Regulatory Perspective on Patient Focused Drug Development in Rare Diseases

Lucas Kempf, MD - Vice President, technical

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What is Patient-Focused Drug Development?

Patient-focused drug development (PFDD) is a systematic approach to help ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation. As experts in what it is like to live with their condition, patients are uniquely positioned to inform the understanding of the therapeutic context for drug development and evaluation.

The primary goal of patient-focused drug development is to better incorporate the patient's voice in drug development and evaluation, including but not limited to:

www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development

4 Goals of Patient Focused Drug Development

- Facilitating and advancing use of systematic approaches to collecting and utilizing robust and meaningful patient and caregiver input to more consistently inform drug development and regulatory decision-making
- Encouraging identification and use of approaches and best practices to facilitate patient enrollment and minimizing the burden of patient participation in clinical trials
- Enhancing understanding and appropriate use of methods to capture information on patient preferences and the potential acceptability of tradeoffs between treatment benefit and risk outcomes
- Identifying the information that is most important to patients related to treatment benefits, risks, and burden, and how to best communicate the information to support their decision making.

“Facilitating and advancing use of systematic approaches to collecting and utilizing robust and meaningful patient and caregiver input to more consistently inform drug development and regulatory decision-making”

- COA guidances, Common issues guidance, Natural History guidance, ect.
- Consistent advice for early engagement with patient support groups
- Patient representatives at AC and now frequently brought to regulatory meetings by the sponsor
- CPIM meetings
- PFDD meetings
- Patient Listening sessions
- NORD MOU
- Use Studies and AE collection-free response
  (angry blogging patients end studies)

“Encouraging identification and use of approaches and best practices to facilitate patient enrollment and minimizing the burden of patient participation in clinical trials”

- Early engagement not only about providing a list of possible trial participants
- Examine SoC and ethically match for pediatric patients
- Conduct Natural History studies to inform trials
- Eligibility criteria guidance emphasizes the trial design considerations to maximize enrollment in small populations, for example 6MW to NORTHSTAR for DMD
- Decentralized trials: when are they appropriate and when are they not due to logistics.
- Travel support, concierge services, Disneyland effect
- Transparent compassionate use programs and criteria

“Enhancing understanding and appropriate use of methods to capture information on patient preferences and the potential acceptability of tradeoffs between treatment benefit and risk outcomes”

- COA validation early
- PFDD meetings now more focused on the context of B/R assessments in specific conditions using proper survey methodology
  - Less Support Group, more Focus Group
  - More internet inclusive, less loudest voice in the room
- For rare diseases need to appreciate the global issues due to variable standards of care, access, risk tolerances, and their effects on trial design and analysis
  - Early plans for stratification; examples pediatric seizures or gene therapies

“Identifying the information that is most important to patients related to treatment benefits, risks, and burden, and how to best communicate the information to support their decision making”

- Embedding from the beginning of the trials methods to collect and analyze
  - Relevant trial endpoints
  - anchoring scales
  - patient surveys, voice of the patient reports
- With compressed, overlapping, or seamless trials, consistent ongoing assessment of risk with communication of risk to patients and updating consents for emergent findings.
- Patient experience data in FDA reviews required
- Early planning for HTA

Thank you