Pfizer Rare Disease

FAMILIAL AMYLOIDOSIS SUPPORT MEETING

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US Medical Director, Tafamidis

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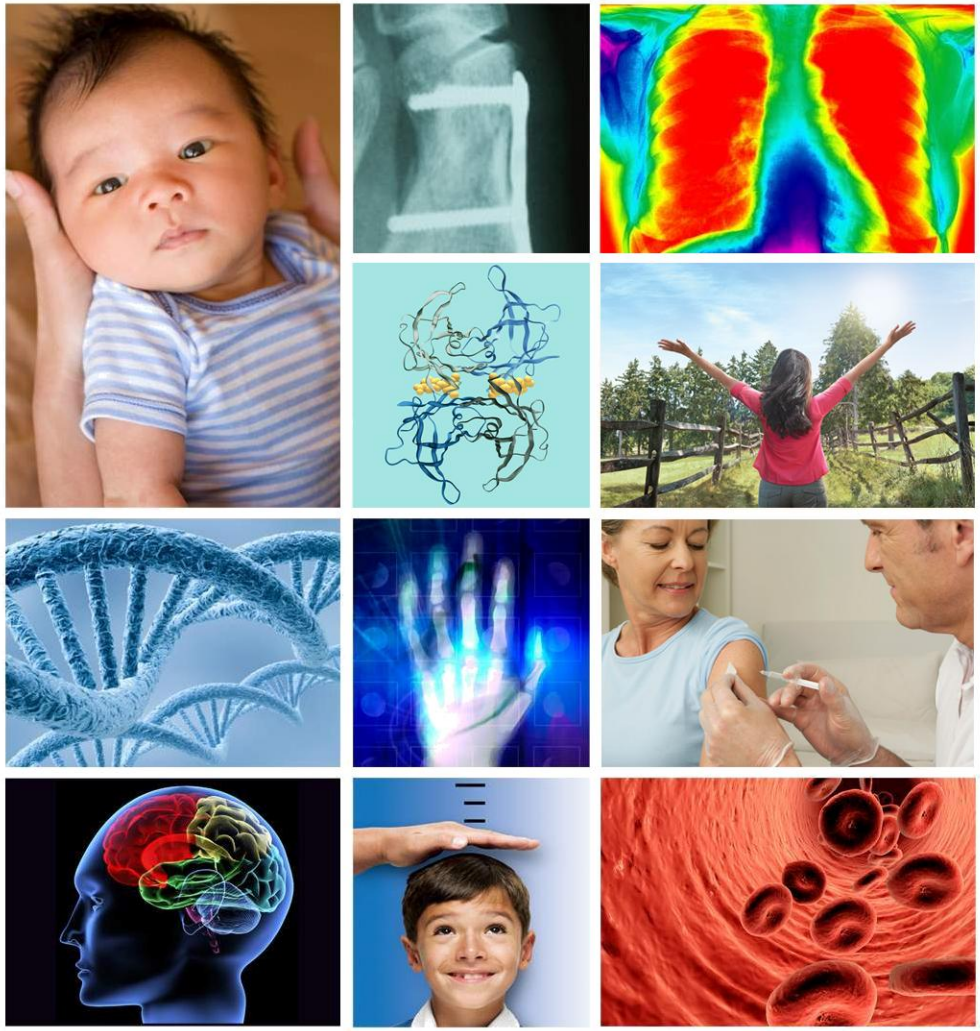
Global Medicines Development Lead
Tafamidis Cardiomyopathy



Amyloidosis Support Groups
www.AmyloidosisSupport.com

Tafamidis Overview

- Status of discussions with FDA regarding TTR-Polyneuropathy (TTR-FAP)
- New collaborative research initiatives
- Update on tafamidis in Europe and Japan
- New clinical study with tafamidis for TTR-Cardiomyopathy (TTR-CM)

- **FDA has specific rules/regulations around interactions with patients and patient groups**
- **One rule deals with an industry representative presenting safety or efficacy information around an agent that is still under review in the US (i.e. not approved for marketing)**
- **Commentary could be seen as 'pre-approval promotion'.**
- **Therefore, no tafamidis data/study results will be presented today**

Update
Status of Tafamidis FDA Discussions

- Pfizer is in active discussions with the FDA to find a path forward for approval
  - Our approach is focused on a novel biomarker
  - As discussions are ongoing with FDA, we cannot share specific details

- We are focusing our efforts on obtaining regulatory approval of tafamidis in the US which we believe is the best way to make this medicine available

- Pfizer stands firmly behind tafamidis and is committed to pursuing the development program for the drug in the US

Update
Tafamidis in Europe and Japan

- Tafamidis approval for the EU (European Medicines Agency) granted in November 2011
- Tafamidis is currently available in 13 European Countries
- Clinical experience in individual patients for more than 5 years
- Tafamidis was approved in Japan in September 2013

Pfizer's Continued Commitment
New Study in TTR-Cardiomyopathy Starting This Year

ATTR-ACT™: Transthyretin Amyloid Cardiomyopathy Tafamidis study

- A multicenter, global evaluation of the efficacy, safety, and tolerability of tafamidis in people diagnosed with transthyretin cardiomyopathy

- Largest study of its kind:
  - **Up to 400 patients** to be enrolled with either familial TTR-Cardiomyopathy or non-hereditary (wild-type/senile systemic amyloidosis) cardiomyopathy
    - **Approximately 250 tafamidis treated patients, 150 placebo**
    - **20-25 sites in the US; additional sites globally**
    - **30-month treatment duration**
  - All patients who complete the study will have the opportunity to receive tafamidis treatment in a long-term extension study.

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Thank You. We Look Forward To Continuing Our Dialogue With The Amyloidosis Support Groups.

…representing the Tafamidis Team, located in Groton CT, Cambridge MA, Collegeville PA, Minneapolis MN, New York, NY, and Pfizer offices around the world:

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