5th EFSPI regulatory statistics workshop: summary of feedback

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Contents

Introduction

This short document summarizes the feedback provided by attendees of the 5th EFSPI regulatory statistics workshop in Basel 12/13th October 2020.

Response

We had 37 attendees complete the feedback questionnaire. The difference in responses in the tables below refers to "Did not attend".

Overall rating

Questionnaire question: *How do you rate the overall quality of the iDMC webinar (0 = useless, 5 = world class)?*

rating	3	4	5	Sum
frequency	2	15	12	29

The median rating was 4.

Questionnaire question: *How do you rate the overall quality of the estimand webinar (0 = useless, 5 = world class)?*

rating	3	4	5	Sum
frequency	1	23	10	34

The median rating was 4.

Most important topic for next year?

Most important
Safety
Subgroup analysis and predictive models
Decentralized trials / role of digital biomarkers
Regulatory acceptance of Bayesian designs
Chances and pitfalls in decision making with real world data in rare diseases
Practical implications of Estimands on study conduct and implications on other stakeholders (e.g. clinical science, operations, data management, programming)
Statistical challenges in biosimilar efficacy trials including estimands.
Challenges in vaccine studies (especially COVID-19 vaccination)
estimands - updated based on further experience
Trial design (adaptive design)
Estimands challenges and examples
Example of studies where the different estimands have different results, and how they were interpreted in the regulatory environment
No idea
Disease modifying drugs clinical trials
Adaptive/platform design
On the experience of discussing estimands with regulatory authorities
n/a
Adaptive Design
Future role of data science in regulatory statistics
Estimands for non-randomized trials - what's next
Involvement of clinicians and clinical assessors
Lessons learned from COVID-19 pandemic
Endpoints in onkology, especially in context of accelerrated approval
Use if synthetic arm in clinical trials
Estimands in phase I (PK) trials
no idea
Complex clinical trial design
Network meta-analysis
Bayesian statistics within clinical trials
Single arm trials
applicability of using RWE data in conjunction with clinical studies
bayesian
Vaccine trial design
Differences between EMA and FDA -in particular, how do you deal with a study where the primary outcome is different in the US and Europe?
single arm trials

Most important
The role of external data (e.g. registries, historical controls)

2nd most important topic for next year?

2nd most important
RWE
Safety
Complex innovative trials
No inspiration
Defining appropriate methods for missing data with estimands, e.g. mmrm and estimands (any changes?)
Estimands for Safety data
Use of RWE in regulatory decisions
Patient reported outcomes - latest developments (SISAQoL, etc.)
time to event endpoints and estimands
Statistical methods to deal with missing data
Casual inference; challenges and examples
Per-protocol population - does it still have value? Does E9 (main text) now need a re-review?
No idea
Clinical trials Results interpretation
Statistical consideration in rare disease development
Sensitivity analyses: What is done in practice in different applications.
n/a
Multiplicity
Estimands in further applications. Safety, non-inferiority, PRO, “RWE”
Covid-19. Some practical examples of RCTs and how the COVID-19 situation was addressed
Case scenarios, breaking down the traditional oncology endpoints and constructing corresponding (varieties of) estimands
Experiences with Platform trials
Combination of devices and drugs
Use and validation of surrogate endpoints
innovative trial designs
no idea
Single arm trials
Clinical Outcome Assessments
Regulatory affairs within clinical trials
Statistical assessment of quality attributes
electronic submission format (CDISC)
Feedback on estimands workgroups
Multiplicity adjustment in platform trials

2nd most important
Methods of dealing with missing data in longitudinal analysis.
RWD/RWE
Estimands - anything new after annother year of implementation?