Hereditary Amyloidosis Support Conference

D.J. Keohane, MD

Senior Director/Neuroscience Team Lead

Pfizer Specialty Care

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

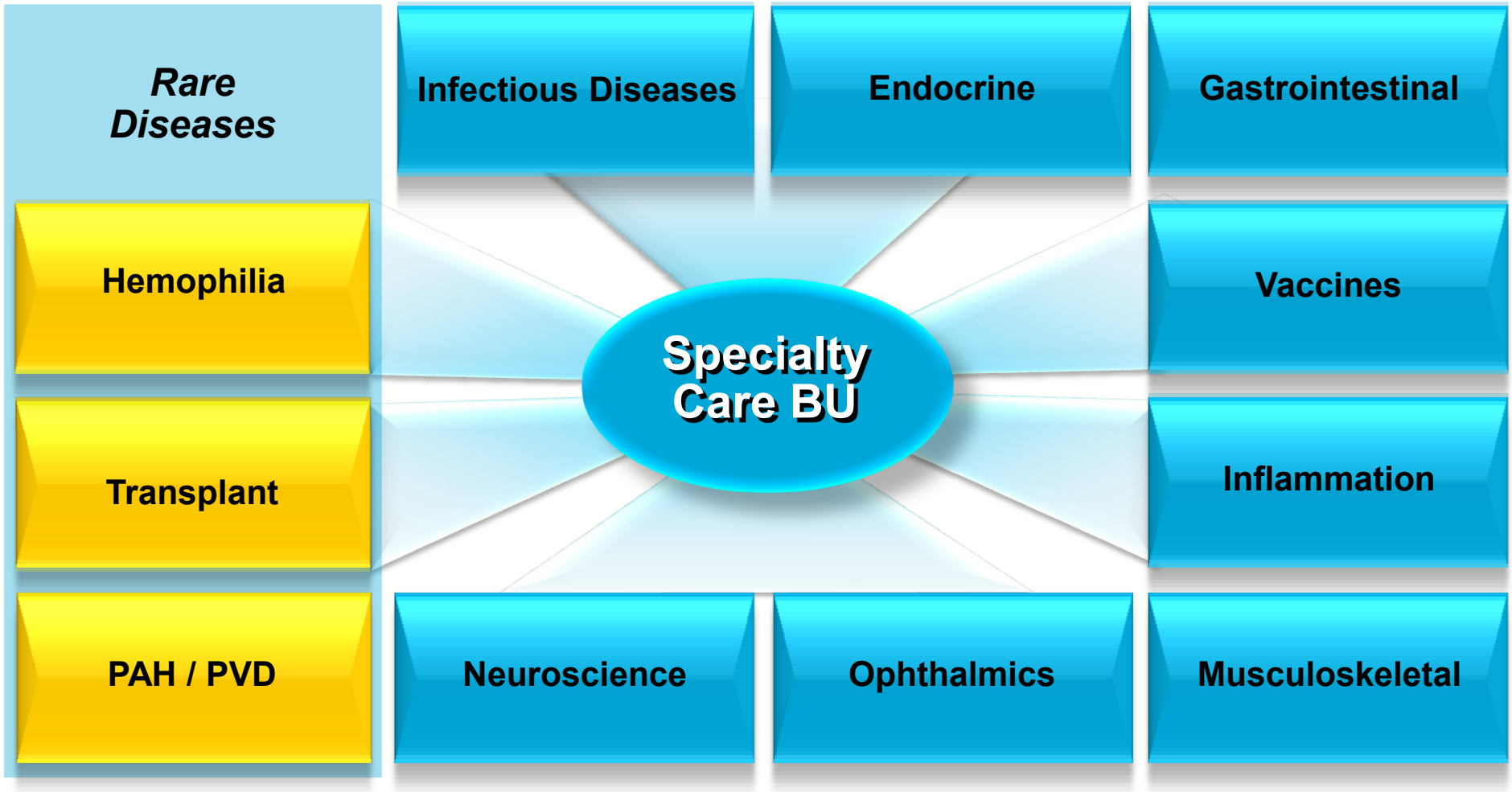
Tafamidis Clinical Trial Update (TTR-FAP)

- 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



Pfizer's Commitment to the Rare Disease Patient