News and Stories - Summer 2025

Support Group Meetings

The AF hosts support group meetings in Tennessee, providing patients and families with opportunities for connection, education, and encouragement.

Board Member and amyloidosis nurse, Adrienne Molteni, leads quarterly meetings in Nashville and Knoxville. The group began in August 2011 with its first gathering at Vanderbilt University Medical Center.

Adrienne shares the value of these gatherings, "The Nashville Area Amyloidosis Patient Support Group meets quarterly for lunch, wonderful conversation and up to date information on amyloidosis therapies, clinical trials and supportive resources. We have patient speakers, provider and pharma presentations and open discussions about the latest in the amyloidosis space. We strive to spread the word about this not-so-rare disease. We believe knowledge is power!".

We are grateful to this remarkable woman for her continued leadership and dedication to supporting amyloidosis patients and their families. To find upcoming support group affiliate dates and locations, please visit: *www.amyloidosis.org/resources*

AF

We earned a 2025 Candid Platinum Seal!

Keep up with our work in the community using the link here: *https://bit.ly/4candid*

It will give you unlimited access. We rely on our profile to gather support; it enables funders and donors to find us and learn about our impact. We'll keep our profile up to date so you can see what your donations help us accomplish.

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www.amyloidosis.org

Save the Date!

SEATTLE AMYLOIDOSIS SUPPORT GROUP ANNUAL IN-PERSON GATHERING

AUG. 7, 2025 at 4 pm

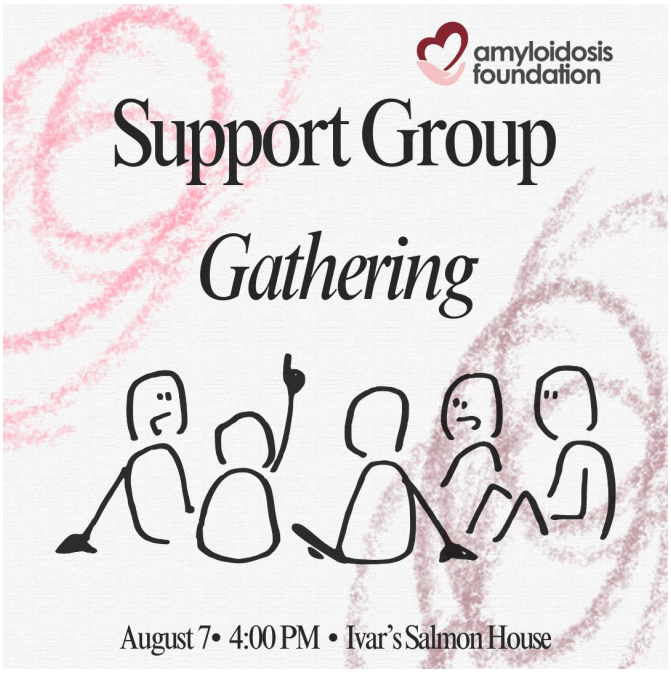
Ivar's Salmon House Restaurant on Lake Union- Seattle

401 NE Northlake Way, Seattle, WA 98105

A private room is reserved.

RSVP to:

sally@pnwamyloidosissupport.org



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

Follow Us!

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Stay connected for all the latest information on Amyloidosis:

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

REGISTER TODAY!!!

Time is running out—just a few weeks remain to join our Run for Your Life: **Run-Walk-Roll-Bike** event!

This fun and flexible **virtual** event lets you participate on your own schedule, all while raising awareness for amyloidosis. Rally your family and friends to sign up and get moving—whether you run, walk, bike, or roll, every step counts!

√ Now through July 31, 2025
√ Registration includes a newly designed event T-shirt!
√ Registration is OPEN now!

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

Let's make every mile matter!

5K

10K

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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 and BridgeBio are competing for people who are switching from Pfizer's blockbuster ATTR amyloidosis drug tafamidis, while all three companies are fighting for new patients.

A three-way tussle has emerged in the growing ATTR amyloidosis market as Alnylam and BridgeBio Pharma have secured FDA approvals to challenge Pfizer.

Tafamidis, which Pfizer sells as Vyndaqel, Vyndamax and Vynmac, had the ATTR amyloidosis with cardiomyopathy (ATTR-CM) market to itself for five years. But BridgeBio won approval for Attruby, a drug with the same mechanism of action as tafamidis, in November 2024. Then the oral transthyretin stabilizers were joined on the market by the first silencer, Alnylam's Amvuttra, in March. Amvuttra was already approved in ATTR with polyneuropathy (ATTR-PN).

ATTR-CM causes stiffening of cardiac muscle and heart failure, leading to death in around 2.5 years if left untreated. Cardiac capacity cannot be regained once lost. BridgeBio estimates there are around 240,000 ATTR-CM patients in the U.S. and BMO Capital Markets analyst Kostas Biliouris said he believes the market is big enough to accommodate multiple assets. Even so, competition is inevitable and the stakes are high.

Pfizer Bullish as Rivals Arrive

Pfizer reported $5.4 billion in sales for tafamidis in 2024, up more than 60% year on year. Matt Outten, chief commercial officer at BridgeBio, said at a Leerink investor event in March that around 24,000 people are on tafamidis. And about 30% to 40% of patients on tafamidis "are probably progressing" while a further 2,000 to 3,000 people are being newly diagnosed each quarter, he added.

Alnylam and BridgeBio are now competing for people who are switching from tafamidis. Pfizer CFO David Denton acknowledged the effect of the new entrants on a call with analysts to discuss 2025 guidance, saying that "Vyndaqel's growth will be tempered primarily due to the unfavorable impact of the IRA and new competition in the U.S."

Pfizer CEO Albert Bourla provided a detailed look at how the company expects the market to evolve at a TD Cowen event in March.

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Alnylam and BridgeBio

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The chief executive is confident Pfizer can keep current users, arguing that physicians "will never switch a patient to something different" when a medicine is working well. He is equally bullish on the company's ability to compete for first-line users.

Bourla said that Pfizer "will maintain a lion's market share" for new prescriptions "because of the network, the understanding of physicians of the brand." Asked if he expects Pfizer to dominate new prescriptions, Bourla said "correct," adding that the company will be "super dominant on the current prescriptions."

Alnylam Targets First-Line Market

Alnylam has other ideas. On a conference call with analysts to discuss the FDA approval, Alnylam CEO Yvonne Greenstreet said Amvuttra has the potential to become the new standard of care. Tolga Tanguler, chief commercial officer at the company, used the call to make the case that ATTR-CM patients, knowing cardiac function cannot be regained, will want to get the injectable Amvuttra as a first-line therapy.

The biotech is forecasting sales of Amvuttra and Onpattro, the company's other approved ATTR-PN drug, will rise to between $1.6 billion and $1.725 billion this year, a 36% increase at the midpoint of the range. Last year, when Alnylam only had approvals in ATTR-PN, the franchise grew 34%.

Alnylam is charging a premium for Amvuttra. The annual wholesale acquisition cost for Pfizer's tafamidis is $268,000. BridgeBio priced Attruby at $244,500 a year. Alnylam set Amvuttra's list price at an annual cost of $476,000. Tanguler said Alnylam will cut the net price of Amvuttra "via rebates and value-based agreements" as prescription uptake increases.

The Inflation Reduction Act could also impact what patients pay for Amvuttra. The law capped Medicare Part D out-of-pocket spending at $2,000. A patient who has already hit the cap, because they are on other medications, will pay nothing to take tafamidis or Attruby. Amvuttra is a Medicare Part B drug. These services have a 20% coinsurance. Medicare Advantage caps the annual cost at $9,350. Alnylam has been optimizing access pathways at those health systems to ensure people can receive the drug "at a convenient location, whether that's an office, at an infusion center or, in some cases, in the patient's home," Tanguler said. The biotech had nearly 2,000 alternate sites of care as of March 20, Tanguler said, and was close to its goal of establishing treatment sites within 10 miles of 90% of patients.

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Alnylam and BridgeBio

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Tanguler said, and Alnylam expects similarly broad coverage in ATTR-CM. The biotech is talking to U.S. payers and expects ATTR-CM sales to start ramping up in the second half of 2025. Alnylam is taking other steps to ensure patients can access Amvuttra, with a focus on the 170 health systems where 80% of ATTR-CM patients are treated.

BridgeBio Builds Launch Momentum

Alnylam is presenting Amvuttra's quarterly subcutaneous dosing as a strength. BridgeBio pushed back against that idea. Outten said some ATTR-CM patients cannot drive or prefer not to. Family members might need to take hours out of their days to drive patients, who are often seniors, for injections, Outten said.

While Alnylam's roll out of alternate sites of care could bring down geographic barriers to Amvuttra use, Outten expects some patients to prefer oral treatments. ATTR-CM patients already take pills multiple times a day for conditions such as high blood pressure, Outten said, so adding another drug has little effect on their routines.

Data on BridgeBio's Attruby launch predate the approval of Amvuttra. The first two months of the launch exceeded expectations, BMO's Biliouris said, with around 500 HCPs writing prescriptions for more than 1,000 patients. Biliouris predicted BridgeBio will generate first-quarter sales of $7 million to $14 million, beating the $5 million to $6 million consensus analyst forecast.

The competitive landscape looks likely to change in the coming years. Pfizer's tafamidis is set to lose patent protection in the U.S. in 2028, allowing cheaper generic copies to come to market. Tanguler said the availability of generics might make combination therapy viable. In an April 4 note to investors, Mizuho analysts said that Pfizer's work on a tafamidis tablet, which is in Phase I, could result in a formulation with patent protection through 2043.

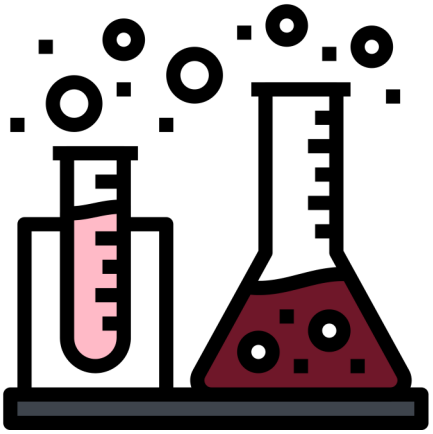
The Mizuho team cited Pfizer's investment in the tablet formulation as evidence the ATTR-CM market "is likely to be more durable beyond 2028." With Alnylam, BridgeBio and Pfizer all betting on the opportunity, and generic companies waiting in the wings, the stage is set for intense competition and blockbuster sales.

By: Nick Paul Taylor, Originally Published by BioSpace.com on April 22, 2025

Amyloidosis Euchre Fundraiser

A special event was held at the Rockford Community Cabin in Rockford, Michigan, to honor the memory of Kevin Q. Sullivan. Kevin had a deep love for playing euchre with family and friends—a tradition he and his family cherished. His wife, Nancy, recalled, "We taught our kids euchre while traveling in the car. One of us would drive, and the other played euchre with the three kids."

Kevin's memory lives on through the stories shared, especially his journey with amyloidosis. Through this heartfelt gathering, nearly $12,000 was raised to support research and raise awareness about the disease. Surrounded by friends, family, and fellow euchre enthusiasts, they celebrated Kevin's life while contributing to a cause that took him far too soon. Every presence, every shared memory, and every donation made a meaningful difference in the fight against amyloidosis. Together, they honored Kevin's legacy and created lasting impact.



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