

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

13:30-17:30 CET

Venue:

Novartis Pharma AG & Hybrid meeting

Auditorium U2 in building Fabrikstr. 6

Novartis Campus, Basel

Registration:

This Seminar is free of charge. However, please register here (by 10th June for in-person attendance): <https://forms.gle/P6FnhKUVTKRRyXWw6>

(For those attending remotely, we will send you a link to join the meeting after your registration and 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

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| 13:30 – 14:00 | Arrival (Opportunity for coffee & 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) |
| 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)
- Prevalent new-user design**
Maria Luisa Faquetti (ETH Zurich)
- 16:45 – 17:30** **Discussion & Closure**

Location of Fabrikstr. 6 on Novartis campus:

Novartis Campus Basel

