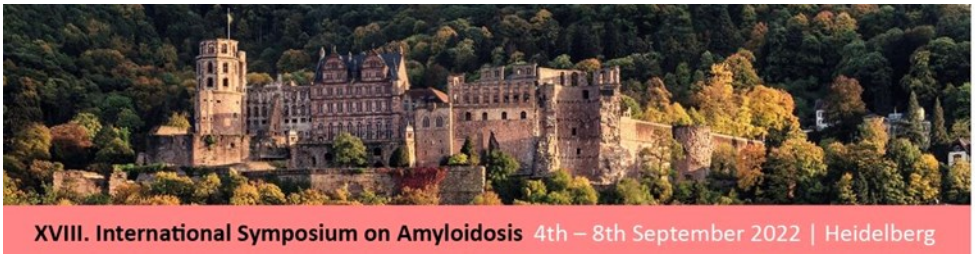
amyloidosis foundation

News and Stories - Fall 2022

"Quo vadis, amyloidosis?"



ISA | 2022

The motto for the XVIII International Symposium of Amyloidosis (ISA), held in the beautiful city center of Heidelberg Germany, was "*Quo vadis, amyloidosis?*". Literally this means, "Where are we going, amyloidosis?". But first, we need to appreciate where we have been and acknowledge the tremendous progress that has been made in amyloidosis. The amyloidosis space is exploding, evidenced by ISA XVIII having the largest attendance ever, with 1088 participants (842 in person and 246 virtual) from 50 countries. Over 4 days, there were 25 lectures, 71 oral presentations, 309 poster presentations and 8 satellite symposia. This rapidly growing interest in amyloidosis brings greater hope for continued progress on the path to "where we are going" and we hope to share some memorable moments of the meeting experience with you.

Awareness and earlier diagnosis continue to be a barrier to improving outcomes despite the development of therapeutics and growth in research. Treatments for AL and ATTR are improving survival, however the current treatments still fall short for the most advanced stages of the disease. The therapeutics available currently focus on decreasing the creation of abnormal protein. New research is focusing further upstream to understand why these proteins are misbehaving. This understanding will lead to earlier diagnoses, and a better understanding of who might be at risk. The use of cutting-edge imaging tests could yield a clearer (literally) understanding of organ involvement which in turn, could lead to less toxicity, less abnormal protein accumulation, and possibly the ability to clear existing amyloid out of the affected organs.

Research on normal amyloid or "functional" amyloid which naturally occurs in bacteria, continued development of animal models, biomarkers, and imaging will help lead us where we are going, *Quo vadis?. The goal has always been "cure" however, we must also embrace the concept of "operational cure" (living with amyloidosis) and continue to focus on ongoing medical management, disease monitoring (including minimal residual disease) and quality of life.* (Continued on page 4)

NEW Grocery Bag!

You have asked and we listened!

We have added a grocery bag to our list of products for sale!

They are $10/each or 3 for $25.

GET YOURS TODAY

https://amyloidosis.org/products/

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
- 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
- Financial Resources
- Caregiver/Patient Binder
- Fundraising Toolkits

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President's Corner

Mary E. O'Donnell

We lost an amyloidosis warrior recently. Charlotte Haffner, an AF Board Member, passed away September 23, 2022. Charlotte was diagnosed with AL Amyloidosis in 2008. She received a heart transplant followed by a stem cell transplant in 2009.

Charlotte dedicated her life to raising awareness of amyloidosis and supporting patients with their amyloid journeys. Her many accomplishments include but are not limited to;

- Establishing patient support group meeting in several locations
- Key to establishing March as Amyloidosis Awareness Month
- Provided one on one support to dozens of fellow amyloid patients

Charlotte will be greatly missed.

On a different note, as we approach the holiday season, please enjoy and stay safe.

Mary

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, (248) 922-9610**
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

"Quo vadis..." Cont.

(Continued from page 1)

A full review of the meeting is beyond the scope of this newsletter but we will try to provide some selected highlights. The conference kicked off with a keynote lecture by Cornelia Weyland, MD, on aging and the immune system which may indirectly provide insight into the role of the immune system in organ dysfunction of amyloidosis. The opening lectures followed:

- Giampoalo Merlini, MD, provided a comprehensive overview of AL amyloidosis and what we can learn from the disease as a model leading to a cure
- Daniel Otzen, PhD, introduced us to functional amyloid (naturally occurring amyloid) which is regulated and beneficial for the micro-organism and not harmful
- Julian Gillmore, MBBS, MD, PhD, FRCP, concluded the opening night with a presentation on gene targeting of ATTRv using editing with CRISPR-cas9 showing a 93% reduction in amyloid

Over the next 4 days, we were taken on a journey from basic research, clinical research to current diagnostic and therapeutic strategies for amyloidosis.

Selected Preclinical Research Topics

- Genetics and risk factors for the development of amyloidosis
- Post translation modification of light chains and amyloidosis
- Cardiac Toxicity of light chains in AL amyloidosis
- Aggregation-prone regions needed for fibril growth in amyloidosis
- AL mouse model using kappa knock-in and seeding
- Transgenic animal models of AL in C elegans (worms) and ATTR in mouse

Selected Clinical Research Topics

- Artificial Intelligence and machine learning
- Genetic risk factors of AL amyloidosis, prognosis and guiding treatment
- Minimal residual disease in AL amyloidosis
- Soluble suppression of tumorigenesis-2 serum biomarker of inflammation/fibrosis
- Neurofilament light chain serum biomarker for neuropathy
- Imaging of amyloidosis for diagnosis, organ involvement and treatment monitoring
- Medical management of organ involvement and quality of life

"Quo vadis..." Cont.

(Continued from page 4)

Spotlight on Imaging

Jon Wall, PhD, Professor and Assistant Dean for the UT Knoxville Research Division, presented 2 ongoing initiatives through their Amyloidosis and Theranostics program.

The first initiative is AT-01, which is an amyloid-reactive synthetic peptide radiotracer that can identify location and amount of amyloid throughout the body. This is done through a whole body PET/CT scan. At the present time, diagnosis is made primarily through congo red stained biopsies of suspicious tissues or organs, along with a myriad of tests. There are many downfalls to the current methods, such as a potential for infection, bleeding, injury, expense for patients, excessive time to diagnosis, and false negatives. Follow up biopsies are often indicated. In addition, biopsies do not provide information on all accumulated amyloid, just where the biopsy was performed. This non-invasive method to rule out or confirm amyloidosis in all organs has been very promising thus far. Surveillance and treatment response over time is more efficient and less invasive through the PET/CT scanning method. Our very own board member, Charlotte Haffner, was the first patient to enroll in this research initiative. Dr. Wall delivered an excellent presentation on the AT-01 peptide at ISA as well as our last Knoxville support group meeting.

The second UTK ongoing clinical trial sponsored by Attralus is the development and testing of AT-02 which consists of a PAR-peptide fused with a IgG1 antibody which binds to all types of amyloid, delivering the antibody to the accumulated amyloid which stimulates the immune system to identify, degrade, and remove the amyloid deposits. The immune system can be an excellent source of protection and self-healing when healthy or given the ammunition it needs to fight disease. The goal of this clinical trial is to prove the ability to prevent precursor proteins from being produced or limiting the capability of the proteins to form harmful amyloid deposits. The hope is to stabilize, slow progression, or even prevent amyloid precursor proteins from forming. AT-02 is the first of its kind in the amyloidosis space.

One of the main focuses of the meeting was awareness for earlier diagnosis which continues to be the best way to improve outcomes. It is imperative to continue to spread the message to providers, friends, family and communities. Some exciting initiatives of awareness leading to earlier diagnosis are Mackenzie's Mission and Global Bridges.

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What is Scintigraphy?

Scintigraphy is a test that uses a substance called a tracer and a scanning machine to create detailed images of your heart.

If the tracer finds amyloid proteins in the heart, it will bind to them and light up on the pictures of your heart like a "heat map with a hot spot."

Scintigraphy can help doctors see if you have cardiac amyloidosis. It can also help identify which type of cardiac amyloidosis you have. This will help determine which treatment is best for you.

What Should I Know About This Test?

People usually get this test at a clinic or hospital.

You can eat or drink normally before taking the test.

Wear comfortable clothes. Keep your jewelry and watch at home.

It will take about 2 to 3 hours to complete this test.

Is This Test Safe? Will It Hurt?

This test is safe.

People who have a fear of small, enclosed spaces may find the scanning machine uncomfortable. They may need medicine to help them relax.

This test involves an injection with a tracer, which has a very small amount of radioactivity. This is similar to the amount of radioactivity you would get with an x-ray. It is very unlikely to cause any problems.

What Should I Expect When Getting Tested?

After the IV is placed in your hand or arm, you will receive an injection of a substance called a tracer.

You will be asked to wait for up to 4 hours for the tracer to pass through your body. You will not be able to feel the tracer.

Next, you will be asked to lie down on a padded table. A machine will scan you to take images of your body and your heart.

During the scan, you will be asked at some points to raise your arms or to lay still. The technician will be able to see, hear, and speak with you at all times. The entire scan will take less than 1 hour.

What Results Might Mean I Have Cardiac Amyloidosis?

Not all forms of cardiac amyloidosis will show up on a scintigraphy test. If the image of your heart looks like a heat map with a "hot spot," you might have a type of cardiac amyloidosis called ATTR-CM.

What Comes Next?

Your doctor will review your test results and talk to you about them. Results will be different for each person. Here are some common examples of what your results might show:

The test results indicate you may have cardiac amyloidosis. If this happens, your doctor will want to run additional tests to find out what type of cardiac amyloidosis you have. The type of cardiac amyloidosis will determine the best treatment for you, and impact prognosis.

The test results indicate you may not have cardiac amyloidosis. If this happens, your doctor will talk to you about what the other medical condition is and what needs to be done to treat it. You may or may not need more tests.

It is unclear if you have a condition or not. This could mean your heart is healthy or it could mean that the test was unable to identify the problem. If this happens, you may need further testing.

"Quo vadis..." Cont.

(Continued on page 5)

The Amyloidosis Foundation was thanked by the program organizers for providing a large number of travel grants to young scientists. In addition, AF research grant funding was acknowledged during many oral presentations.

In the midst of and despite lingering unmet needs, the key take away from ISA XVIII is lots of progress and HOPE. The meeting concluded with a lot to look forward to in two years at ISA XIX to be held at Mayo clinic in Rochester, MN in 2024.

AF

Save The Date!

#GivingTuesday is a global day of philanthropy when the world comes together to give. We are teaming up with our amazing community with a goal to raise $20,000, which will go toward #AmyloidosisResearch. Will you join us? Be involved and make a difference!

Donations for #GivingTuesday can be made at:

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