**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

**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 is a substance that does not contain any active medicine and looks like the study medicine. The model aimed to investigate the benefits of taking tafamidis and the risk factors for admission into the hospital and death in people with transthyretin amyloid cardiomyopathy (ATTR-CM for short).

**Who took part in the study?**

- **Tafamidis group:** 177 people took tafamidis and were followed for up to 30 months.
- **Placebo group:** 264 people took a placebo and were followed for up to 30 months.

**What were the results of this study?**

Researchers found 5 factors that could help identify people who were less likely to be admitted into the hospital because of heart problems, or to die, than those who took a placebo:

- **Risk of death**
  - People took tafamidis or a placebo for up to:
    - **Study start date:** December 2013
    - **Study end date:** February 2018

**What did this study look at?**

Researchers compared a group of people who took tafamidis with a group of people who took a placebo. They looked at how likely people were to be admitted into the hospital or how long they stayed in the hospital; or how likely people were to die. The researchers reported the following findings:

- People who took tafamidis were 41% less likely to be admitted into the hospital because of heart problems.
- People who took tafamidis were 42% less likely to die than those who took a placebo.

**Who was included in the model?**

Researchers included people who had ATTR-CM. This is a condition in which an abnormal protein forms amyloid and builds up in organs such as the heart. 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. It may lead to a type of heart disease called heart failure. When heart failure occurs, it causes the person to feel tired and short of breath.

**Risk factors for admission into the hospital**

Factors that could help determine a person’s risk of being admitted into the hospital included:

- People with a high left ventricular ejection fraction (a measurement of how much blood can be pumped out of the heart with each heartbeat)
- The distance a person could walk in 6 minutes (the farther, the better the outcome)
- Low levels of a protein called troponin I (a measurement of heart damage)
- People with wild-type (not inherited) ATTR-CM

**Risk factors for death**

Factors that could help identify people who were less likely to live long enough to benefit from tafamidis included:

- The New York Heart Association scoring system was used to group people based on the severity of their symptoms
- Factors that could help identify people who were less severe symptoms
- People with hereditary ATTR-CM, which is linked to aging (not inherited)
- People with a high left ventricular ejection fraction (a measurement of how much blood can be pumped out of the heart with each heartbeat)
- The distance a person could walk in 6 minutes (the farther, the better the outcome)