Basel Epidemiology Seminar

Please take note of the agenda for the upcoming

Basel Epidemiology Seminar on

Methods for rare diseases, small or special populations

Date and time:
June 13th, 2024
13:30-17:30pm CET
Followed by Apero

Venue:
Novartis Pharma AG & Hybrid meeting
Auditorium U2 in building Fabrikstr. 6
Novartis Campus, Basel

Registration:
This hybrid Seminar is free of charge.
Please register here (**by 10th June for in-person & by 12th of June for virtual attendance**)
https://forms.gle/P6FnhKUVTKRRyXWw6
If you have any technical issues in registering, please contact baselepiseminars@iqvia.com

(For those attending remotely, we will send you a link to join the meeting after your registration and shortly before the meeting date.
For those attending in-person further details will be provided shortly before the meeting date)

Basel Epidemiology Seminar (BES) is an independent, non-profit forum for discussion between epidemiologists. More information can be found here: *https://baselepiseminar.github.io/home/*

Agenda

13:30 – 14:00	**Arrival** (Opportunity for coffee (self paid) & chat in the cafeteria of the same building)
14:00 – 14:10	**Welcome from the BES organizing committee** Sigrid Behr (Novartis)
14:10 – 14:40	**Introduction** **Rare diseases: Challenges to generate Real-World Evidence** Daniel Rosenberg (Johnson & Johnson)
	Overview of the regulatory landscape for development of medicines intended for rare diseases Keith McDonald (IQVIA)

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14:40 – 15:30 **Strategies for comparative studies**
Multi-regional, long-term PASS using mixed data sources
Laura Girardat-Rotar (IQVIA)

Collaborate to innovate: iptacopan and RWE in PNH treatment
Jilles Fermont (Novartis)

15:30 – 15:45 **Break**

15:45 – 16:45 **Strategies for comparative studies - continued**
Challenges for comparative designs in rare diseases
Audrey Muller (Johnson & Johnson)

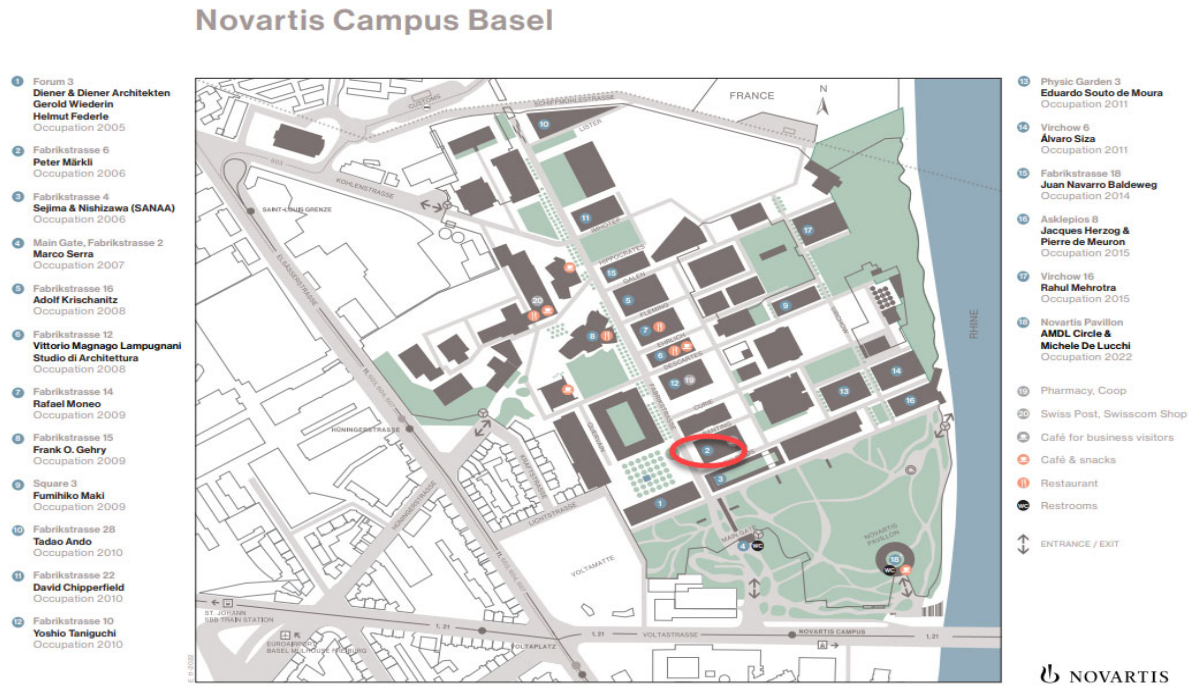
Exploring the Applicability of the Prevalent New User Design in Rare Diseases
Maria Luisa Faquetti (ETH Zurich)

16:45 – 17:30 **Discussion & Closure**

17.45 – 18.45 **Apéro - location VoltaBrau (in person seminar attendees)**

VoltaBräu,Voltastrasse30, CH-4056 Basel **https://www.voltabraeu.ch/**

Location of seminar - Auditorium U2 in building Fabrikstr. 6 -on Novartis campus:



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Biographies & Outlines

Daniel Rosenberg

Daniel has worked as a pharmacoepidemiologist in the pharmaceutical industry in both large and small companies for over 20 years. Daniel started his career with a Post Doctoral fellowship under the direction of Prof. Dr. Harry Guess in Pharmacoepidemiology at University of North Carolina, followed by nearly a decade at GlaxoSmithKline. Daniel founded the Epidemiology group at Actelion pharmaceuticals and led this group until Johnson and Johnson's acquisition of Actelion in 2017. In his present position at J&J within the Global Epidemiology department, Daniel leads a Cross Therapy Area Group of different Disease Area Epidemiology Teams and the Rare Disease Epicenter team. In addition to his interest in rare diseases, Daniel continues his work to shape and develop the use of RWE in the regulatory setting, and currently serves as the EFPIA lead representative to the ICH M14 Expert Working Group who are developing a harmonized guidance on the use of real-world data in pharmacoepidemiology studies for safety assessment. Currently, Daniel chairs the Rare Disease Special interest group (SIG) within the International Society of Pharmacoepidemiology.
In his introductory talk, Daniel will explore themes specific to design and data source challenges in the generation of Rare Disease Real-world Evidence in the context of the framework for generating adequate evidence using fit-for-use relevant and reliable real-world data.

Keith McDonald

Keith McDonald is head of drug development strategy within IQVIA Regulatory Affairs. This group provides support and advice for early phase development from indication prioritization through clinical development planning, study design optimisation and regulatory strategy. Prior to joining IQVIA in 2021, Keith was deputy director of Licensing Division at MHRA with operational responsibility for clinical trials and new medicines approval in UK along with the MHRA Innovation Office, the Early Access to Medicines Programme and was a steering group member for the Innovative Licensing & Access Pathway. He was MHRA delegate at the Coordination Group on Mutual Recognition and Decentralised procedures and vice chair from 2016-18.
In his presentation Keith will provide an overview of the regulatory landscape for development of medicines intended for rare diseases including some recent developments.

Laura Girardat-Rotar

Laura Girardat-Rotar is a trained Epidemiologist working at IQVIA with more than 10 years of working experience gained within pharma, Academia and CRO. She has strong experience managing and overseeing large regulated studies for both HEOR (e.g. external comparators, BoD) and Regulatory (e.g. PASS) purpose across various TA's including rare diseases. She holds a PhD in Epidemiology & Biostatistics from the University of Zürich and is a former recipient of the prestigious Marie Curie Scientific Fellowship.
In her presentation for BES, Laura will discuss a multi-regional, long-term PASS study design using mixed

data sources. This design is becoming increasingly important (especially for rare diseases). It allows for the generation of on-going evidence on patient safety while helping to improve outcomes and reduce patient burden in the cases when RWE helps answer questions for treatment practice due to 2ary data approach. During her talk, she will provide details of the operational aspects and methodical considerations of working with secondary data to address key challenges throughout the study.

Jilles Fermont

Jilles Fermont is Global RWE & Data Science Lead at Novartis in Basel, responsible for the evidence generation for a range of products. Jilles obtained his PhD in Medicine from the University of Cambridge, and previously held positions in epidemiology and health economics at the Universities of Oxford and Harvard, the National Institute of Public Health, as well as the Dutch Ministry of Health where he led the RWE activities.
In this talk, we will explore opportunities and challenges surrounding study design and setup, data extraction and statistical methodology of an external control arm study in paroxysmal nocturnal hemoglobinuria. I will underscore the importance of collaboration across various analytical domains and with external partners and discuss the potential impact with both payers and regulators.

Audrey Muller

Audrey Muller has worked as a pharmacoepidemiologist in the pharmaceutical industry for the last 16 years. Prior joining industry, she was an epidemiologist in the French national institute for medical research (INSERM) for 8 years. In her present position at J&J within the Global Epidemiology department, Audrey is leading the Cardiopulmonary Therapy Area Epidemiology team and is based in Basel.
In her talk she will discuss in the context of rare diseases how performing robust comparative effectiveness analyses is challenging. The talk will focus on optimization-based techniques in small cohorts of patients with unbalanced characteristics at baseline.

Maria Luisa Faquetti

Maria Luisa Faquetti is a trained pharmacist and pharmacoepidemiologist currently working as a postdoctoral researcher at the Pharmacoepidemiology group at ETH Zurich. Her research focuses on investigating drug safety, specifically related to drug-drug interactions.
In her presentation at BES, Maria Luisa will provide an overview of the prevalent new-user (PNU) design, which is becoming increasingly popular for studying the effects of medications using real-world data. Introduced by Sami Suissa in 2017, the PNU design is typically used to assess comparative drug effects in studies where incident new users are scarce, and it allows the inclusion of the clinically important subgroup of patients switching from an older treatment to a newer study drug. During her talk, she will share insights and methodological considerations on the prevalent new user design using case examples, and she will discuss its potential applicability and challenges in rare diseases.