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Going beyond Probability of Success for Early Development studies

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Please provide a brief biography for the Presenting author(s)
Trevor Smart is Senior Lead Statistician for Early Development Statistics at UCB. He works on projects covering clinical development planning, study design and strategy, as the compounds come into the clinic, whilst supporting other statisticians in Early Development. Prior to this Trevor was Head of Early Development Statistics at UCB from 2018, Head of Neuroscience, Early Development Statistics at Lilly 2011 to 2018 and prior to that working within Early Statistics at Pfizer with different roles from 1998 to 2011. Trevor's main focus has been design work appropriate for early clinical studies, including proof of concept studies and the use of biomarkers. Prior to joining the pharmaceutical industry, Trevor was a teacher, and statistician supporting agricultural and environmental research, and has completed degrees at Reading, York and Oxford University.

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

A single presentation/poster

Single topic session or workshop abstracts

Probability of success for confirmatory studies is critical prior to investing in the study, however, for studies in early development, such as a proof of concept (PoC) study, making good decisions is far more important than having success. How can we ensure we make good decisions? Probability of success is of operational relevance in forward planning, but little in terms of strategic relevance. Much more relevant are the probabilities

- the compound is good or good enough, given the PoC is successful and we progress to the next study;
- we progress, given it is a good compound;
- the compound is not good enough, given we progress and
- we progress, given the compound is not good enough.

The compound may work, but not well enough, in which case we don't want to progress it. The probability associated with this, i.e. probability of wrongly progressing, is not what is typically referred to as a false positive, type 1 error, which can therefore be misleading. Similarly, both power, conditional on a single value of truth, and probability of success, integrating across all possible truths through a prior, do not capture what is needed to ensure a good decision is made.

Trevor will show us how we can use operating characteristics, design priors, prior-elicitation and pre-posteriors to help answer these questions. Or at least produce visualisations to enable the team to understand what a PoC can deliver and the compromises we make.