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Decision-Making Criteria and Methods for Initiating Late-Stage Clinical Trials from a Multi-Stakeholder Perspective: A Scoping Review

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I am a postdoctoral researcher at Université Paris Cité, where I am collaborating with Prof. Raphaël Porcher in the Personalized Medicine Team of METHODS, CRESS UMR 1153, Inserm. My area of expertise is in statistical machine learning, focusing on Causal Inference in the counterfactual framework (Rubin causal model) and its applications to healthcare. My research focuses on individual and average treatment effects, risk/benefit classification, compliance and use of observational data. This research is a continuation of my thesis work, conducted at LAMSADE, Paris Dauphine-PSL University, under the supervision of Prof. Jamal Atif and Dr. Florian Yger as part of the MILES machine learning team.

I am also involved in Deeptech innovation and entrepreneurship. I am currently studying for a specialized master’s degree EDI at Mines-PSL, which is located in the dynamic ecosystem of Campus PariSanté. I am particularly interested in the role of research labs and startups in the development of healthcare innovation, as well as the ethical and regulatory aspects.

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Dr. Aysun Cetinyurek Yavuz is a senior Researcher at Radboud UMC. She is involved in teaching courses on meta-analysis and clinical trials. She is seconded to Dutch Medicine Evaluation Board as methodology assessor and involved in scientific advice and assessment of market access authorisation requests. She is also involved in More-Europa project. She is providing consultancy to the Cardiology group at Radboud UMC. She is currently the co-chair of the PSI Special Interest Group “Small populations” where they share knowledge on methodological challenges for small populations such as pediatrics and rare diseases. Her interests are around the topics of Bayesian borrowing, use of external data, rare diseases, mixed models and meta-analysis.

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Laura completed her Master of Biostatistics in 2010 and a PhD in Biostatistics in 2015. Laura is currently working as a statistical assessor for the Dutch Medicines Evaluation Board, where she is one of the lead statistical assessors of oncology products. She is co-lead of the Estimands Implementation Group, which has members from the EMA as well as the European National Competent Authorities.

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Kit Roes is Professor of Biostatistics at Radboud University Medical Center Nijmegen (Netherlands) and is chair of the Methodology Working Party of the European Medicines Agency. His research focus is design and analysis of clinical trials, with an emphasis on innovative designs, rare diseases and bridging the gap between clinical trials and real world evidence. His experience includes over 25 years in clinical research in the pharmaceutical industry and academic life sciences, serving clinical research and drug development as expert as well as in different (international) senior management positions.

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Christoph works since more than 30 years as a statistician in the pharmaceutical industry. He is Bayer's expert statistician for Womens' Healthcare and for Health Technology Assessment. Christoph is an EFSPI council member and since over 10 years the regulatory chair of EFSPI. He is also member of several scientific advisory groups for EMA and the European Commission. In his spare time Christoph teaches experimental gynaecology at the Saarland State University and leads the local chapter of the pan-European party Volt.

Christoph's involvement in women's health started when he joined Schering AG in 1996. His Ph.D. thesis was entitled "Development of two methodological guidelines for the evaluation of bleeding patterns – A tool for registration and prescription on the basis of an empirical analysis". Christoph (co-)authored many papers on methodological topics in women's health and he is also a co-developer of several PRO instruments in the field of women's health.

Raphaël Porcher
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Raphaël is professor of biostatistics at Université Paris Cité, a member of the METHODS team of the Center of Research in Epidemiology and Statistics (CRESS-UMR1153), and a chair in the PR[AI]RIE-PSAI Artificial intelligence institute. His research interests are centered on statistical and machine learning methods for personalized medicine and methods for causal inference on treatment effects. He participates or has participated to several European Commission-funded projects.

Julien Tanniou
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Julien is an expert statistical methodologist with extensive experience in biostatistics, clinical trial design and real-world evidence. He is recognised for his contributions to subgroup analyses and statistical methodologies, with numerous scientific publications and presentations. His experience encompasses academia, industry, and regulatory agencies, providing statistical input and methodological support.

Single topic, multi-speaker session, Workshop or Single presentation submission

A single presentation/poster

Single presentation or poster submission

The decision-making process in drug development involves “go/no-go” decisions, particularly at the transition from early to late-stages trials. While the decisions are solely made by drug developers, they must take into account the perspectives of multiple stakeholders—such as regulatory agencies, HTA bodies, payers, patients, and ethics committees—to ensure well-informed and robust decision-making. These perspectives influence key considerations, including resource allocation, risk mitigation, regulatory compliance, etc. To support this process, quantitative methodologies, including Bayesian and hybrid frequentist-Bayesian approaches, have been introduced to improve decision-making. However, these methodologies often do not fully account for the diverse priorities and needs of all stakeholders. This scoping review examines criteria and methods used in decision-making at the Phase II to III transition, with a focus on broadening the probability of success (PoS) concept beyond efficacy alone. Our review explores PoS for different success definitions, such as regulatory approval, market access, financial viability, and competitive performance. Key themes include decision criteria selection, trial design optimization, utility-based approaches, financial metrics, and multi-stakeholder considerations in decision-making. Our findings highlight both the limitations of current methodologies and potential paths forward, including the integration of real-world data (RWD) and advanced analytics. This work provides a foundation for advancing late-stage clinical trial decisions toward a more balanced, data-driven, and stakeholder-aligned approach.