

PPTA Perspective on the FDA Review Process for Orphan Drug Marketing Applications

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Vice President Global Regulatory Policy
FDA Public Hearing
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- International trade and standards-setting organization
- Represents:
 - Source Plasma collection centers and
 - Manufacturers of plasma-derived and recombinant analog therapies, collectively referred to as plasma protein therapies

- Plasma protein therapies
 - are used in the treatment of a number of rare diseases
- These diseases are
 - often genetic, chronic, life threatening conditions
 - that require patients to receive regular infusions or injections of plasma protein therapies for the duration of their lives

Baxter

BioScience



GRIFOLS

CSL Behring

octapharma

Talecris
BIOTHERAPEUTICS



CANGENE



K
KEDRION



Biotest

- Whether FDA's practice of making approval decisions regarding the overall risk benefit profile of therapies for the particular patient population for which they are being considered has adequately addressed the needs of the patients with rare diseases?
 - Federal Register/ Vol. 75 , No. 8 3 ; 4/30/10

- PPTA appreciates agency efforts to review available laws, regulations and policies with focus on facilitating the development of biological products used to treat patients with rare plasma protein disorders
- Recent approvals indicate the agency is taking tremendous strides in providing a new paradigm for review and approval of therapies to treat rare plasma protein disorders

- Harmonization of Clinical Trial Requirements
 - Current regulatory requirements are different
 1. Ex: U.S. v. EU - number of patients required US.
 2. Entails repeating studies on a very small patient population
 - Acceptance of studies conducted abroad (e.g., not under US IND)
 1. Use of existing post market safety and efficacy data
 - Goal: Harmonization of clinical trial requirements

- Clinical Trial Design
 - Clinical Trial Requirements
 - Clinical Trial Design
 - Size of study
 - Recruitment of participants
 - Participant compliance
 - Endpoints
 - Use of surrogates/biomarkers

- Patient Registries
 - Better use of international Registries
 1. Ex: **ESID Database for primary immunodeficiencies**
 - Improve quality of registries
 - Develop standard methodology for registries

- Facilities/Equipment
 - Rare orphan therapies may not be produced as often as other drugs
 - Validated processes may use legacy facilities and/or equipment compared to newer products
 - Need regulatory acknowledgment of acceptability in terms of GMP

- PPTA thanks FDA for holding public hearing and recognizing the uniqueness of developing therapies for rare disease
- PPTA looks forward to continuing to work with FDA on these issues