Future Cardiology

ISSN: 1479-6678 (Print) 1744-8298 (Online) Journal homepage: www.tandfonline.com/journals/ifca20

Effect of acoramidis on risk of death and heart problem-related hospitalizations in people with transthyretin amyloid cardiomyopathy: a plain language summary

Daniel P. Judge, Kevin M. Alexander, Francesco Cappelli, Marianna Fontana, Pablo Garcia-Pavia, Martha Grogan, Mazen Hanna, Ahmad Masri, Laura Obici, Prem Soman, Xiaofan Cao, Ted Lystig, Jean-François Tamby, Suresh Siddhanti, Adam Castaño, Leonid Katz, Jonathan C. Fox, Kenneth W. Mahaffey & Julian D. Gillmore

To cite this article: Daniel P. Judge, Kevin M. Alexander, Francesco Cappelli, Marianna Fontana, Pablo Garcia-Pavia, Martha Grogan, Mazen Hanna, Ahmad Masri, Laura Obici, Prem Soman, Xiaofan Cao, Ted Lystig, Jean-François Tamby, Suresh Siddhanti, Adam Castaño, Leonid Katz, Jonathan C. Fox, Kenneth W. Mahaffey & Julian D. Gillmore (05 Aug 2026): Effect of acoramidis on risk of death and heart problem-related hospitalizations in people with transthyretin amyloid cardiomyopathy: a plain language summary, Future Cardiology, DOI: 10.1080/14796678.2026.2702094

To link to this article: https://doi.org/10.1080/14796678.2026.2702094

Published online: 05 Aug 2026.

Future CARDIOLOGY

Plain Language Summary of Publication

Effect of acoramidis on risk of death and heart problem-related hospitalizations in people with transthyretin amyloid cardiomyopathy: a plain language summary

Daniel P. Judge[a], Kevin M. Alexander[b,c], Francesco Cappelli[d], Marianna Fontana[e], Pablo Garcia-Pavia[f], Martha Grogan[g], Mazen Hanna[h], Ahmad Masri[i], Laura Obici[j], Prem Soman[k], Xiaofan Cao[l], Ted Lystig[l], Jean-François Tamby[l], Suresh Siddhanti[l], Adam Castaño[l], Leonid Katz[l], Jonathan C. Fox[l], Kenneth W. Mahaffey[c] & Julian D. Gillmore[e]

Author affiliations can be found at the end of this article.

First draft submitted: 1 April 2026; Accepted for publication: 7 July 2026

Where can I find the original article on which this summary is based?

The full article, titled 'Efficacy of acoramidis on all-cause mortality and cardiovascular hospitalization in transthyretin amyloid cardiomyopathy', was published in the *Journal of the American College of Cardiology* and is available for free at: https://www.sciencedirect.com/science/article/pii/S0735109724105566

Summary

How to say (download PDF and double click sound icon to play sound)...

- **Acoramidis:** AH-kor-AH-mid-dis
- **Transthyretin amyloid cardiomyopathy:** Trans-THY-re-tin AH-mi-loy-d CARD-eo-my-OP-athee

What is this summary about?

Transthyretin amyloid cardiomyopathy (ATTR-CM for short) is a serious heart condition caused when a **protein** called transthyretin (TTR for short) becomes unstable. In this disease, unstable TTR protein breaks apart and misfolds to form harmful clumps called **amyloid fibrils**, which build up in the heart over time, causing thickening of the heart muscle. This makes it difficult for the heart to pump blood effectively, resulting in heart failure. There are two main types of ATTR-CM: wild-type ATTR-CM (caused by unknown reasons) and variant ATTR-CM (caused by specific protein-destabilizing variants in the TTR **gene**). Medicines for ATTR-CM help people with the disease live longer and stay out of hospital. Acoramidis is a medicine available in the US, Europe, and a few other countries for treating ATTR-CM in adults.

This is a summary of an investigation of participants in a clinical trial called ATTRibute-CM, in which researchers looked at the effects of acoramidis in reducing the risk of **death from any cause** or hospitalizations due to heart problems compared with **placebo** (which is an inactive treatment).

What are the key takeways?

People who took acoramidis had a lower combined risk of dying from any cause or of being hospitalized due to heart problems. These benefits started as early as 3 months after starting treatment with acoramidis and continued through 30 months of the study.

Taylor & Francis by informa

ISSN 1744-8298

Future Cardiol. (Epub ahead of print)

The treatment worked consistently well across different groups of people, including men and women, people with different causes of ATTR-CM (wild-type and variant), and participants from different study countries. No major safety concerns were reported with acoramidis.

The results of this study may provide useful information about the use of acoramidis for the treatment of people with ATTR-CM.

Protein: A large complex molecule that is essential to the structure, function, and regulation of the body. A protein is composed of long chains of amino acids that fold into specific 3D shapes to function correctly.

Amyloid fibrils: Clumps of misfolded proteins.

Genes: Short pieces of DNA. Many genes provide instructions for making specific proteins.

Death from any cause: Includes deaths that result from any kind of disease, other physical condition, injury, or natural causes.

Placebo: A treatment that looks like the genuine medicine but has no active properties. Placebos are used to help researchers distinguish between the true effects of treatment and other factors linked with being in the study, such as being closely monitored and cared for by the research team, or simply expecting the treatment to be helpful.

What is the purpose of this plain language summary?

Acoramidis is approved in the US, Europe, and a few other countries for the treatment of wild-type and variant ATTR-CM in adults. The approval of acoramidis was based on the results of the ATTRibute-CM study. The results of this study may differ from those of other studies. Health professionals should make treatment decisions based on all available evidence, not on the results of a single study.

Acoramidis is approved to treat the condition that is discussed in this summary. The purpose of this plain language summary is to help you understand our research findings.

Who is this article for?

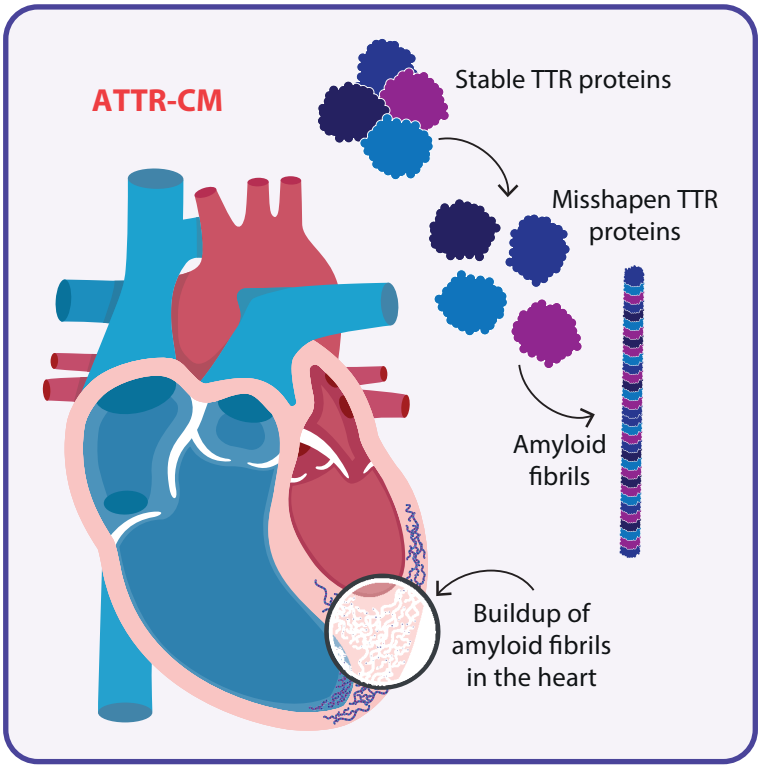
This summary may be helpful to non-scientific professionals who are involved in the care of people with ATTR-CM, people living with ATTR-CM and their families, caregivers, and patient advocates.

Who sponsored this study?

This study was **sponsored** by BridgeBio Pharma, Inc.

Sponsor: A company or organization that oversees and pays for a clinical research study. The sponsor also collects and analyzes the information from the study.

What is transthyretin amyloid cardiomyopathy (ATTR-CM)?



ATTR-CM is a serious heart condition that develops when a protein called transthyretin or TTR (also known as prealbumin) becomes unstable, breaks apart, and misfolds to form harmful amyloid fibrils that build up in the heart.

The buildup of amyloid fibrils in the heart muscle causes it to thicken and stiffen. This makes the heart less effective at pumping blood around the body, resulting in heart failure. Without treatment, ATTR-CM worsens over time and leads to heart failure, frequent hospitalizations due to heart problems, reduced ability to exercise or perform daily activities, a reduced **quality of life**, and often death.

Quality of life: The overall well-being of a person, including physical, mental, and social aspects. In clinical studies, researchers measure quality of life to understand how a disease and its treatment affect a person's daily life.

There are two types of ATTR-CM:

'Wild-type' ATTR-CM
- This type of ATTR-CM develops for unknown reasons.
- It typically affects people over 60 years old.
- It is not hereditary, and family members do not need to be tested.

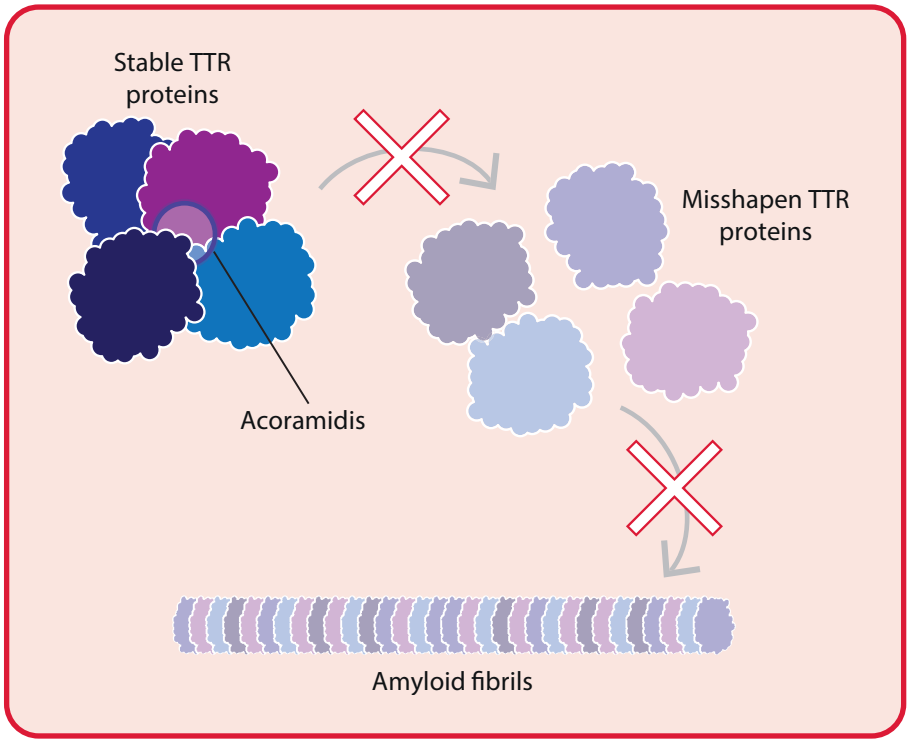
'Variant' ATTR-CM
- This type of ATTR-CM is also called 'hereditary' ATTR-CM.
- It is caused by a faulty gene and can be passed down from parents to their children.
- The age when a person first experiences symptoms of ATTR-CM depends on the DNA variant, with some versions having onset as early as 30 years old and others being more common after 60 years old.
- Variant ATTR-CM is less common than wild-type ATTR-CM.

What is acoramidis?

Acoramidis is an oral (taken by mouth) medicine designed to mimic (in other words, imitate or copy) the protective benefits of a rare **genetic variant** called p.T139M. People with the p.T139M variant have very stable TTR protein, which makes them less likely to develop ATTR-CM and, if they do develop the disease, they are more likely to have milder symptoms.

Acoramidis works by tightly binding to the TTR protein and keeping it stable, preventing it from breaking apart and forming harmful amyloid fibrils that build up in the heart. This makes it different from another type of ATTR-CM medication called TTR silencers that reduce the production of TTR protein.

Genetic variant: A piece of DNA that is different from the most common form of DNA that makes up a gene. A protective genetic variant can protect a person from developing a disease. For example, the genetic variant called p.T139M protects people from developing ATTR-CM by making the TTR protein extra stable.

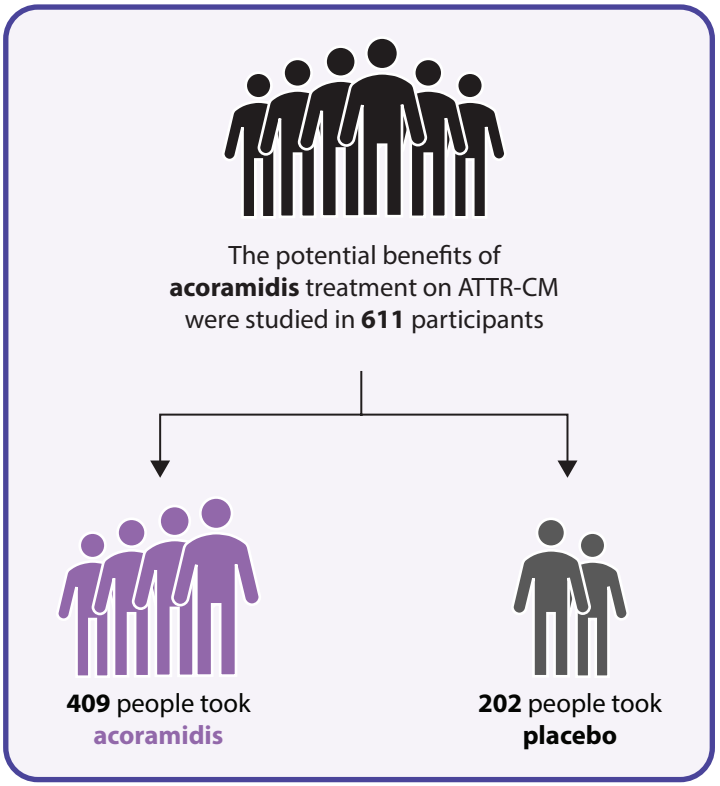


Laboratory tests show that acoramidis stabilizes 90% or more of the TTR protein circulating in the body, thus nearly completely stabilizing the protein.

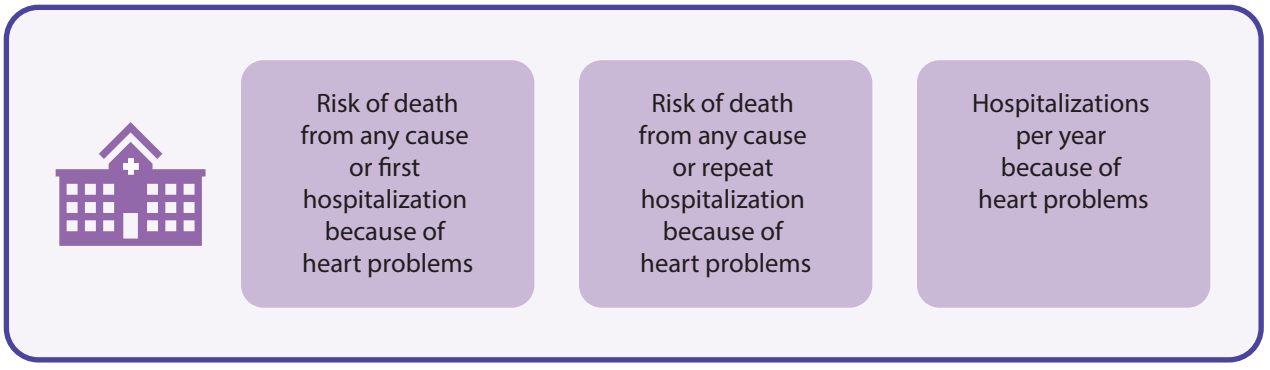
In the ATTRibute-CM study, participants who took acoramidis had fewer deaths and fewer hospital stays for heart problems than those who took a placebo. Participants who took acoramidis had less decline in physical function and quality of life over the duration of the study than those who took a placebo.

What did this study look at?

The ATTRibute-CM study included 632 people with ATTR-CM. The potential benefits of acoramidis treatment on ATTR-CM were investigated in 611 of the study participants, who received acoramidis or placebo. The safety and side effects of acoramidis were investigated in all 632 study participants.



Medicines for ATTR-CM help people with the disease live longer and stay out of hospital. Therefore, in this analysis, researchers wanted to look at the effect of acoramidis on the following outcomes:



In this study, when researchers defined death from any cause, they also included heart transplantation and implantation of a mechanical device to help the heart pump properly.

Researchers wanted to find out if acoramidis reduces the risk of a bad outcome (such as death or hospitalization) in people with ATTR-CM, by how much and by when, compared with placebo.

Risk reduction can be measured by comparing the percentage of people experiencing a bad outcome while taking the medicine compared with those taking placebo. For example, a bad outcome might occur in 30% of people taking the medicine and in 60% of people taking placebo. This would be an absolute risk reduction of 30% (the difference in percentages between the two groups) and a relative risk reduction of 50% (the difference in percentages divided by the placebo group's percentage). Researchers use standard statistical methods to identify the true effect of the treatment on the outcome.

In addition, researchers wanted to find out how likely it is that acoramidis will benefit an individual person with ATTR-CM. This can be measured by using the Number Needed to Treat value, also called NNT, which is the number of people you would need to treat to prevent one additional bad outcome. For example, if a medicine has an NNT of 5, it means that, on average, five people would need to be treated with the medicine to prevent one additional bad outcome.

In this investigation, the researchers wanted to know the:

? Number needed to treat with acoramidis to prevent one additional person from dying or being hospitalized for the first time for heart problems over 30 months of treatment

? Number needed to treat with acoramidis to prevent one heart problem-related hospitalization per year over 30 months of treatment

Who took part in this study?

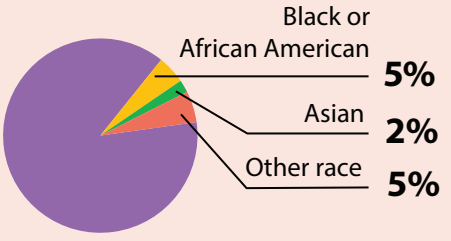
People with ATTR-CM (wild-type and variant) took part in this study. The characteristics of participants in this study were similar to those of people with ATTR-CM in the general population.

The potential benefits of acoramidis treatment on ATTR-CM were studied in **611** participants.

91% were men

Average age: **77** years

90% had 'wild-type' ATTR-CM



88% were White

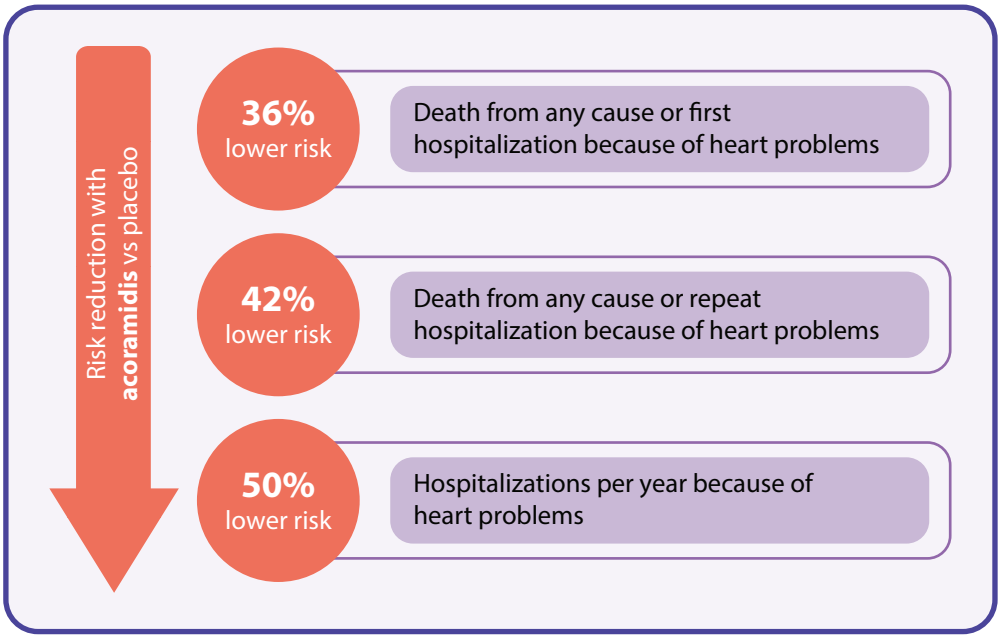
What were the results of the study?

The percentage of people who died or had a first hospitalization due to heart problems during the study was 35.9% in the acoramidis group and 50.5% in the placebo group.

Compared with people taking placebo, those taking acoramidis had a 36% lower risk of death due to any cause or having a first hospitalization for a heart problem. This benefit compared with placebo could be seen as early as 3 months after starting acoramidis treatment and continued over the study duration of 30 months.

The average number of events (death or heart problem-related hospitalizations) that people experienced during the study was lower (0.64 per person) in people taking acoramidis compared with people taking placebo (1.10 per person). People taking acoramidis had a 42% lower risk of death from any cause or repeat hospitalization for a heart problem.

The average number of hospitalizations per year because of heart problems was also lower (0.22 per person) for people taking acoramidis compared with people taking placebo (0.45 per person). Thus, people in the acoramidis group had a 50% lower risk of heart problem-related hospitalizations per year than people in the placebo group.



Researchers found that the protective effect of acoramidis on death from any cause or first hospitalization for a heart problem was seen across different groups of study participants. These included men and women, people with wild-type and variant ATTR-CM, and participants from different study countries.

Researchers found that, on average, seven people with ATTR-CM needed to be treated with acoramidis to prevent one additional death or first hospitalization for heart problems over 30 months of treatment. Researchers also found that five people needed to be treated with acoramidis to prevent one heart problem-related hospitalization per year over 30 months of treatment.

What were the side effects of acoramidis treatment?

In this study, side effects of acoramidis treatment that occurred in a greater proportion of acoramidis participants than placebo participants included stomach-area (upper abdominal) pain and diarrhea. Upper abdominal pain occurred in 6% of 421 people taking acoramidis and 1% of 211 people taking placebo. Diarrhea occurred in 12% of 421 people taking acoramidis and 8% of 211 people taking placebo. Most of these side effects were mild.

Additionally, the side effects of acoramidis experienced in participants with variant ATTR-CM were similar to those with wild-type ATTR-CM. These results can be found in other papers published on the ATTRibute-CM study, linked in the 'Where can I find more information about this study' section.

What were the limitations of the study?

- After the first 12 months of the study, some participants also started taking another medication for ATTR-CM called tafamidis, based on their doctor's advice. More participants took tafamidis in the placebo group than in the acoramidis group. This could make acoramidis look less effective than it really is and make placebo seem more effective than it really is.
- A small number of participants (2.1% overall) were taking a heart failure medication called a sodium-glucose cotransporter 2 (SGLT2) inhibitor at the start of the ATTRibute-CM study.
- Because few participants in the ATTRibute-CM study were women or Black/African American, these results may not fully reflect experiences of these groups.

What were the main conclusions?

- In the ATTRibute-CM study, acoramidis treatment reduced the risk of death from any cause or hospitalization because of heart problems compared with placebo.
- These positive effects of acoramidis were seen early in the study, after 3 months of treatment, and continued over the study duration (through 30 months).
- The most common side effects with acoramidis were mild and included abdominal pain and diarrhea.
- The results of this study may provide useful information about the use of acoramidis for the treatment of people with ATTR-CM.

Where can I find more information about this study?

- The full citation to the original article is: Judge DP, *et al*. Efficacy of acoramidis on all-cause mortality and cardiovascular hospitalization in transthyretin amyloid cardiomyopathy. *J Am Coll Cardiol*. 2025;85(10):1003-1014. This article is available for free at: https://www.sciencedirect.com/science/article/pii/S0735109724105566
- The first paper published on the ATTRibute-CM study, 'Efficacy and safety of acoramidis in transthyretin amyloid cardiomyopathy', was published in the *New England Journal of Medicine* and is available for free at: https://www.nejm.org/doi/full/10.1056/NEJMoa2305434
- A paper published on the ATTRibute-CM study comparing participants with variant and wild-type ATTR-CM, 'Efficacy of Acoramidis in Wild-Type and Variant Transthyretin Amyloid Cardiomyopathy', was published in *JAMA Cardiology* and is available for free at: https://jamanetwork.com/journals/jamacardiology/fullarticle/2841140
- A plain language review of the key results of the ATTRibute-CM study was published in *Future Cardiology* and is available for free at: https://www.tandfonline.com/doi/abs/10.1080/14796678.2025.2591426
- You can read more about ATTRibute-CM at ClinicalTrials.gov (www.clinicaltrials.gov): enter the NCT number, NCT03860935, into the search field to find the ATTRibute-CM study.

Acknowledgments

The researchers and sponsor thank all of the participating patients, their families and caregivers, investigators and study center staff. The authors also thank Jennifer Greenman, LCSW, from the Amyloidosis Research Consortium for reviewing this plain language summary, and Shweta Rane, PhD, CMPP, of BridgeBio Pharma, Inc. for critical review and editorial support. Some sections of this plain language summary were generated with artificial intelegence using ChatGPT Enterprise Version (GPT-5.2, Open AI).

Disclosure statement

Kevin M. Alexander – Consultant: Arbor Biotechnologies, Attralus, Intellia Therapeutics, Prothena. Xiaofan Cao – Employment: BridgeBio Pharma, Inc. Francesco Cappelli – Consultant: Alnylam Pharmaceuticals, Amicus Therapeutics, AstraZeneca, BridgeBio Pharma, Inc, Novo Nordisk, Pfizer. Adam Castaño – Employment: BridgeBio Pharma, Inc. Marianna Fontana – Consultant: Akcea Therapeutics, Alexion Pharmaceuticals, Alnylam Pharmaceuticals, AstraZeneca, BridgeBio Pharma, Inc, Intellia Therapeutics, Ionis Pharmaceuticals, Janssen Global Services, Novo Nordisk, Pfizer; Grant: BridgeBio Pharma, Inc, Pfizer. Jonathan C. Fox – Employment: BridgeBio Pharma, Inc. Pablo Garcia-Pavia – Consultant: Alexion Pharmaceuticals, Alnylam Pharmaceuticals, AstraZeneca, Attralus, Bayer, BridgeBio Pharma, Inc, Intellia Therapeutics, Ionis Pharmaceuticals, Neuroimmune, Novo Nordisk, Pfizer; Grant/Contract: Alnylam Pharmaceuticals, AstraZeneca, BridgeBio Pharma, Inc, Intellia Therapeutics, Novo Nordisk, Pfizer. Julian D. Gillmore – Consultant: Alnylam Pharmaceuticals, AstraZeneca, Attralus, BridgeBio Pharma, Inc, Intellia Therapeutics, Ionis Pharmaceuticals, Lycia, Pfizer. Martha Grogan – Consultant: Alnylam, BridgeBio Pharma, Inc, Pfizer, Janssen Pharmaceuticals, Novo Nordisk; Grant/Contract: Alnylam Pharmaceuticals, BridgeBio Pharma, Inc, Intellia Therapeutics, Novo Nordisk, Janssen Pharmaceuticals, Pfizer. Mazen Hanna – Consultant: Alexion Pharmaceuticals, Alnylam Pharmaceuticals, BridgeBio Pharma, Inc, Ionis Pharmaceuticals, Pfizer. Daniel P. Judge – Consultant: Alexion Pharmaceuticals, Alleviant Medical, Alnylam Pharmaceuticals, Attralus, Cytokinetics, Lexeo Therapeutics, Novo Nordisk, Pfizer, Renovacor, Tenaya Therapeutics; Grant/Contract: Array Biopharma, BridgeBio Pharma, Inc, MyoKardia, Pfizer. Leonid Katz – Employment: BridgeBio Pharma, Inc. Ted Lystig – Employment: BridgeBio Pharma, Inc. Kenneth W. Mahaffey – Consultant: Applied Therapeutics, Bayer, Bristol Myers Squibb, BridgeBio Pharma, Inc, CSL Behring, Elsevier, Fosun Pharma, Human, Johnson & Johnson, Moderna, Myokardia, Novartis, Novo Nordisk, Otsuka, Phasebio, Portola, Quidel, Theravance; Grant/Contract: American Heart Association, Apple, Inc, Bayer, California Institute Regenerative Medicine, CSL Behring, Eidos, Ferring, Gilead, Google, Idorsia, Johnson & Johnson, Luitpold, Novartis, PAC-12, Precordior, Sanifit; Other: Human, Medeloop, Precordior, Regencor (equity). Ahmad Masri – Consultant: Akros Pharma, Alexion Pharmaceuticals, Alnylam Pharmaceuticals, AstraZeneca, Attralus, BioMarin Pharmaceutical, BridgeBio Pharma, Inc (formerly Eidos Therapeutics), Bristol Myers Squibb, Cytokinetics, Haya Therapeutics, Ionis Pharmaceuticals, Lexicon Pharmaceuticals, Pfizer, Prothena Biosciences, Tenaya Therapeutics; Grant/Contract: Attralus, Cytokinetics, Ionis Pharmaceuticals, Pfizer. Laura Obici – Consultant: Alnylam Pharmaceuticals, AstraZeneca, BridgeBio Pharma, Inc, Ionis Pharmaceuticals, Novo Nordisk, Pfizer, Sobi–Swedish Orphan Biovitrum. Suresh Siddhanti – Employment: BridgeBio Pharma, Inc. Prem Soman – Consultant: Alnylam Pharmaceuticals, BridgeBio Pharma, Inc, Pfizer. Grant/Contract: Pfizer. Jean-François Tamby – Employment: BridgeBio Pharma, Inc. The authors have no other relevant affiliations or financial involvement with any organization or entity with a financial interest in or financial conflict with the subject matter or materials discussed in the manuscript apart from those disclosed.

Medical writing support was provided by Anja Becher, PhD, and Sarah Graham, PhD, of Oxford PharmaGenesis Ltd., and was funded by BridgeBio Pharma, Inc.

Patient reviewers on this PLSP have received honorarium from *Future Cardiology* for their review work but have no other relevant financial relationships to disclose.

Peer reviewers on this manuscript have no relevant financial or other relationships to disclose.

ORCID details

Daniel Judge 0000-0002-3407-0248
Kevin Alexander 0000-0003-4024-3691
Francesco Cappelli 0000-0002-6173-5583
Marianna Fontana 0000-0002-9233-9831
Pablo Garcia-Pavia 0000-0002-5470-2257
Martha Grogan 0000-0002-2541-4440
Mazen Hanna 0000-0002-7251-3003
Ahmad Masri 0000-0002-6390-6526
Laura Piera Obici 0000-0001-7468-700X
Prem Soman 0000-0003-1425-6536
Xiaofan Cao 0009-0004-0337-2069
Ted Lystig 0000-0002-4523-0761
Jean-François Tamby 0009-0002-8892-4488
Suresh Siddhanti 0009-0008-3880-6209
Adam Castaño 0000-0003-4482-5840
Leonid Katz 0009-0006-7599-8022
Jonathan Fox 0000-0002-1250-5239
Kenneth Mahaffey 0000-0002-3791-2148
Julian Gillmore 0000-0001-6174-9232

Author affiliations

[a]Division of Cardiology, Medical University of South Carolina, Charleston, SC, US; [b]Division of Cardiovascular Medicine, Department of Medicine, Stanford University School of Medicine, Palo Alto, CA, US; [c]Stanford Center for Clinical Research, Stanford School of Medicine, Palo Alto, CA, US; [d]Tuscan Regional Amyloidosis Centre, Careggi University Hospital, Florence, Italy; [e]National Amyloidosis Centre, University College London, Royal Free Hospital, London, UK; [f]Department of Cardiology, Hospital Universitario Puerta de Hierro Majadahonda and Centro Nacional de Investigaciones Cardiovasculares (CNIC), Madrid, Spain; [g]Department of Cardiovascular Medicine, Mayo Clinic, Rochester, MN, US; [h]Cleveland Clinic, Cleveland, OH, US; [i]Division of Cardiology, Oregon Health & Science University, Portland, OR, US; [j]Amyloidosis Research and Treatment Centre, Fondazione IRCCS Policlinico San Matteo, Pavia, Italy; [k]Division of Cardiology, University of Pittsburgh Medical Center, Pittsburgh, PA, US; [l]BridgeBio Pharma, Inc., San Francisco, CA, US