Breakout session feedback

BO4 Trials incorporating historical controls or with adaptive features

EMA Patients United · efpia European Federation of Pharmaceutical Industries and Associations · BARC · PPD · Norwegian Medicines Agency

Key outcomes

1. *Agencies open for discussion for new designs, including incorporation of historical data (or RWD) BUT lot of discussion about management of Type I error, meaning of this in Bayesian context...*
2. *Sources of data need to be assessed in view of the question*
3. *Good practice: look at the question first, then look at the available sources of data and last define the global design and approach*
4. *Need to draw the line between advice, collaborative discussion between regulators and sponsors and approval activities ("there is no pre-approval")*

Potential solutions/call for action

- Interest for learning together from both sides
- Opportunity for a platform to share experiences, maybe not only on a product basis
- Value the good practice: Have the question first, and then have a multistakeholder discussion in order to better address the question. Explain clearly why you are proposing a given approach rather than the "traditional" approach (eg RCT...)
- Ensuring data sharing to avoid unnecessary burden, to improve education, training and re-use of data.
- Need to improve Drug Development using innovation, not only in paediatrics or rare disease area

Breakout Session #5
Implementation & Operational aspects

Chairs: Olga Kholmanskikh (CTFG, FAMHP), Josse Thomas (BAREC)

Three main topics discussed

- Collaborative, competitive and mixed approaches for planning, conducting and reporting of CCTs
- Practical aspects and solutions to transform challenges to opportunities
- Best practices sharing: the way forward

Examples

- **EU PEARL**
  - Build standard templates
  - Integrated Research Platforms
  - Used for multiple disease settings eg: Depression, TB, NASH , Neurofibromatosis
    - A proxy for building new projects

- **STAMPEDE**

  A platform for multiple treatments in PC
  - 7 Pharmaceutical companies
  - 15 years – 5 changes of std of care, handle carefully, plan ahead
  - Work backwards – coordinate with the sites

- **RECOVERY**

Key Topics

- CI responsibilities – Huge and massive
  - Consider Dividing out responsibilities
- Simultaneous and Sequential tasks
  - Flexible staffing – Core and Flex
  - Bigger teams – need more team management
- Laying the foundation - Help mitigate the burden
  - Build standard templates ,
  - Clin Ops best practice tools and checklist
  - Structured Cover letters, Tracking amendments to MP and Intervention specifics
  - Integrated Research Platforms
  - Built for any disease – Depression, TB, NASH , Neurofibromatosis
    - A proxy for building new projects
  - Legal concepts – Liability – CT agreements
  - IP protection

Feed Back

- Planning/Setup
  - Acknowledgement of increased challenges with multiple sub-studies/subprotocols
  - Design include the Why and How questions for communication
- Conduct/execution
  - Mitigate challenges through planning
  - Templates for protocols, communication between stakeholders
  - Anticipation of amendments
- Oversight
  - Consideration of the governance – particularly with multiple assets
  - Understanding between partners, multiple companies
  - Sponsor vs multiple Sponsor (Co-Sponsorship – CTR)
- Recommendations
  - Shared mindsets to all collaborators/stakeholders
  - Real time access to information/communication (without undermining data integrity)

Breakout session feedback

BO session 6

Education and Training

Key outcomes

1. *Complexity of trial is increasing and the need of training is always higher*
2. *Useful material already available but limited to closed communities. Experience sharing and common template helped to streamline and optimize the implementation and operations of complex clinical trials*

→ Whose responsability is it to organise, update and share the learnings/trainings with all key players?

3. *Take time to build 'center of excellence' and to establish the necessary connexions between all the stakeholders*
4. *Importance of the involvement of patient experts at each steps and at each level, especially at a national level*
5. *Need to raise the barriers of education, especially the funding*

Potential solutions/call for action

- Need to develop efficient knowledge sharing platforms (eg peer coaching, discussion groups) between all the key players to share the learnings
- Need to develop trainings with all key players but accessible to everyone (e.g. without languages, exposure or financial barriers)
- Need modular training for CCTs, treating general aspects, as well as targeted training towards specific subgroups of members
- Need to include trainings in the curriculum of medical staff and ensure the access to the appropriate courses