News and Stories - Summer 2024

FDA Rare Disease Innovation Hub to Enhance and Advance Outcomes for Patients

Recent rapid advances in the identification of promising drug targets and development of gene therapies offer momentum and potential to meet the needs of patients with rare diseases. In 2023, over half of all the novel drugs and biologics approved by the FDA's Center for Drug Evaluation and Research (CDER) and the FDA's Center for Biologics Evaluation and Research (CBER) were to prevent, diagnose or treat a rare disease or condition.

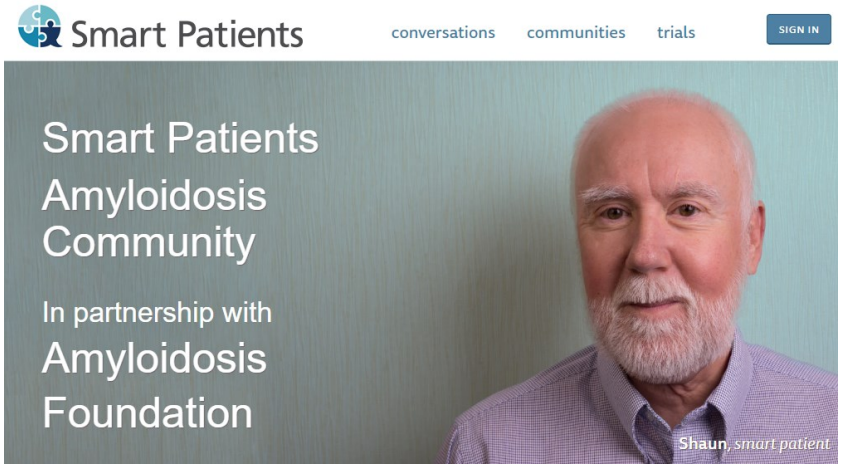
The Hub will work across rare diseases but will especially focus on products intended for smaller populations or for diseases where the natural history is variable and not fully understood, as we recognize that development of therapies for these conditions can be particularly challenging. The Hub would have three primary functions:

-Serve as a single point of connection and engagement with the rare disease community, including patient and caregiver groups, trade organizations, and scientific/academic organizations, for matters that intersect CDER and CBER.
-The Hub will help the larger rare disease community navigate important intersections across the FDA that affect patients with rare diseases, such as medical devices, including diagnostic tests, and combination products.
-Enhance intercenter collaboration to address common scientific, clinical and policy issues related to rare disease product development, including relevant cross-disciplinary approaches related to product review, and promote consistency across offices and Centers.
-Advance regulatory science with dedicated workstreams for consideration of novel endpoints, biomarker development and assays, innovative trial design, real world evidence, and statistical methods. **AF**

Smart Patients Community

The Amyloidosis Foundation (AF) is proud to work with Smart Patients, an online community for patients and caregivers. Smart Patients was designed to extend the reach of our resources to an even broader group of patients who can learn from each other about treatments, the latest science, and how it all fits into the context of their unique experience.

Join the Smart Patients amyloidosis community today - ask a question, and support others who can learn from you.



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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**

REGISTER BEFORE AUGUST 15!!!

The 'Run for Your Life' Run-Walk-Roll-Bike is off to a great start!!!

Participate in this annual (and virtual) event at your leisure to help raise awareness of amyloidosis! Encourage your family and friends to register and participate!

Run/walk/bike/roll anytime between *May 15, 2024 and August 15, 2024*. REGISTRATION INCLUDES A T-SHIRT!

REGISTER HERE OR USE THE QR CODE ABOVE:
https://bit.ly/RunWalkRollBike2024 **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

Alnylam Reports Positive Topline Results

Alnylam Reports Positive Topline Results from HELIOS-B Phase 3 Study of Vutrisiran, Achieving Statistical Significance on Primary and All Secondary Endpoints in Both Overall and Monotherapy Populations.

Key Points:
– Achieved 28% and 33% Reduction in Composite of All-Cause Mortality and Recurrent Cardiovascular Events in the Overall and Monotherapy Populations, Respectively
– Reduced All-Cause Mortality by 36% and 35% in the Overall and Mono-therapy Populations, Respectively, in a Pre-Specified Secondary Endpoint
– Demonstrated Clinically Significant Benefits on 6-Minute Walk Test, Kansas City Cardiomyopathy Questionnaire and NYHA Class – Key Measures of Disease Progression
– Observed Consistent Effects in All Key Subgroups, Including Baseline Tafamidis
– Demonstrated Encouraging Safety, Consistent with Established Profile
– Alnylam to File a U.S. Supplemental New Drug Application Using a Priority Review Voucher

Alnylam Pharmaceuticals, Inc. (Nasdaq: ALNY), the leading RNAi therapeutics company, announced on June 24, 2024 positive topline results from its HELIOS-B Phase 3 study of vutrisiran, an investigational RNAi therapeutic in development for the treatment of ATTR amyloidosis with cardiomyopathy (ATTR-CM).
The study met the primary endpoint, demonstrating a statistically significant reduction in the composite of all-cause mortality and recurrent cardiovascular (CV) events during the double-blind period in both the overall population (HR 0.718, p-value 0.0118; n=654) and in the monotherapy population (patients not receiving tafamidis at baseline; HR 0.672, p-value 0.0162; n=395).
The study also demonstrated statistically significant improvements across all secondary endpoints in both the overall and monotherapy populations. This includes key measures of disease progression: 6-minute walk test (6MWT), Kansas City Cardiomyopathy Questionnaire (KCCQ) and New York Heart Association (NYHA) Class at Month 30 ($p<0.025$ for all). Importantly, treatment with vutrisiran also reduced all-cause mortality in the overall population (HR 0.645, $p<0.025$) and in the monotherapy population (HR 0.655, $p<0.05$) up to Month 42. This was a pre-specified, intent-to-treat analysis that included up to six months of data from the open-label extension.

Alnylam, Cont'd

"I'm thrilled by these overwhelmingly positive data from the HELIOS-B study, which suggest that vutrisiran has the potential to address the needs of patients with ATTR amyloidosis with cardiomyopathy, a steadily progressive, debilitating, and ultimately fatal disease," said Pushkal Garg, M.D., Chief Medical Officer of Alnylam. "The results showed that vutrisiran improved cardiovascular out comes, including survival, function and quality of life in all patient groups with ATTR cardiomyopathy. We are moving with urgency to file these compelling data with regulators to bring this medicine to patients around the world."
In addition, vutrisiran demonstrated consistent effects on the primary composite endpoint and all secondary endpoints across all key subgroups, including baseline tafamidis use, ATTR disease type and measures of disease severity.
In the HELIOS-B study, vutrisiran demonstrated encouraging safety and tolerability, consistent with its established profile. Rates of adverse events (AEs), serious AEs and AEs leading to study drug discontinuation were similar between the vutrisiran and placebo arms. No AEs were seen ≥3% more frequently in the vutrisiran arm compared to the placebo arm.

"I am overjoyed by the results of the HELIOS-B study, which suggest the potential for vutrisiran to be a transformative medicine for patients with ATTR amyloidosis with cardiomyopathy," said Yvonne Greenstreet, MBChB, Chief Executive Officer of Alnylam. "Assuming favorable regulatory review, vutrisiran has the potential to become the new standard of care for the treatment of this disease, driving Alnylam's next era of substantial growth."

HELIOS-B (NCT: NCT04153149) is a Phase 3, randomized, double-blind, placebo-controlled multicenter global study designed and powered to evaluate the efficacy and safety of vutrisiran on the reduction of all-cause mortality and recurrent cardiovascular events as a primary composite endpoint in patients with ATTR amyloidosis with cardiomyopathy in the overall and monotherapy populations. The study randomized 655 adult patients with ATTR amyloidosis (hereditary or wild-type) with cardiomyopathy. Patients were randomized 1:1 to receive vutrisiran 25mg or placebo subcutaneously once every three months during a double-blind treatment period of up to 36 months. After the double-blind period, all eligible patients remaining on the study may receive vutrisiran in an open-label extension period.

Detailed results from the HELIOS-B study have been submitted as a late-breaking abstract to the European Society of Cardiology for presentation. The Company plans to proceed with global regulatory submissions starting later this year, including filing a supplemental New Drug Application with the U.S. Food and Drug Administration using a Priority Review Voucher. **AF**

Patient Story Update!

By: Jason Conway

Update: July 2024

I like to think of my amyloidosis story as a true success story. When I was diagnosed with AL amyloidosis with cardiac involvement in January of 2015 my life changed drastically. Living life with amyloidosis was such an uncertainty and I had no idea how my life was going to play out. But here I am and I am doing really well. I am living a life that is basically a normal life.

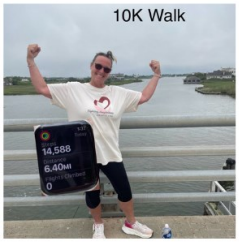
There are big moments that I remember since being diagnosed with amyloidosis. I remember Dr. Michael Wheat telling us that he suspected that I had amyloidosis. I remember being told that I did, in fact, have amyloidosis with cardiac involvement. And, I remember my first appointment with Dr. Jayesh Mehta, my oncologist at Northwestern Memorial Hospital in Chicago. These memories are so vivid because there was so much emotion attached to these events.

Interestingly enough, there are memories that I have that are tied to events that are a bit more mundane. Early on Dr. Sanjiv Shah, my cardiologist at Northwestern, told me that my heart would begin to repair itself and would improve. This seemed like an unattainable goal because my BNP was so elevated. Years later, with a BNP in the normal range, Dr. Shah informed me that I was now just a normal guy with a normal heart. It took a moment to register, but that was a big win to me.

Over the years, I have built a close relationship with Dr. Mehta and my health has continued to be stable, and our conversations during my appointments no longer really seem to be focused solely on amyloidosis and my treatments. We talk about family, work, travel. It was during one of these conversations that he revealed to me that during my first appointment, he was really concerned about my prognosis because my heart was in such bad shape. Then, he shared with me my numbers and said that I am doing truly remarkable. At this point, he said that I have my whole life ahead of me.

With amyloidosis, early diagnosis is the key and quality of life is the result. My quality of life includes friends, travel, and work, but most importantly, family. Time spent with my wife is cherished and I soak in every moment with her. Watching our children graduate and move on to the next stages of their lives is priceless. I will never be able to repay my doctors and the Amyloidosis Foundation for my quality of life. Just know that I am living my best life while being the voice for amyloidosis and the need for early diagnosis. **AF**

AF Run-Walk-Roll-Bike Snapshots



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MAY 15 - AUGUST 15
VIRTUAL

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ROLL · BIKE

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