amyloidosis foundation

News and Stories - Summer 2016

Successful Run 4Tres in the O.C. Half Marathon

Terry Loughran and Kevin Hauri lost their good friend, Tres Heald, in 2015 after his battle with Amyloidosis.

On May 1, 2016, they ran with Eric Heald, Tres' brother, in the Orange County, CA Half Marathon to honor Tres and to raise money to support those still fighting the disease.

This amazing team raised over $10,700 and all proceeds have been donated to the Amyloidosis Foundation.

"It was awesome," Tres' wife Kelli Heald said of the weekend. "It's pretty exciting for us. In 2015, the Amyloidosis Foundation awarded a research grant to my husband's physician, Dr. Michael Rosenzweig from City of Hope, to continue amyloidosis research. It's thrilling for us to see that our donations will be going to support research." (Kelli was quoted in the *LA Times)*.

Thank you to everyone who donated to the team! ***AF***

(more photos on page 8)

2016 Pittsburgh Amyloidosis Research Benefit

The Amyloidosis Foundation is proud to announce the **2016 Amyloidosis Research Benefit** in **Pittsburgh, PA** on **Friday, October 28.** Our goal is to raise donations for the AF Research Grant Program.

Since 2005, the foundation has awarded over $1 million to young researchers pursuing a cure for amyloidosis. Dr. Darcy Tannehill, amyloidosis patient and Pittsburgh resident, is the Chairwoman for our fundraiser.

The event will be at the Montour Heights Country Club starting at 5:30pm. The evening will include strolling appetizers, dinner, live music, cash bar and a silent auction.

Proceeds from this event will allow us to continue our support for ground-breaking medical research.

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Amyloidosis Documentary—Coming in July

Thank you to Leslie Schumacher-McKee and AF Board Member Charlotte Haffner for sharing their stories in our upcoming amyloidosis documentary, which will be released in July 2016.

We appreciate their willingness to spread awareness and support the Amyloidosis Foundation. This video was produced by Katelyn Payne, who is majoring in Broadcast Journalism and will graduate this year from MTSU in Tennessee. Her mother has amyloidosis.

Katelyn interviewed patients and physicians at the 2015 AF Patient Day event, Amyloidosis: Raising Awareness, Improving Care, and Enhancing New Treatments, that was held in Nashville last October. There is a link to this trailer on our Facebook page and also on our website, **www.amyloidosis.org**. Here is the direct link as well: **https://vimeo.com/165433217**.

Please share this video with your family and friends, to show why new therapies are so very important to our community.

We look forward to watching this important film. ***AF***

2016 Annual Amyloidosis Foundation Golf Outing

Join us for the 2016 Amyloidosis Foundation Golf Outing on **Friday, September 30 at the Fountains Golf Club in Clarkston, MI.** We will play as a scramble so golfers of all skill levels are welcome. This is always a fun event and we look forward to your support.

Proceeds will be used to fund our amyloidosis research grant program.

Bring your friends and family! Visit our website today to register, donate or be a sponsor: **www.amyloidosis.org**.

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Web: **www.amyloidosis.org**
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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
- Accurate informational pamphlets
- New & comprehensive website with information for patients, caregivers and physicians
- Toll Free Number **1-877-AMYLOID**
- Listing of experienced physicians that specialize in amyloidosis, it's diagnosis and treatment. Email us anytime with questions: **info@amyloidosis.org**

President's Corner

Earlier this year we created the Amyloidosis Foundation Patient Advisory Council, to ensure we keep the needs of patients foremost in everything we do. We will share their ideas and progress in upcoming communications.

Please join us for the Pittsburgh Amyloidosis Research Benefit on October 28, it will be a night to celebrate those in our amyloidosis community while generating donations for the AF research grant program. We are grateful to Darcy Tannehill for spearheading this event and look forward to a wonderful evening.

We are very thankful for those who participated in or donated to the many fundraisers so far this year. It's touching to see such generous support in raising funds to find a cure for amyloidosis and spreading awareness at the same time.

We hope everyone enjoys the beautiful Summer weather with friends and family.

- Mary O'Donnell

The Prieber Open- Celebrating Chris Priebe

We invite you to golf on **August 13, 2016,** in memory of Chris Priebe and to support the Amyloidosis Foundation. This outing will be played at two courses, Fox Creek and Whispering Willows in Livonia, MI.

This special event will make it possible to continue offering important programs within the community to fight Amyloidosis. **Learn more and register to play with this link**: **http://bit.ly/1OPw1IE**. **Deadline is July 15**, we look forward to seeing you there!

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:

info@amyloidosis.org

Amyloidosis Foundation Support Group Meetings

The AF offers support group meetings in Northern California, Tennessee and Washington state.

AF Board Member Dena Heath started the N. CA group, which celebrated their 10 year anniversary in 2014. Their next meeting is July 23 in Walnut Creek.

Amyloidosis patient and AF Board Member, Charlotte Haffner, runs the quarterly meetings in Nashville and Knoxville. Her first group meeting at Vanderbilt University Medical Center was in August 2011. Their next meetings are in September.

In Seattle, Dena Fantle facilitates our group meetings, which typically are potluck lunches or dinners at members homes a few times a year.

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Charlotte says, "I think the real benefit is that you are surrounded by people who have gone through some or all of the things you have associated with this disease. You constantly learn from each other. Newly diagnosed patients gain hope they didn't have before attending."

"They see you can have a new 'normal life', your life may never be the same again, but it is your 'new' normal. If you keep a positive attitude you can still have a quality of life for many years."

Thank you ladies for your continued leadership to amyloidosis patients and families.

For future group meeting dates and locations, please visit our website: **http://www.amyloidosis.org/resources/**. ***AF***

Smart Patients—Amyloidosis Online Support Community

Smart Patients is an online peer-to-peer community where those affected by amyloidosis support and learn from each other. SP believes that patients and caregivers are the most underutilized resource in healthcare and they want the patient experience to be top priority. By providing a safe and private place to interact online, Smart Patients helps amyloidosis patients and families share information and resources. While there are many social networks and online communities, the custom built forums at Smart Patients offer a welcoming, warm and engaging experience for members of all kinds.

Conversations are arranged using tags to make it easy to find what you're looking for, and only searchable by other members. Sharing stories strengthens bonds.

Smart Patients

Helping people affected by amyloidosis discuss emotions, treatments, news, and more, can nurture lasting relationships.

Members say their initial fears are alleviated almost immediately upon connecting with a group who understands firsthand what they are going through.

At Smart Patients, "Peer support *is* medicine." We invite you to join the Smart Patients amyloidosis community by visiting **www.smartpatients.com/af**. ***AF***

Daughter Raises Donations for Amyloidosis Research

Addison Lacy, daughter of Adrienne & Josh Lacy (who has amyloidosis), had to start a business at her school. She is nine years old and just finished 3rd grade at Ann Brock Elementary in Burleson, Texas (a suburb of Fort Worth). Her business, making loom band bracelets and selling them for $2 each, was part of her gifted and talented program. The students had to research various charities, pick one to create a business for and then donate the proceeds.

Says Addison, "I chose the Amyloidosis Foundation because my daddy got sick when I was five and I wanted to help find the cure for Amyloidosis. My goal was $100 but I got over $450!"

Josh and Adrienne have two children, Addison (Addi) and a younger son Chet. Josh is a former college athlete who started to get sick in February 2012, when their children were only five and one. Josh was diagnosed on July 12, 2012 when he was 32 years old. He has AL Amyloidosis, which affects more than five of his organs. In August that year, Josh was in congestive heart failure and kidney failure, and was too sick for a stem cell transplant (SCT). He did eight months of chemotherapy and then had a SCT in May 2013 at the University of Texas-Southwestern in Dallas. Josh hasn't had any follow up treatment since, only monitoring by his physician. He no longer is in heart failure, his kidney numbers aren't great but are better than they were.

Last year, Josh spoke at the Amyloidosis Research Consortium (ARC) Patient Forum with the FDA in Silver Spring, MD on November 16. 2015. He bravely told his story, sharing details of his journey and the need for supporting new therapies that address patients' needs.

Josh says he can still be beat by a two year old in a foot race, but he and Adrienne continue to try to see all the good things life has to offer every day. ***AF***

Treating Systemic Amyloidosis *by Martha Skinner, MD*

Amyloidosis is a rare disease that affects approximately 3,000 persons per year in the United States. It was first discovered 150 years ago by the well known German pathologist, Dr. Rudolf Virchow. Although the disease has been recognized for many years, treatment has only been available for the past 15 years. And for some types of amyloidosis, treatment is not even yet available. You may ask, "Why has it taken so long to develop treatment?" The main reason is that amyloidosis is a very complicated disease. The systemic amyloidosis are actually several very different disease; each has a different cause and different disease pattern.

Systemic amyloidosis means that amyloid deposits occur in many parts of the body, as opposed to localized forms of amyloidosis, such as Alzheimer's disease where amyloid deposits are found only in the brain. They systemic form was recognized to have various disease patterns in the mid-1900s; some people became very ill and died shortly after, while others had an underlying inflammatory disease for many years before amyloidosis developed, and a third group had a familial pattern of disease. These main types became known as primary (occurring out of the blue), secondary (occurring after inflammation) and hereditary. Basic research studies began in the 1970s after Dr. Alan Cohen made the brilliant discovery by electron microscopy that amyloid deposits in tissues were comprised of fibrils. This was of key importance because it meant the fibrils could be isolated from tissue and identified biochemically. For the next several years, laborious research work on amyloid deposits identified most of the amyloid proteins. Abnormal antibody light chains were found to cause AL amyloidosis. A protein associated with inflammation comprised the fibrils in AA amyloidosis. And mutations in transthyretin (ATTR) were found in fibrils of the most common familial amyloidosis. Other proteins with mutations causing familial types included fibrinogen, apolipo-proteins A1 and A2, lysozyme, and gelsolin.

The next steps before treatment could be recommended followed in the 1980s. Clinical researchers matched patients' clinical findings with the protein type and looked for the mechanism by which amyloid diseases were turned on. They studied the natural history of each amyloid type, including all laboratory data, patterns of organ system involvement and likelihood of survival. AL amyloidosis was a bone marrow abnormality where an abnormal light chain antibody was being produced, traveled around in the blood, an then deposited in tissues causing serious damage. It was found to be the most common type of amyloidosis, occurring in about 85% of patients. AA amyloidosis was discovered to occur after inflammation of a severe nature. Hereditary amyloidoses were found to be genetic diseases that were inherited from parent to child. The abnormal protein was made in the liver, traveled in the blood, and deposited as fibrils in tissues. Laboratories developed ways to test for each disease type using clinical samples of biopsy tissues, blood, urine, and DNA donated by generous patients.

In the 1990s researchers could finally think about treatment. Swedish researchers pioneered the first with major treatment for ATTR amyloidosis, in 1991. They performed a liver transplant on a patient to removed the factory making the abnormal TTR protein and give a new factory. This was a giant step forward and became a treatment helpful to many patients around the world. It remains the only treatment for ATTR, although it is a major surgical procedure with some risk, and optimal benefit requires the patient to be early in the course of the disease. There is hope that a new treatment with a "smart" pill will work for this disease. A government sponsored clinical trial is enrolling patients to test a pill in many centers internationally that may work to stop the TTR protein from forming amyloid fibrils. It is hoped that the pill will be an alternative to liver transplant and appropriate for patients at all stages of disease.

High dose chemotherapy and stem cell transplantation (HDM/SCT) was pioneered for AL amyloidosis in the mid 1990s. ***(cont. on page 7)***

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HDM/SCT could be offered because technology had just been developed to collect stem cells from peripheral blood. The rationale was that a high dose of chemotherapy, given early in the disease, would kill the abnormal clone of none marrow cells making the light chain amyloid protein. Since chemotherapy killed other cells in the bone marrow too, stem cells were infused afterwards to make a new bone marrow. The success of this treatment was remarkable and patients with AL amyloidosis were getting better for the first time ever. HDM?SCT continues to be a successful major treatment. However, the treatment comes with a cost; it is aggressive and has risks and can only be given if the heart and other involvement are not too severe. Physicians are developing new oral chemotherapy treatment protocols using melphalan combined with dexamethasone that are showing success and offer alternatives to patients who are too ill for HDM/SCT or wish to choose an option that has less risk. At the same time pharmaceutical companies are developing immunomodulatory drugs and one newer compound, lenalidomide or Revlimid, is reasonably well-tolerated and particularly in combination with dexamethasone produces good responses and improvement in organ function. Another novel drug, the proteasome inhibitor bortezomib (Velcade) is also under investigation for AL amyloidosis.

For AA amyloidosis, the Neurochem Company (Montreal, Canada) has developed the first ever targeted inhibitor treatment with the drug, Kiacta. Patient participation in an international clinical trial has shown it effective and it awaits FDA approval (Dember, et al New Eng J Med, 2007).

Treatment for amyloidosis today first requires that the physician recognize the clinical amyloid syndromes and obtain appropriate biopsies to make the diagnosis. Then appropriate tests must be done to determine whether or not the patient has AL amyloidosis, the most common and most progressing form. For difficult cases and for identification of mutant proteins, amyloid referral centers provide specialized diagnosis techniques. Along with the major therapy as discussed above for the specific type, supportive treatments are important for patients with all types of amyloidosis. Many need treatment for the specific organ damage caused by the amyloid deposits. Patients with nephrotic syndrome may need diuretics and support stockings for edema. Patients with congestive heart failure due to amyloid in their heart need special diets and medication under the care of a cardiologist who understands the needs related to amyloid heart disease. ***(cont. on page 8)***

2016 Pittsburgh Amyloidosis Research Benefit

(cont. from page 1)

Join us on this special night as we raise funds for research and celebrate our amyloidosis community. Tickets are $175 and we have tables of eight for $1400. Event sponsorships are also available. Please find a link to purchase tickets on our website at www.amyloidosis.org.

We have a block of rooms reserved for this event at the **Sheraton Pittsburgh Airport Hotel**. The room rate is $118 per night, which is good for three days prior to the benefit and three days after. They will provide complimentary transportation to and from the event as well. Rooms at this rate will be available until September 30.

Call **412/262-2400** to reserve your room. **Please mention that you are attending the Amyloidosis Research Benefit on October 28**. We have a link to the hotel on our website as well, www.amyloidosis.org.

We look forward to seeing everyone this Fall in Pittsburgh! For more information, please contact us at: info@amyloidosis.org. ***AF***

Running 4Tres

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Treating Systemic Amyloidosis

(cont. from page 7)

Patients with autonomic neuropathy may need medication to support the blood pressure and gastrointestinal dysfunction. Nutritional supplementation may be important to maintain weight.

A century and a half after the discovery of amyloidosis, the outlook for patients has shifted to hopeful. Enormous credit for this advancement goes the federal, foundation and private grants that have supported the brilliant amyloid research teams.

With amazing and ever improving technical scientific advances, researchers have been able to develop hopeful treatments. In addition, very special thanks go to the dedicated patients who participated in clinical trials, often without any hope of benefit for themselves.

With the continued partnership of supporting foundations, dedicated researchers, and brave patients we can look forward to the future development of better treatments that are specifically targeted to the abnormal amyloid protein and lead to our goal of a cure. ***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!