntsman Cancer Institute, Salt Lake City, UT was the next speaker on the agenda. He spoke about novel agents for the treatment of AL amyloidosis. He mentioned that CAEL-101 has specifity of antibody binding for AL. He has seen a 97% cardiac response and a 50% renal response.

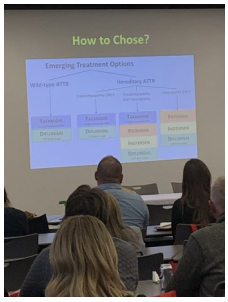
Vaishali Sanchorawala, Director, Autologous Stem Cell Transplant Program; Director, Amyloidosis Center, Boston University School of Medicine, Boston, MA spoke about Hematology, the role of stem cell transplantation (SCT), and Grand Rounds.

Following this session was a question/answer period with a multidisciplinary panel discussion on how to approach the treatment to patients with advanced (Stage IV) AL amyloidosis.

Session 2 started with Jo Abraham, Clinical Medical Director for the Division of Nephrology; Director, Nephrology Fellowship Program; Director, Nephrology, Utah Amyloidosis Program, University of Utah Health/ Huntsman Cancer Institute, Salt Lake City, UT.

Dr. Abraham spoke about kidney involvement with amyloidosis, including ALect2, a type of amyloidosis mainly associated with kidney damage.

Many experts agree that ALECT2 amyloidosis should be considered while looking for a diagnosis when a patient has evidence of renal (kidney) disease. A patient's symptoms may appear as renal failure or "nephrotic syndrome". Nephrotic syndrome is a group of symptoms that relate to kidney issues including: protein found in the urine, high cholesterol levels, low blood protein levels, and swelling.



Luke Gessel, Specialist, Gastroenterology, Utah Amyloidosis Program, University of Utah Health/ Huntsman

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Cancer Institute, Salt Lake City, UT was the next speaker. He spoke about gastrointestinal involvement. With amyloidosis, the small bowel is the most affected gastrointestinal organ.

The next speaker was Chakravarthy Reddy, Specialist, Pulmonology, Utah Amyloidosis Program, University of Utah Health/ Huntsman Cancer Institute, Salt Lake City, UT.

He spoke about the different ways that amyloidosis manifests as pulmonary issues. It was insightful to patients, doctors and researchers alike.

The speakers of session 3 started with Mathew Maurer, Medical director of The HCM Center New York-Presbyterian Hospital/ Columbia University Medical Center, New York, NY. Dr. Maurer is also on the Scientific Advisory Board for the AF.

He spoke about Cardiology Grand Rounds and the advances in TTR (transthyretin) protein stabilization and degradation of amyloid fibrils for ATTR amyloidosis. So much has changed in the last few years. There have been many advances.

Jose Nativi, Director, Cardiovascular Intensive Care Unit; Director, Cardiology, Utah Amyloidosis Program, University of Utah Health/ Huntsman Cancer Institute, Salt Lake City, UT, told about the survival rates between the different types of amyloidosis with cardiac manifestations. He mentioned that cardiac AL is a medical emergency. He also told about the non-invasive testing called Pyrophosphate scintigraphy (PYP scan).

Session 4 began with Kelsey Barrel, Specialist, Neurology, Utah Amyloidosis Program, University of Utah Health/ Huntsman Cancer Institute, Salt Lake City, UT.

Her main focus was the burden and management in amyloidosis neuropathy, which starts in the toes and moves to the feet, legs and hands.

Sami Khella, Chief, Department of Neurology, Penn Presbyterian Medical Center, University of Pennsylvania School of Medicine, Philadelphia, PA, spoke of the impact of gene silencers in neuropathy and quality of life with patients diagnosed with hereditary amyloidosis. He told about the scales used for a clinical practice, which focuses on weakness versus those used in clinical trials.

Katelyn Swade ended this session talking about the

role of genetic counseling in amyloidosis.

She is a Genetic Counselor, Utah Amyloidosis Program, University of Utah Health/ Huntsman Cancer Institute, Salt Lake City, UT.

Isabelle Lousada, CEO and President, Amyloidosis Research Consortium-Boston, MA started session 5 with a talk on quality of life in amyloidosis, followed by a patient panel discussion about patient referrals.

Each one had a different story, but there were many similarities. Most went to several physicians and specialists before being diagnosed. It was noted to the physicians in the audience that they need to look at symptoms in a multidisciplinary fashion.

Closing out the symposium was session 6, with a Multidisciplinary Panel Discussion about referral patterns for early diagnosis, access to therapy and research collaboration. It was a day full of learning for all of those in attendance. We are looking forward to next year's event.

AF

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