MEDICAL UNIVERSITY OF VIENNA

Wiener Biometrische Sektion
der Internationalen Biometrischen Gesellschaft
Region Österreich – Schweiz

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QUYNH NGUYEN

SECTION of DATA SCIENCE AND METHODS, PAUL-EHRLICH INSTITUT

THE USE OF COMPLEX CLINICAL TRIALS – A REGULATORY REVIEW

February 21st, 2024 at 9:00 am

Informatikbibliothek, Center for Medical Data Science,
Spitalgasse 23, Room 88.03.806
Medical University of Vienna, 1090 Wien

HOST: Martin Posch

ABSTRACT

In platform trials, multiple treatment arms are evaluated with the possibility to add or drop treatments during the ongoing trial at different time points. Systematic literature reviews [1,2] have been performed to identify complex clinical trials (which include umbrella, basket and platform trials). An increased use of complex clinical trials has been identified. However, literature and guidance on regulatory acceptance of specific design aspects in complex clinical trials are still limited.
To help overcoming this lack, we performed a systematic review of scientific advice procedures at the European Medicines Agency (EMA) to identify complex clinical trials. We only considered advices on products which are in the remit of the Paul-Ehrlich-Institute (PEI) such as monoclonal antibodies, vaccines, and advanced therapy medicinal products (ATMPs). The database at PEI consisted of more than 50 000 documents covering around 2 500 scientific advice procedures. Following the PRISMA guidance on systematic reviews we identified more than 150 documents representing around 25 unique complex clinical trials for data extraction.
We present preliminary results of our analyses on the design characteristics of the proposed complex clinical trials submitted for scientific advice. General design aspects such as the study phase, the number of treatment and control groups or the primary endpoint will be described. Furthermore, design aspects frequently discussed for complex clinical trials, e.g. the use of a common control, the use of non-concurrent controls or the need for multiplicity control were also evaluated. Where possible and available, we identified the corresponding regulatory acceptance or concerns of these aspects.
[1] Meyer, E.L., Mesenbrink, P., Dunger-Baldauf, C., Fülle, H.-J., Glimm, E., Li, Y., Posch, M. and König, F. (2020). The Evolution of Master Protocol Clinical Trial Designs: A Systematic Literature Review. Clinical Therapeutics, 42(7), pp.1330–1360. doi:10.1016/j.clinthera.2020.05.010.
[2] Park, J.J.H., Siden, E., Zoratti, M.J., Dron, L., Harari, O., Singer, J., Lester, R.T., Thorlund, K., and Mills, E.J. (2019). Systematic review of basket trials, umbrella trials, and platform trials: a landscape analysis of master protocols. Trials 20, 572 (2019). https://doi.org/10.1186/s13063-019-3664-1