



**University
of Basel**

Faculty of
Medicine



Small clinical trials, big opportunities!

**Continuing Education Series:
Frontiers in Drug Development**

**Biozentrum,
Spitalstrasse 41,
4056 Basel**
Thursday, 27 June 2024
08:30 – 16:15

ECPM[®] 
European Center of Pharmaceutical Medicine

**CONTINUING
EDUCATION**

Under the auspices of
EUCOR, the European Campus
IFAPP, Int. Federation of Associations of Pharmaceutical Physicians
and Pharmaceutical Medicine

Accredited by SwAPP/SGPM and FPH SPITAL
Recognized as PharmaTrain Centre of Excellence



Invitation

In the realm of drug development, small clinical trial populations hold immense promise despite their size. These trials present unique opportunities for innovation and significant discoveries that ultimately improve patient outcomes. We aim to cover a comprehensive agenda tailored to address the specific needs and complexities inherent in conducting small-scale clinical trials, with several examples in the context of rare diseases. Topics to be discussed include the design of such trials, regulatory considerations, patient engagement and recruitment strategies, data collection and analysis methodologies, as well as case studies and success stories.

Small clinical trial populations are often linked with limited patient populations, necessitating collaboration with patient

advocacy groups and healthcare providers to identify suitable individuals. Ethical oversight and regulatory compliance are essential components of conducting small clinical trials for rare diseases. Drug developers must engage in transparent and collaborative relationships with regulatory bodies to navigate the complex regulatory landscape effectively. Certainly, statistical power is another crucial consideration in the design and execution of small clinical trials. By addressing statistical considerations, we aim to equip attendees with knowledge and tools necessary to design and interpret studies effectively.

We look forward to meeting you and your colleagues on 27 June, 2024 for a comprehensive look at small clinical trial populations!



A handwritten signature in black ink, appearing to read 'Thomas Szucs'.

Prof. Dr. Thomas D. Szucs
Director



A handwritten signature in black ink, appearing to read 'Annette Mollet'.

Dr. Annette Mollet
Head of Education & Training



A handwritten signature in black ink, appearing to read 'Nastazja Laskowski'.

Nastazja Laskowski
Course Director

Small Clinical Trials, Big Opportunities!

Chairperson: Annette Mollet & Thomas Szucs

08.00 – 08:30

Registration for external participants

08.30 – 10:00

What can we Learn from Space with N=1 Trials?

Marcel Egli, University of Lucerne, Lucerne

Individualised Therapies for Rare Individuals

Karen Cheng, Rare Disease Patient Advocate iMyoS and MSU, Basel

10:00 – 10:30

Coffee Break

**Legal and Regulatory Issues in Rare Diseases:
off label and compassionate use**

Thomas Szucs, ECPM, University of Basel, Basel

**Statistical Considerations and Study Design for Small
Populations**

Marc E. Buyse, IDDI & Cluepoints, Louvain-la-Neuve, Belgium

12:00 – 13:00

Lunch

Rare Disease Research in Big Pharma

Paulo Fontoura, F. Hoffmann – La Roche, Basel

**Art & Science of Developing an Ultra Rare Compound
in Small Biotech**

Djordje Filipovic, AB2Bio, Lausanne

14:30 – 14:45

Coffee Break

**Recommendations for Design of Small
Population Trials**

Richard Jones, Hemay Pharmaceuticals, Naples, USA

**Regulatory Aspects for Rare Disease and Orphan
Drug Trials**

Thomas Schochat, SwissMedic, Berne

About Frontiers in Drug Development Seminars

ECPM offers one day seminars on new trends and developments in drug development science. These seminars provide the opportunity to integrate work and further education, to discuss with experts face-to-face and to build an international network. They take place on the fourth day of each of the six modules of the ECPM Diploma Course and are mandatory for students taking the Diploma Course. Additionally they are open to our alumni and other interested scientists and can be booked separately.

Learning Outcomes

Participants will learn about:

- Gain an understanding of the unique opportunities and potential discoveries associated with small clinical trial populations.
- Learn strategies for addressing the specific needs and complexities inherent in conducting small clinical trials, including design considerations and regulatory compliance.
- Explore real examples illustrating successful approaches to designing, executing and interpreting small clinical trials, with a focus on improving patient outcomes and advancing therapeutic innovation.

Date and Venue

Thursday, June 27, 2024
University of Basel
Biozentrum
Spitalstrasse 41
4056 Basel
Switzerland

Registration

Via our homepage www.ecpm.ch or
www.ecpm.ch/frontiers-in-drug-development-s3

Deadline for Registration: June 17, 2024

Credits

Six workshops over a period of two years, which equal 1 ECTS credit. Accredited as continuing education with eight credits by the Swiss Society of Pharmaceutical Medicine (SGPM) and the Swiss Society of Pharmaceutical Professionals (SwAPP). Accredited with 50 FPH-points in hospital pharmacy and 50 FPH-points in clinical pharmacy by the Swiss Society FPH Spital (Fachgesellschaft FPH Spital).

Fee

Course fee including certificate and electronic course material is CHF 580 for ECPM Alumni and CHF 480 for SwAPP/SGPM members. A reduced fee of CHF 210 applies to participants from academia and nonprofit organizations.

After registration, you will receive an invoice. For short notice registration, attendance can only be guaranteed if the seminar fee is paid onsite by credit card.

Cancellation Policy

Refund of fee will be given if cancellation is received in written format (ecpm@unibas.ch) before the deadline for registration, after this date no refund can be given.

Seminar Organizer

This course is organized by the European Center of Pharmaceutical Medicine.

**Educating
Talents**
since 1460.

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ECPM
Institute of Pharmaceutical Medicine
Klingelbergstrasse 61
4056 Basel
Switzerland

www.ecpm.ch

