Hereditary Amyloidosis Support Conference

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Regulatory Considerations for our Discussion Today

- The pharmaceutical industry is highly regulated.
- FDA has specific rules/regulations around interactions with patients and patient organizations.
- One very specific rule deals with an industry representative presenting safety or efficacy information around an agent that is still investigation in the US (ie. not approved for marketing).
  - Commentary would be seen as “pre-approval promotion”
  - FDA has recently cited two companies for such an issue:
    - One based on commentary on a website
    - Another based on commentary from a clinical trial investigator
- Therefore, my presentation today will be top-line.
Tafamidis Clinical Trial Update (TTR-FAP)

- **Study Fx-005** - completed
  - 18 month DB, PC study of safety & efficacy in 128 patients with V30M TTR-FAP

- **Study Fx-006** – completed
  - 12 month OL extension study of Fx-005 in 86 patients with V30M TTR-FAP

- **Study Fx1A-OS-001** – completed
  - Cross-sectional correlation study of the clinical outcomes measures (NIS-LL & Norfolk QOL-DN) in 51 patients with V30M TTR-FAP and 16 healthy controls

- **Study Fx1A-201** – completed
  - 12 month OL study of safety & efficacy in 21 patients with non-V30M TTR-FAP

- **Study Fx1A-303** – ongoing
  - OL study of 80 V30M patients originally enrolled in the Fx-005 study
US Regulatory Update for tafamidis

- US NDA for tafamidis submitted to FDA in Q1 2011
- Pfizer received Refusal-to-File (RTF) from FDA in Q2 2011
  - Did not provide comment as to acceptability of clinical data
  - FDA determined submission was not sufficiently complete to permit a substantive review
  - Pfizer believes additional information needed to support filing is available without further clinical studies
- Pfizer continues to work towards addressing issues in RTF and is committed to resubmission of the application this year
Pfizer Specialty Care: A Broad In-Line Portfolio Targeting Serious Diseases

Rare Diseases
- Hemophilia
- Transplant
- PAH / PVD

Infectious Diseases
Endocrine
Gastrointestinal

Vaccines
Inflammation
Ophthalmics
Musculoskeletal
Pfizer’s Commitment to the Rare Disease Patient

- **2009**
  - **Protalix Biotherapeutics**
    - 12/01/09

- **2010**
  - **Pfizer adds focus on rare diseases**
    - Cambridge-based group may compete with Genzyme
  - **Ergonex**
    - 05/12/2010

- **2011**
  - **New Rare Diseases Group Anchored in SCBU**
  - **FoldRx**
    - 09/01/10
  - **Zacharon Pharmaceuticals**
    - 04/07/11