MASTER PROTOCOL REFERENCES & RESOURCES

The articles listed below provide a range of references and resources for learning about the basic overview on the concept of master protocol, adaptive platform trials, basket trials, emerging best practices, and regulatory guidance related to master protocols.

OVERVIEW ON MASTER PROTOCOLS

  • This seminal paper sparked broad interest in master protocol studies. It provides an overview of basic design and operational characteristics of master protocol studies.
  • Available at: https://www.nejm.org/doi/10.1056/NEJMra1510062

  • Provides a detailed overview of advantages and limitations of basket and umbrella designs, as well as examples of adaptive platform studies and master protocols that use a hybrid design approach.
  • Available at: https://pubmed.ncbi.nlm.nih.gov/28177494/

  • Highlights persistent inconsistencies in the description of basket, umbrella, and platform trials. The appendices offer very helpful tools that can be used to support early adopters’ efforts to conduct their own review of master protocol studies.
  • Available at: https://www.sciencedirect.com/science/article/pii/S2451865419301103

  • Provides a through overview of key master protocol trial characteristics, disease characteristics, and global geographic representation. It also highlights the need to strengthen the landscape of existing master trials through ongoing nomenclature development and the development of master protocol studies in diverse global regions and therapeutic areas.
  • Available at: https://trialsjournal.biomedcentral.com/articles/10.1186/s13063-019-3664-1
- Describes the benefits and tradeoffs of master protocol studies for biopharmaceutical companies, including factors that contribute to overall time and cost savings associated with the master protocol approach

- Provides an overview of the benefits and challenges of utilizing the master protocol approach. Additionally, the Leukemia & Lymphoma Society and the Pancreatic Cancer Action Network describe study characteristics and lessons learned from recently developed master protocol studies.

ADDITIONAL ADAPTIVE PLATFORM TRIAL RESOURCES

- Compares and contrasts design and operational efficiencies in platform trials and traditional randomized control trials.
- Available at: https://journals.sagepub.com/doi/abs/10.1177/1740774515626362

- Provides a detailed description of the development of the GBM-AGILE trial and reviews important nomenclature, statistical characteristics, stakeholder engagement considerations, and creative funding strategies relevant to the design and implementation of adaptive platform trials.
- Available at: https://www.hbs.edu/faculty/Pages/item.aspx?num=53315

BASKET TRIAL RESOURCES

- Gives an overview of the overarching goals of basket trials and makes recommendations for how to address key design challenges related to basket trials.
- Available at: https://ascopubs.org/doi/10.1200/JCO.2016.69.9751

- Summarizes the overarching purpose basket trials and provides an overview of key biostatistical considerations to advance the use of genome-driven oncology.
- Available at: https://www.annualreviews.org/doi/abs/10.1146/annurev-med-062016-050343
EMERGING BEST PRACTICES

  - Builds on experiences from NCI Match, Tapur, LUNG-MAP, and Beat AML trials to provide a roadmap of emerging best practices for the design and implementation of master protocol studies in oncology.

  - Summarizes a set of 10 multi-stakeholder, consensus-based recommendations on the design and conduct of complex innovative trials, including master protocol studies. In addition to critical study-level considerations, the authors highlight key capacity building efforts within the broader clinical trial ecosystem that are needed to further the development of master protocol studies.
  - Available at: [https://www.nature.com/articles/s41416-019-0653-9](https://www.nature.com/articles/s41416-019-0653-9)

  - The Adaptive Platform Trial Coalition provides a detailed overview of common features and best practices related to the design, conduct, oversight, and reporting of adaptive platform trials.
  - Available at: [https://www.nature.com/articles/s41573-019-0034-3](https://www.nature.com/articles/s41573-019-0034-3)

REGULATORY GUIDANCE
