IBIG Forum: Parma, 29-31 October 2024 (Chiesi Farmaceutici)

29th October 2024 - Pre-forum Courses (9.00 – 17.00)

"Approaches to use historical information for the design and analysis of clinical trials and their applications"

Gaëlle Saint-Hilary (Saryga), Pavel Mozgunov (MRC Biostatistics Unit, University of Cambridge)

The use of historical information in the design and analysis of clinical trials has become increasingly recognized for its potential to enhance study efficiency. This training course aims to provide a comprehensive overview of methodologies for integrating historical data into clinical trials in a robust manner, examples of their application in practice, and to provide the attendees with some hand-on experience of using these methodologies.

Participants will explore the benefits and challenges associated with incorporating historical information, along with the regulatory concerns regarding its impact on decision-making risks. We will focus on borrowing external information using aggregate data on the control arm or information on the treatment difference from previous studies. The emphasis will be placed on Dynamic Bayesian Borrowing approaches. These methods allow the use of historical data as informative priors, and permit to adjust the degree of borrowing given the observed differences between historical and current patient populations, controlling that the impact of historical data. We will also cover alternative approaches to the borrowing and discuss the approaches that use patient-level data.

The course will present real trial examples relevant to pharmaceutical development to illustrate these methods at different stages of drug development. Participants will apply these approaches to data, assessing their impact on trial's operating characteristics, and understanding their implications for decision-making.

By the end of the course, attendees will:

- Understand the foundations and practical applications of borrowing historical information in clinical trials.
- Learn how to implement these methods using R.
- Appreciate the considerations necessary to minimize bias and ensure the relevance of borrowing historical data to new trials.
- Engage in discussions about future developments and the potential for these methods to optimize the clinical trial process.

This course is designed for statisticians who wish to deepen their understanding of integrating historical data into trial design and analysis, ultimately aiming to improve the efficiency of clinical trials. The (gentle) introduction to both Bayesian analysis and programming in R will be provided. Attendees are required to bring their laptops for the course.

Optimizing the Programming of ADaM CDISC Datasets for the Implementation of Estimands Framework: "A First Course on the E9(R1) Estimands Framework being implemented on Data Standards"

Marian Mitroiu (Biogen), Cedric Davister (Merck KGaA)

Objective: To understand the complexity of applying the ICH E9(R1) estimands Addendum through CDISC standards, this course intends to provide you with some knowledge and practical skills needed to implement the estimands framework on data standards.

Overview: Based on the white paper 'Implementation of ICH E9(R1) Estimands Framework using Data Standards'[3] developed by a PHUSE working group, gain an understanding of the E9(R1) framework and its objectives, aligning trial planning, design, conduct, analysis, and interpretation based on an illustrated example presented in the white paper.

Practices: Learn some possible practices for incorporating the E9(R1) framework principles into data tabulation (SDTM) and data analysis (ADaM) data packages.

Illustrated Example: Work out from an illustrated example [4] with two estimand constructs that showcase the practical application of the recommendations, providing some actionable insights.

The workshop/course is organized in two parts:

A	B
Introduction to PHUSE white paper	**Introduction to CDISC data structures to accommodate for:**
Introduction to estimand framework	**Intercurrent events**
introduction to the estimand illustrated example	**Strategies for intercurrent events**

30th October 2024 – Forum Day 1

9.00 – 9.30	**Registration**	
9.30 – 9.45	**Welcome & Introduction**	IBIG Steering Committee
Innovation in statistics		**Marco Costantini Veronica Sciannameo**
9.45 – 10.15	**AI-Generated Synthetic Patients: A Step Towards Efficiency?**	Paola Berchialla
10.15 – 10.45	**Patient Preference Studies**	Ileana Baldi
10.45 – 11.15	**Coffee Break**	
11.15 – 11.45	**Estimating the bridging effect of a novel vaccine clinical assay using a causal inference framework**	Meike Adani
11.45 – 12.15	**A conflict-adaptive prior Effective Sample Size measure**	Silvia Calderazzo
12.15 – 12.45	**Speed poster presentation**	
12.45 – 13.45	**Lunch**	
13.45– 14.15	**Personalized treatment selection via product partition models with covariates**	Matteo Pedone
14.15 – 15.00	**A statistician's view of personalised medicine**	Stephen Senn
Statistics & Regulatory		**Giulia Zigon**
15.00 – 15.30	**An update on estimands implementation and future work**	Khadija Rerhou Rantell
15.30 – 16.00	**Coffee Break**	
16.00 – 16.30	**Predicting safety signals from blinded clinical data: the Bayesian Detection of potential Risk using Inference on Blinded Safety data (BDRIBS) framework**	Luca Grassano
16.30 – 17.00	**Dual criteria approach to early conditional approval allowing for inclusion of historical information with application in time-to-event group sequential trial**	Marco Ratta

31st October 2024 – Forum Day 2

Design and analysis of early phase studies		**Daniele Bottigliengo Elisa Rizzo**
9.30 – 10.00	**U-DESPA: a Utility-based Bayesian approach for dosage optimization handling PK, PD safety and efficacy in oncology clinical trials**	Pavel Mozgunov
10.00 – 10.30	**Extending the Bayesian Logistic Regression Model integrating non-DLT adverse events**	Luca Genetti
10.30 – 11.00	**Estimand framework and handling intercurrent events in pharmacokinetics studies: recent experiences and regulatory inputs**	Lorenzo Legramandi
11.00 – 11.30	**Coffee Break**	
11.30– 12.00	**Patient-oriented response-adaptive designs based on a novel information measure in multi-arm trials with quantitative endpoints**	Gianmarco Caruso
12.00 – 12.30	**Concentration-QTc modeling for risk assessment of QTc interval prolongation: opportunities and challenges**	Giovanni Smania
12.30 – 12.40	**Closure**	IBIG Steering Committee
12.40 – 14.00	**Lunch**	

Pre-Forum Course	*Before 31 July*	*After 31 July*
SIMeF members	200€	300€
Non members	300€	400€
IRCCS/No Profit/Public Institutions	200€	300€
Students*	100€	150€
Forum		
SIMeF members	400€	500€
Non members	500€	600€
IRCCS/No Profit/Public Institutions	400€	500€
Students*	150€	250€
20% off for registration to both course and Forum (not applicable to students) *20% off for attendees presenting a poster (not applicable to students)* *25% off for registration to both course and Forum and poster presentation (not applicable to students)* *Poster discount is only applicable to the first author.*		

***Students are those who have no paid professional activities. Employees or freelancers who are also studying are not eligible for students discounted fee.**