amyloidosis foundation

News and Stories - Spring 2025

'Light the Night' Recap

The Amyloidosis Foundation launched the 'Light the Night for Amyloidosis' initiative in 2020 to align with 'Amyloidosis Awareness Month' in March. Each year, we invite communities to help shine a light on amyloidosis. For patients and their families, this campaign offers hope instead of helplessness, connection instead of isolation, and ongoing support through life-saving research. By raising awareness together, we help bring more people out of the darkness.

This year's campaign was a tremendous success, with illuminations across the globe. Participating countries included the United States, Canada, and the United Kingdom. Many returning participants joined new ones, and we hope these new advocates will continue to light the way for amyloidosis awareness in the years ahead. As a united community, we gather to celebrate, honor, and remember those impacted by amyloidosis.

Our mission is simple: to provide light and hope to everyone affected by amyloidosis. With greater awareness comes greater knowledge, leading to earlier diagnoses and improved outcomes.

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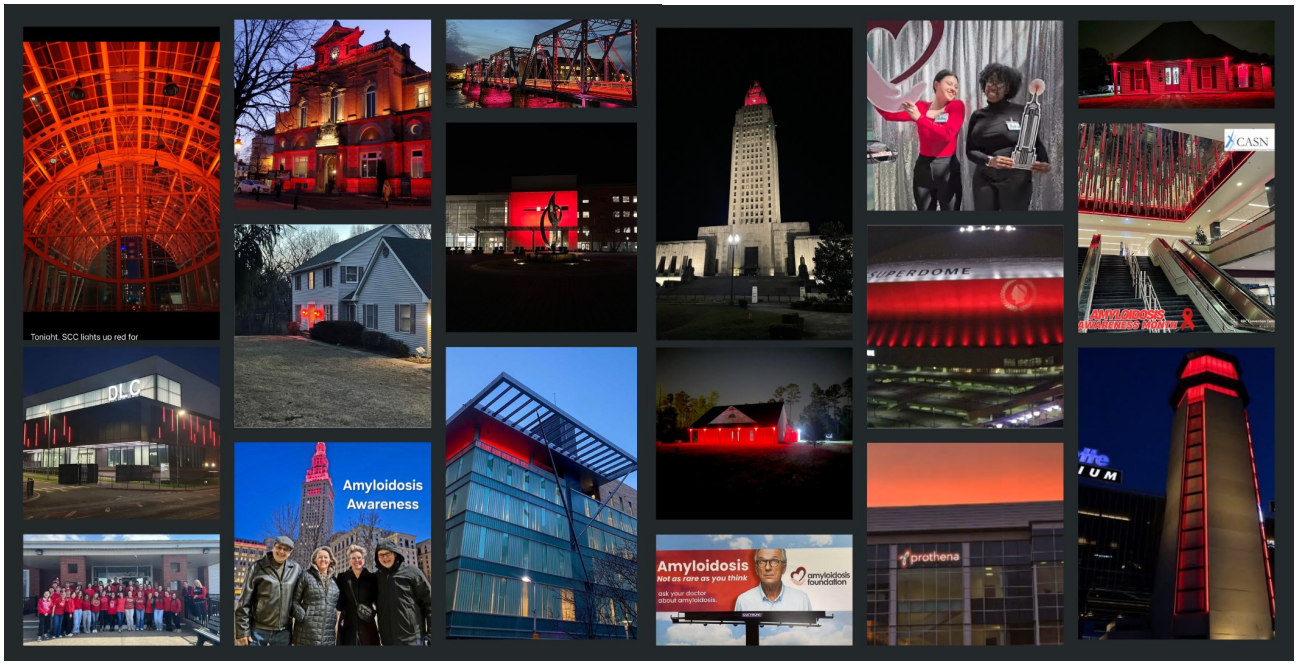

Education • Awareness • Support • Research

www.amyloidosis.org

Wednesday Webinars

Webinar Sessions are on Wednesdays from 6:00 PM - 6:45 PM Eastern Time Zone

Join us for our LIVE webinars on all things about amyloidosis!

We've teamed up with One Amyloidosis Voice and Somebody To Talk To (STTT) to bring you relevant amyloidosis content!

*These sessions cannot be recorded due to patient involvement and HIPAA laws.

To Register: **https://bit.ly/47t5aMd**

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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!

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Stay connected for all the latest information on Amyloidosis:

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

SAVE THE DATE!!!

Mark your calendars for the Run for Your Life Run-Walk-Roll-Bike! Invite your family and friends to sign up and join the fun!

This annual (and virtual) event allows you to participate at your convenience, all while helping raise awareness for amyloidosis.

You can run, walk, bike, or roll anytime between May 1, 2025, and July 31, 2025. *Registration includes a newly designed T-shirt!* **Registration is OPEN!**

Scan the QR code or register here:
https://bit.ly/2025Run

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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

Alnylam Announces FDA Approval of AMVUTTRA®

Mar 20, 2025

– Novel Mechanism of Action Delivers Rapid Knockdown of Transthyretin, Addressing the Disease at its Source –
– Proven Consistency of Effect on Cardiovascular Outcomes, Function, and Quality of Life in ATTR-CM Population Representative of Today's Patients –
– Only Therapeutic Approved in the U.S. to Address Both Cardiomyopathy and Polyneuropathy Manifestations of ATTR Amyloidosis –
– Alnylam Offers Multiple Programs to Support Broad and Seamless Patient Access; Majority of Patients Expected to Pay $0 in Out-of-Pocket Costs for AMVUTTRA –

Alnylam Pharmaceuticals, Inc. (Nasdaq: ALNY), the leading RNAi therapeutics company, announced the U.S. Food and Drug Administration (FDA) approval of the supplemental New Drug Application (sNDA) for its RNAi therapeutic, AMVUTTRA® (vutrisiran), for the treatment of the cardiomyopathy of wild-type or hereditary transthyretin-mediated amyloidosis (ATTR-CM) in adults to reduce cardiovascular mortality, cardiovascular hospitalizations and urgent heart failure visits. The approval expands the indication for AMVUTTRA, which now becomes the first and only therapeutic approved by the FDA for the treatment of ATTR-CM and the polyneuropathy of hereditary transthyretin-mediated amyloidosis (hATTR-PN) in adults.

"The FDA approval of AMVUTTRA for ATTR-CM marks a pivotal advancement for patients, providing a new and clinically differentiated treatment option that has been shown to improve outcomes, including cardiovascular mortality, and reduce progression for those living with this devastating disease," said Yvonne Greenstreet, MBChB, Chief Executive Officer of Alnylam. "I would like to extend my deepest gratitude to the patients who participated in our clinical trials, their families and caregivers, the clinical researchers, regulators, and my colleagues at Alnylam who made this approval possible. Today represents a significant milestone in our nearly twenty years of partnership with the ATTR amyloidosis community, but we are not stopping here. We will continue to innovate for patients with ATTR amyloidosis so they can live longer, better, healthier lives."

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20250319752041/en/ **AF**

Intellia Therapeutics Announces FDA Regenerative Medicine Advanced Therapy (RMAT) Designation Granted to Nexiguran Ziclumeran (nex-z) for the Treatment of Transthyretin (ATTR) Amyloidosis with Cardiomyopathy

March 26, 2025

Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading clinical-stage gene editing company focused on revolutionizing medicine with CRISPR-based therapies, today announced that the U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to nexiguran ziclumeran (nex-z, also known as NTLA-2001) for the treatment of transthyretin (ATTR) amyloidosis with cardiomyopathy (ATTR-CM).

"This is a meaningful step forward in our efforts to accelerate the development of nex-z to bring this potentially transformative treatment to patients as quickly as possible," said Intellia President and Chief Executive Officer John Leonard, M.D. "In both cardiomyopathy and polyneuropathy, nex-z has been shown, after a single dose, to lead to unprecedented rapid, durable and consistent reductions in serum TTR, which positively impacts markers of disease progression."

The RMAT designation was established under the 21st Century Cures Act to expedite the development and review of promising therapeutic candidates, including genetic therapies, that are intended to treat, modify, reverse or cure a serious or life-threatening disease. RMAT designation includes benefits, such as early interactions with the FDA, including discussions on surrogate or intermediate endpoints that could potentially support accelerated approval and satisfy post-approval requirements, and potential priority review of a product's biologics license application (BLA).

Nex-z has been granted Regenerative Medicine Advanced Therapy designations by the U.S. FDA for both cardiomyopathy and polyneuropathy. Nex-z has also been granted Orphan Drug Designation by the U.S. FDA and European Commission.
View the full press release here: https://ir.intelliatx.com/press-releases **AF**

2025 Grant Awardees

We are proud to feature our annual grant recipients, whose research targets the challenges in the field of amyloidosis.

Michael S. Hughes, MD
Preclinical Efficacy and Activity of the 11-1F4 CAR Macrophage in AL Amyloidosis

Amyloidosis Foundation Research Grant, 2025
Columbia University New York, NY

Emre Karayol, MD
Identification of Novel Therapeutic Targets within the Proteostasis Network in AL Amyloidosis - Follow Up Grant Award

Amyloidosis Foundation Research Grant, 2025
Brigham and Womens Hospital, Boston, MA

Taxiarchia Kourelis, MD
Development and validation of a human organoid model for renal AL Amyloidosis

Amyloidosis Foundation Research Grant, 2025
Mayo Clinic, Rochester, NY

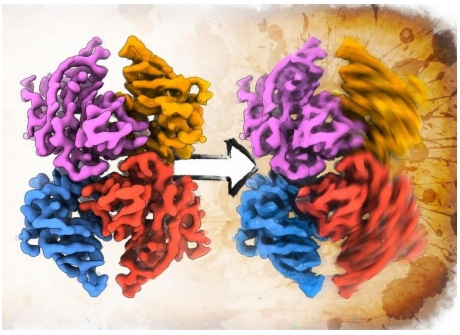
Advocating during Rare Disease Week

In March, we united in Washington DC to raise awareness and advocate for progress for the millions of individuals and families impacted by rare diseases. It was a moment to amplify our voices, share stories of resilience, and remind our leaders that amyloidosis patients matter.

We highlighted policy priorities for legislators. Every voice plays a crucial role in the fight for improved access to treatments, increased research funding, and stronger support for those who are too often forgotten.

Did you know?

These insights could drive the development of new drugs to treat transthyretin amyloidosis, a progressive and fatal disease.



Transthyretin, a small but crucial protein, plays a vital role in transporting hormones through the blood and spinal fluid. However, when it misfolds after secretion, it can lead to serious health problems. Misfolded transthyretin forms toxic clumps in the heart and along nerves, causing transthyretin amyloidosis (ATTR)—a progressive and often fatal disease. ATTR affects up to 25% of men over 80, leading to symptoms such as shortness of breath, dizziness, and numbness or tingling in the extremities.

In a breakthrough study, researchers at Scripps Research have revealed new structural insights into transthyretin. Their findings, published in Nature Structural & Molecular Biology, show how the protein's three-dimensional asymmetry may contribute to its instability. This discovery could pave the way for new drug treatments to combat ATTR.

"We've unveiled a molecular complexity that has been hidden from researchers for decades, which enables us to design better medicines to stabilize transthyretin," says co-senior author Gabriel Lander, PhD, professor at Scripps Research.

"The new structures reveal differences in two thyroid hormone binding sites previously thought to be identical, and help explain why a drug binding to one site changes the ability of drugs to bind the opposing site," adds Jeffery Kelly, PhD, the Lita Annenberg Hazen Professor of Chemistry at Scripps Research and a co-senior author of the study. **AF**

WAINZUA (eplontersen) approved in the EU

for the treatment of hereditary transthyretin-mediated amyloidosis in adults with stage 1 or stage 2 polyneuropathy

– Second major approval for WAINZUA, which is marketed in the U.S. as WAINUA™ –

– EU approval based on NEURO-TTRansform Phase 3 results showing WAINZUA demonstrated consistent and sustained benefit improving neuropathy impairment and quality of life versus placebo –

Read more here: **https://ir.ionis.com/news-releases/** **AF**

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