

Understanding the benefits of taking tafamidis and the risk factors for admission into the hospital and death in people with transthyretin amyloid cardiomyopathy

Authors: Camille Vong, Martin Boucher, Steve Riley, and Lutz O. Harnisch



Date of summary: October 2020

The full title of this article is:
Modeling of Survival and Frequency of Cardiovascular-Related Hospitalization in Patients with Transthyretin Amyloid Cardiomyopathy Treated with Tafamidis

Study number: NCT01994889

Study start date: December 2013

Study end date: February 2018

© The authors, CC-BY-NC [2021]

DOI: 10.1007/s40256-021-00464-y

Amyloid

<A-muh-loyd>

Cardiomyopathy

<KAR-dee-oh-my-AH-puh-thee>

Tafamidis

<tah-FAM-ah-dis>

Transthyretin

<trans-thy-REH-tin>



What did this study look at?

- Transthyretin amyloidosis is a condition in which an abnormal protein forms amyloid and builds up in organs such as the heart
- It may lead to a type of heart disease called transthyretin amyloid cardiomyopathy (ATTR-CM for short)
- When amyloid protein builds up in the heart, it causes the heart walls to become thick and stiff
- This means that the heart struggles to pump enough blood around the body
- This condition is called heart failure



Tafamidis is an approved medicine for people with ATTR-CM

- It works by slowing the build-up of amyloid deposits in the heart

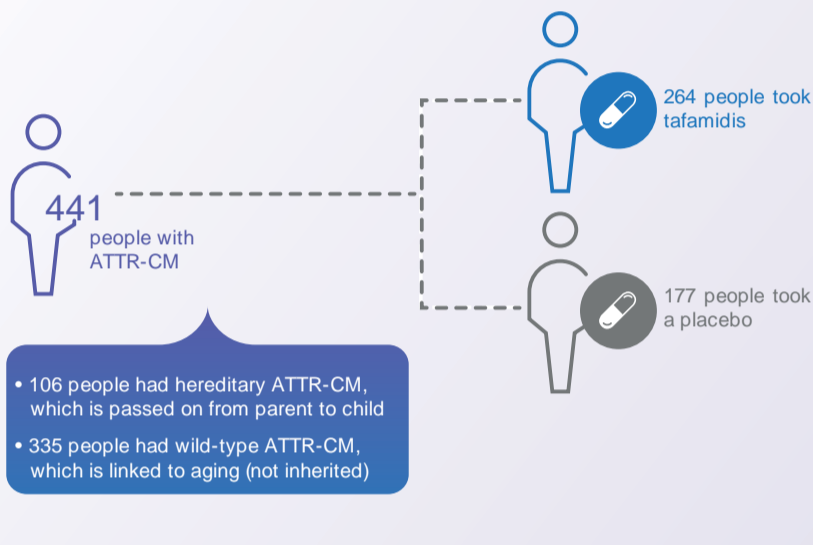


What was the aim of the study?

- Researchers developed a mathematical model using results from a clinical trial in which people received tafamidis or a placebo. A placebo does not contain any active medicine and looks like the study medicine
- They used this model to understand if tafamidis can:
 - reduce the risk of people with ATTR-CM being admitted into the hospital; or
 - reduce their risk of death
- Researchers also looked at whether different factors were linked to how likely people were to be admitted into the hospital or how long they were likely to live



Who took part in the study?



People took tafamidis or a placebo for up to:



30 months



What were the results of this study?



Risk of being admitted into the hospital because of heart problems



Tafamidis



Placebo

People with less severe symptoms of heart failure* who took tafamidis were

41%
LESS LIKELY

to be admitted into the hospital because of heart problems than those who took a placebo

*The New York Heart Association scoring system was used to group people based on the severity of their symptoms of heart failure. Those in class I or II were considered to have less severe symptoms

Researchers found 4 factors that could help identify people who were less likely to be admitted into the hospital because of heart problems:

2 were based on people's characteristics at the start of the study



The distance a person could walk in 6 minutes (the farther, the better the outcome)



People with a high left ventricular ejection fraction (a measurement of how much blood can be pumped out of the heart with each heartbeat)

2 were based on people's blood test results at the start of the study



Low levels of urea nitrogen in people's blood (indicates how well a person's kidneys and liver are working)



Low levels of a protein called troponin I (a measurement of heart damage)



Risk of death



Tafamidis



Placebo

People who took tafamidis were

42%
LESS LIKELY

to die than those who took a placebo

Researchers found 5 factors that could help identify people who were at a lower risk of death:

3 were based on people's characteristics at the start of the study



The distance a person could walk in 6 minutes (the farther, the better the outcome)



People with a high left ventricular ejection fraction (a measurement of how much blood can be pumped out of the heart with each heartbeat)



People with wild-type (not inherited) ATTR-CM

2 were based on people's blood test results at the start of the study



Low levels of urea nitrogen in people's blood (indicates how well a person's kidneys and liver are working)



Low levels of a hormone called NT-proBNP (a measurement of heart failure)



What were the main conclusions reported by the researchers?

- People with ATTR-CM who took tafamidis were less likely to be admitted to the hospital because of heart problems, or to die, than those who took a placebo
- Factors that could help determine a person's risk of being admitted into the hospital or death were consistent with those previously reported for people with ATTR-CM. These include people's characteristics (such as how far they can walk in 6 minutes) and blood test results
- For more information about this study, please visit:
 - <https://clinicaltrials.gov/ct2/show/NCT01994889>