# News and Stories - Winter 2025

## *Giving Tuesday Recap*

Thank you so much for all of the love and support. We set off with lofty goals of raising $20,000. Although we did not meet our goal this year, we came close! Together, with your help, we were able to raise $17,841.50 in just 24 hours. Some donated directly on our link, some sent in a donation, and others started a Facebook fundraiser specifically for #GivingTuesday. We thank you and are humbled for allowing us to make a difference!


**AF**

## *SAVE THE DATE!*

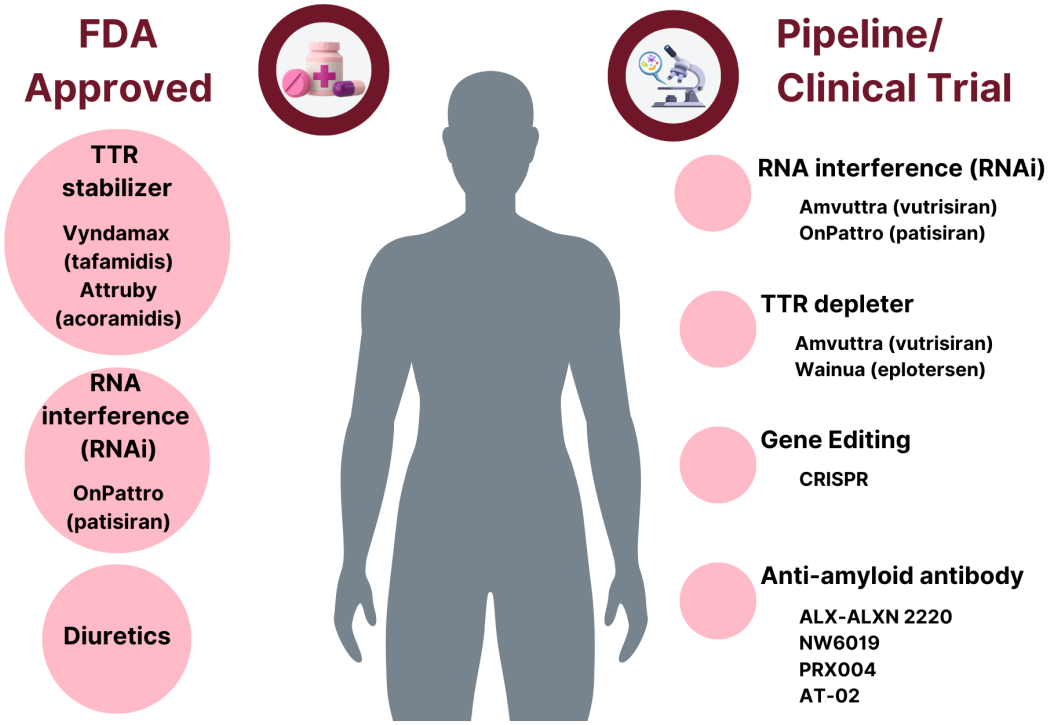
"Light The Night For Amyloidosis" is our annual amyloidosis awareness campaign. It was created to draw attention to symptoms, diagnosis, treatment, and hopefully someday, a cure!

Since 'Amyloidosis Awareness Month' also occurs in March, we are asking everyone to light their entryway, home or business with red bulbs for the month of March.

We have also contacted sites across the globe to do the same. Last year we had over 100 sites in 5 countries light for our cause! We have red bulbs available for purchase on our website at: **https://bit.ly/redbulb**

**AF**

# ATTRwt, ATTRv cardiac treatment & development of additional therapies




## 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!

amyloidosis foundation

Stay connected for all the latest information on Amyloidosis:

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

# *Missed the deadline? Donate today!*

Did you miss the 2024 charitable donation deadline? Donate today to be sure not to miss it for 2025!

**Tips for deducting charitable contributions:**

There are a lot of nuances when it comes to filing taxes, so here are a few things to keep in mind when deducting your donations:

- Ensure that you are donating to a qualified charitable organization.
- You must file Form 1040 and itemize deductions on Schedule A.
- If you receive any benefit from your donation, you can only deduct the difference between the donation and the value of the goods or services you received.
- When deducting a donation, get a receipt with the name of the organization and the date and amount of the contribution (We will send you a letter/receipt within 2 weeks or you can choose an electronic version right away).
- If you're donating property valued at $5,000 or more, you must get a qualified appraisal.

**Donate today HERE: https://www.amyloidosis.org (Donate button in upper right corner).**

**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, (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**

# Attruby™ (acoramidis), a Near Complete TTR Stabilizer (≥90%), approved by FDA to Reduce Cardiovascular Death and Cardiovascular-related Hospitalization in ATTR-CM Patients

– Attruby is the first and only approved product with a label specifying **near-complete stabilization of TTR**. Attruby has been shown to preserve the native function of TTR as a transport protein of thyroxine and vitamin A and to demonstrate benefit on cardiovascular outcomes
– Attruby demonstrated the most rapid benefit seen in any Phase 3 study of ATTR-CM to date:

- In as few as 3 months, the time to first event (all-cause mortality (ACM) or cardiovascular-related hospitalizations (CVH)) durably separated relative to placebo
- A 42% reduction in composite ACM and recurrent CVH events relative to placebo at Month 30
- A 50% reduction in the cumulative frequency of CVH events relative to placebo at Month 30

– To honor the courage of our U.S. clinical trial participants, BridgeBio will provide these patients Attruby free for life
– To learn about our extensive suite of programs to provide access to Attruby call 1-888-55-BRIDGE (1-888-552-7434)
– With this approval, BridgeBio will receive a $500 million payment under our royalty funding agreement

PALO ALTO, Calif., Nov. 22, 2024 (GLOBE NEWSWIRE) — BridgeBio Pharma, Inc. (Nasdaq: BBIO) (BridgeBio or the Company), a new type of biopharmaceutical company focused on genetic diseases, today announced that the U.S. Food and Drug Administration (FDA) approved Attruby™ (acoramidis), an orally-administered near-complete (≥90%) stabilizer of Transthyretin (TTR) for the treatment of adults with ATTR-CM to reduce cardiovascular death and cardiovascular-related hospitalization. The FDA approval is based on positive results seen in the ATTRibute-CM Phase 3 study, where Attruby significantly reduced death and cardiovascular-related hospitalization, and improved quality of life.

Attruby is the first and only approved product with a label specifying near-complete stabilization of TTR. Attruby was designed to mimic a naturally occurring "rescue mutation" of the TTR gene (T119M) that targets the root cause of ATTR-CM, destabilization of the native TTR tetramer. (Continued on page 5)

# Attruby™, continued

(Continued from page 4)

The ATTRibute-CM Phase 3 study enrolled 632 participants with symptomatic ATTR-CM, associated with either wild-type or variant TTR. Participants were randomized 2:1 to receive Attruby or placebo for 30 months. As published in The New England Journal of Medicine, the trial successfully met its primary endpoint of a 4-component composite endpoint of ACM, CVH, N-terminal prohormone of brain natriuretic peptide (NT-proBNP), and 6-minute walk distance with a Win Ratio of 1.8 ($p<0.0001$). Attruby demonstrated a statistically significant treatment effect at 30 months on the Kansas City Cardiomyopathy Questionnaire and 6-minute walk test. Additionally, the increase in NT-proBNP on treatment was about half that of placebo.

"Transthyretin cardiac amyloidosis is a progressive disease with a poor prognosis when left untreated. Having a new first line treatment option which provides excellent TTR stabilization and improves outcomes in this disease gives patients more options," said Martha Grogan, M.D., of the Mayo Clinic. "Encouraging data suggests Attruby reduces all-cause mortality and cardiovascular hospitalization as early as three months after initiation of therapy. With continued advances in therapy, this previously fatal disease is becoming a manageable chronic cardiovascular condition."

BridgeBio offers a patient support services program, ForgingBridges™, for people in the U.S. prescribed Attruby and their families to receive help accessing this new therapy. ForgingBridges includes insurance resources, financial assistance options and a dedicated support team to assist in the treatment journey. More information about BridgeBio's patient support services program is available on ForgingBridges.com or by calling 1-888-55-BRIDGE (1-888-552-7434).

"With the landmark approval of Attruby, we gain the ability to serve patients with ATTR-CM. I'm grateful to each trial participant, their families, and the physicians, scientists and our team at BridgeBio who made this possible," said Neil Kumar, Ph.D., founder and CEO. "Our journey is not over as we look to pursue approvals globally, next in Europe, Japan, and Brazil, and to continue exploring the full potential of this treatment. I am thrilled to extend our mission of 'putting patients first' with this third FDA approval in less than 10 years."
, whether as a result of new information, future events or otherwise.

To read the press release in its entirety, visit: **https://bit.ly/attruby**

**AF**

# Rare Disease Week

One of our staff members will be participating in Rare Disease Week at our nations Capitol in February. This multi-day event, hosted by the Rare Disease Legislative Advocates (a program of the EveryLife Foundation for Rare Diseases), brings together rare disease advocates from across the country to make their voices heard by their Members of Congress.

Rare Disease Week is the opportunity for the rare disease stakeholders to come together in Washington D.C. Rare Disease Week consists of many events including a Rare Disease Congressional Caucus Briefing, Legislative Conference, meetings with Members of Congress, and more. It is a great opportunity to educate Members of Congress on the issues that are most important to the amyloidosis community. This year marks the 9th year that Amyloidosis Foundation has participated!


**AF**

# February Nashville Support Group Meeting

February 1, 2025 at11:00 am
Chateau West
3408 West End Ave.
Nashville, TN 37203
Speakers will be VAMP Director; Dr. Muhamed Baljevic and patients.
There will also be ASH (American Society of Hematology) updates and more!
There is no cost (meal included), just bring yourself and a guest or two so we can continue to spread the word. Knowledge is power. Please **RSVP** as soon as possible to: Adrienne Molteni at Adriennemolteni@gmail.com or 615-497-1770.

**AF**

# Did you know?

Did you know the Amyloidosis Foundation has it's very own YouTube channel? Here you can also find links to our webinars!

Go to:
**https://www.youtube.com/@amyloidosisfoundation** or www.youtube.com and then type in 'Amyloidosis Foundation' in the search field and click "subscribe".
Please share with your friends and family to educate them on this disease and create awareness.

**AF**

# Rare Disease Day 2025


***February 28, 2025*** is Rare Disease Day, which occurs on the last day of February each year.

The main objective of Rare Disease Day is to raise awareness amongst the general public and decision-makers about rare diseases and their impact on patients' lives.

Building awareness of rare diseases is so important because 1 in 10 people will live with a rare disease at some point in their life.

Despite this, there is no cure for the majority of rare diseases and many go undiagnosed.

Almost every state participates in this important day. Share your story & get involved in your community, use this link and search by your state: **www.rarediseaseday.us/.**

**AF**

# Formulary Changes

A formulary change is when an insurance company modifies the list of prescription drugs that it will help pay for. Recent formulary changes in amyloidosis medications, particularly regarding drugs like tafamidis (Vyndamax and Vyndqel), have seen some Medicare Part D plans restricting coverage due to high costs, meaning patients may need to switch plans or seek financial assistance to access these vital treatments for conditions like transthyretin amyloid cardiomyopathy (ATTR-CM).

If you need help navigating these changes, reach out to us at **248-922-9610.** We have resources to apply for assistance and may be able to help.

**AF**

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Scan this code with the camera on your mobile device and get directed to our webpage!