News and Stories - Fall 2024

'Run For Your Life!' Recap

With enthusiasm and gratitude, the Amyloidosis Foundation is proud to celebrate another successful year of 'Run For Your Life!'. Thank you to the 331 runners, walkers and rollers that took part in our event! The participants walked, ran, biked and rolled at their leisure to help raise awareness of amyloidosis in their local community. This event raised over $10,500 for research, education and awareness. We greatly appreciate the wonderful support we have received!




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OCTOBER WEBINAR

Updates in AL amyloidosis: Fast Forward in 2024!

On October 17, 2024, Dr. Landau will discuss novel insights into the recognition and diagnosis of Light Chain (AL) Amyloidosis, and highlight strategies that are being studied to facilitate early diagnosis. She will share data that informs current management of newly diagnosed and relapsed patients and share investigative approaches that are currently being studied and hold great promise. Lastly, she will describe efforts to promote organ preservation as well as recovery and touch on organ replacement.

Register here: https://www.pathlms.com/amyloidosis/webinars/55024

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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:
https://bit.ly/4GivTue24

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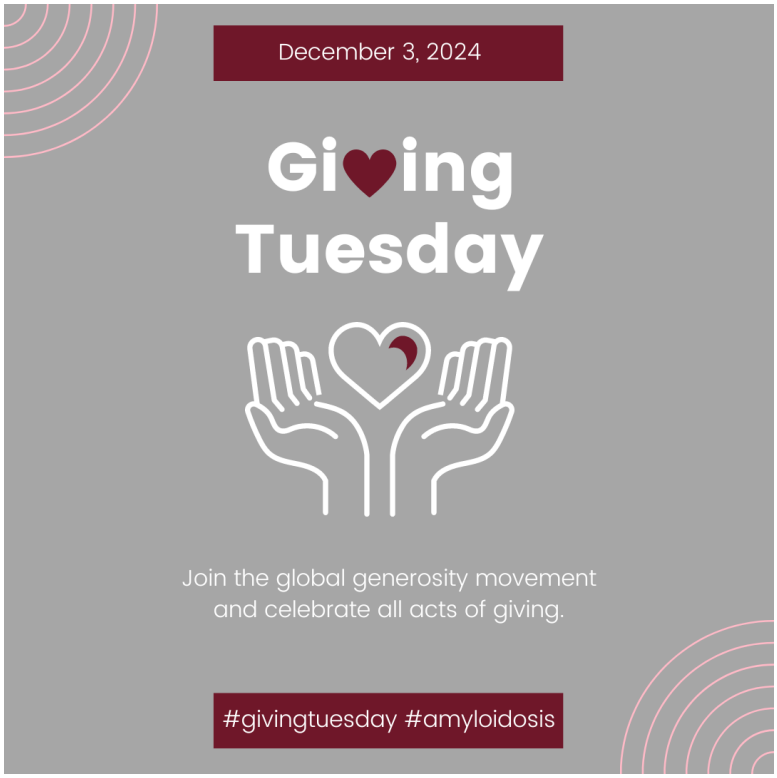


Patient Resources

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
- 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
- Webinars
- Caregiver/Patient Binder
- Fundraising Toolkits

Follow Us!

Stay connected for all the latest information on Amyloidosis:

Web: **www.amyloidosis.org**
Twitter: **@Amyloidosisfdn**
Facebook: **@amyloidosisfdn**
Instagram: **@amyloidosisfoundation**
YouTube: **@amyloidosisfoundation4994**

Awareness Gear

Show your support by shopping our collection of swag designed to raise awareness!

Logo T Shirt
$25.00

Zebra T Shirt
$25.00

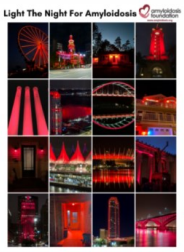
Gray Logo T Shirt
$15.00

Wristband
$5.00

Ribbon Awareness Pin
$8.00

Notecards
$15.00

Red Light Bulb
$6.00



"Light The Night For Amyloidosis" Poster
$15.00

Gray Run Shirt
$25.00

Maroon Run Shirt
$25.00

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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, (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

The International Symposium on Amyloidosis (ISA) 2024

The International Symposium on Amyloidosis (ISA) 2024 was held at the Mayo Clinic in Rochester, MN May 26-30, 2024. Every two years, scientists and clinicians come together and present the latest information and research impacting patients living with all forms of amyloidosis. The interest in amyloidosis and the level of participation in this biannual conference continues to expand with 1120 participants from 52 countries attending (some virtually) this year's conference. 571 abstracts were presented, which is beyond the scope of this newsletter review and many of the topics discussed were also listed in the newsletter review of the 2022 ISA meeting in Heidelberg (see AF website). We will do our best to provide some of this year's highlights and continued hope for the future.

The meeting kicked off with awards. The Merlini Award is presented to a recipient for lifelong achievement for excellence in research. This year Maria Joao M. Saraiva received the award for her work on polyneuropathy in familial amyloidosis. This was followed by a presentation of the inaugural Skinner Award, which is named in honor of Dr. Martha Skinner. The award was established with Dr. Skinner's generous support and recognizes women scientists who have made outstanding contributions to the field of amyloidosis. Dr. Gunilla Westermark from Uppsala University, Sweden, and Dr. Laura Obici from the Rare Diseases Unit and Amyloidosis Research and Treatment Center in Pavia, Italy were co-recipients of this award. On this night, the Amyloidosis Foundation was also recognized for its continued travel support of many young investigators allowing them the opportunity to present their work and participate in the meeting.

Diagnosis has continued to be a major area of concern and with the development of promising new treatments, more and more patients are being diagnosed. However, there is still more work to do to make providers aware of the diagnosis. Despite all the advances, earlier diagnosis continues to be the most critical element to improving prognosis by catching the disease prior to irreversible organ damage. Acknowledging this, diagnostic tests to detect amyloidogenic proteins in blood or urine and imaging agents to detect deposition in organs before organ symptoms (subclinical) were presented in several different abstracts. Artificial Intelligence (AI) is on the rise everywhere and work is still needed to see how AI will hopefully impact earlier diagnosis.

Amyloid depleting agents, for all forms of amyloid, to remove amyloid from organs continue to be a major focus of research. These drugs are at various stages of research and are not yet available outside of clinical trials. If we improve earlier diagnosis, these agents will still be needed to help reduce chronic organ dysfunction and symptoms, requiring continued medical management of involved organs to help improve quality of life for all, particularly those achieving durable remissions.

Quality of life and symptom management, including medical management of involved organs continue to be discussed at the conference and many abstracts were presented on that topic.

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The International Symposium on Amyloidosis (ISA) 2024

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Agents that can improve heart failure, like spironolactone, eplerenone, or SGPL2 inhibitors (traditionally used in diabetes) were also discussed. Other medications such as beta-blockers, have been understood to be contraindicated in amyloidosis, may have a role in some patients if managed by experts in amyloidosis.

Standard initial treatments for most patients diagnosed with ATTR-CM, ATTR-PN, and AL is very exciting but raises some questions about initial staging systems. Do they remain valid in the modern era, and should other biomarkers be considered? Abstracts on these topics were presented.

For AL, careful timing and delaying the use of auto stem cell transplant (autoSCT) appears safe, and the role of SCT is being redefined to determine which patients will benefit the most. The use of second-line chemotherapy, particularly venetoclax, which targets 11;14 translocation (found in many patients with AL), may require further discussions however, since autoSCT has become safer when performed at centers of excellence, we will continue to learn more from future studies. Other second or relapse therapies such as bispecific antibodies and CART were also presented along with studies to clarify when to initiate therapy and better define or detect recurrence.

For ATTR, the role of dual agents was presented and continues to be studied. Imaging to help detect response, is still needed in addition to improvements in technetium scans after treatment did not correlate with response. Data has emerged that treatment does alter the ability of the scan to detect and potentially diagnose ATTR-CM. Several studies were presented. Tafamidis has been the mainstay for ATTR-CM, but acromadis, vutrisiran, eplontersen, and other agents are in the wings. The role of organ transplant will also continue to evolve as more therapies become available to prevent progression after transplantation.

The field of amyloidosis continues to broaden, and areas of unmet needs are continuing to sprout, many of these need more time and research to validate. We also need to continue to push for more widespread awareness and close the gaps in health inequities in both diagnosis and treatment. As this conference demonstrated, amyloidosis research and understanding is moving rapidly and new breakthroughs are occurring in quick succession. We look forward to continued efforts: to make the diagnosis earlier before symptomatic organ involvement; better imaging agents and biomarkers for assessing responses to treatment and prognosis; improving medical management of organ dysfunction and symptoms addressing QOL; advancing therapeutics or combinations for reducing amyloid organ deposition or reversal; elucidating the pathophysiology and role of AI to impact all the above. Patients and physicians should have great optimism and hope for the future.

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Welcome Our NEW Board Member!

We wanted to be the first to congratulate Erskine "Kim" White, Ed. D., on his election to our board! Kim completed his Doctorate at Tennessee State University, and had a 27-year career in the classroom as a professor at the University of Nashville Tennessee, where he was also a contributing member of the USN Committee on Diversity and Community Life.

He has been an integral part of our support community, speaking at support group meetings where he has shared his input from a patient's perspective. We are looking forward to working with him and we are so thrilled to have him join us!

Read his letter to the community:

"Even a quick look at my resume will tell you I have a rather unconventional work history. However, a second look will reveal a singular, consistent purpose throughout all my various jobs and careers, one which I now hope to continue in my "retirement" years. Simply put: my primary purpose during all my working years has been to do good for others— for everyone to be sure but particularly those who are vulnerable, marginalized, and overlooked.

First, this meant trying to help organize poor people so they can win some measure of justice for themselves in this world. Next, it meant pastoring declining churches, helping them grow, and of course, helping their members navigate and find greater purpose in the various joys and trials we all must experience in life.

Eventually, this led me to a 27-year career in the classroom, where I instantly realized that without planning it, everything I had learned in my prior work settings had prepared me for effective teaching. I was able to teach students some wisdom about the world they live in and how it came to be. And I strove to teach the "whole student", not just the developing, searching young person who needs exams and grades. I would still be in the classroom were it not for the limitations imposed upon me by my disease.

Now I am retired, but I am not done. After struggling a few years to accept, adapt to, and embrace my new reality, I have come to realize that amyloidosis, with all its evolving infirmities and disabilities,

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Board Member

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is actually an opportunity to continue trying to do good for others.

This is a disease about which more attention and education are sorely needed. People who have it (and their caregivers) are best suited to help each other move forward, playing the tough hand they have been dealt. They can also best help medical educators and practitioners learn to acknowledge the "whole patient" that are treating, not just the physiologic symptoms she or he presents, for ultimately, it is the whole person who must live well with a currently incurable, degenerative disease which has a terminal diagnosis.
I know I do not have all the answers on how to do this newest kind of good for others, but I am someone who never stops learning and I know the work involved is important." **AF**

Positive HELIOS-B Phase 3 Study of Vutrisiran

Alnylam announced detailed results from the HELIOS-B Phase 3 study of vutrisiran, an investigational RNAi therapeutic in development for the treatment of ATTR amyloidosis with cardiomyopathy (ATTR-CM). The data was presented at the European Society of Cardiology (ESC) Congress 2024, which took place August 30-September 2 in London, United Kingdom. Results from the HELIOS-B study were also simultaneously published in The New England Journal of Medicine.

As previously reported, the HELIOS-B study met all 10 of its primary and secondary endpoints, across both the overall and monotherapy populations, with statistical significance. In the study, treatment with vutrisiran substantially reduced the risk of death and cardiovascular events relative to placebo.

Read the press release here:

https://investors.alnylam.com/press-release?id=28411 **AF**

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