

Plasma Protein Therapeutics Association (PPTA) Remarks

PDUFA IV Public Meeting

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- International trade and standards-setting organization
- Represents:
 - Plasma-derived therapies (PDUFA)
 - Recombinant analog therapies (PDUFA)
 - Source Plasma (non user fee)

Baxter

BioScience

ZLB Behring



GRIFOLS

Talecris
BIOTHERAPEUTICS



NABI
BIOPHARMACEUTICALS



CANGENE

Bayer 




Ortho-Clinical Diagnostics
a *Johnson-Johnson* company

- Plasma Protein Therapies
 - Regulated by Center for Biologics Evaluation and Research (CBER)
 - Reviewed within Office of Blood Research and Review (OBRR)
- PPTA concerned that CBER and OBRR are resourced adequately

- Transparency in the review process
 - Real-time tracking
 - Improve predictability
- Post-market surveillance
 - Industry input in design

Questionnaire 1

- CMA Pilots 1 & 2
 - No member company participated
- Reasons for nonparticipation
 - Pilot 1
 1. Project not ready
 2. No project that met criteria
 - Pilot 2
 1. Sufficient reviewer access already
- Future user fee funding
 - No
 - Yes, if costs reasonable
 - Yes  promotes quicker approval/consistency of advice

Questionnaire 2

- Performance Goals
 - Timelines should correspond to target approval dates, not FDA response timeline
 - Labeling review should be moved up/last minute changes affect launch dates
 - Reduce cycle times for resubmissions
 - Lot release within 10 days
 - No new goals but better disclosure of funding allocations
 - No existing goals should be dropped

Questionnaire 2 – Special Protocol Assessment

- Not used to date
- May be used in future
- Will not use: inadequate benefit to offset effort
- No need for SPA's
- **CBE – 30 Performance**
 - CBER Performance good but it is time to review criteria for classification

- Support current performance goals with some enhancements, eg. resubmission, labeling reviews, lot release
- Debatable value of PDUFA III, CMA pilots/SPA
- Concerned that CBER funding adequate
- Increased transparency in review process
- Interactive design of post-market surveillance, if funded