SYMMETRIC

# Orphan Drugs Clinical Trials

International Training Course

ONLINE TRAINING Available

8 - 9 October 2020
Radisson Blu Edwardian, Kenilworth
London, UK

# Course description

The course presents a unique blend of Orphan Drugs regulatory guidance and clinical trials strategies. The participants will explore the legal basis for approvals, learn how to design a study and which methodology to use. Evidence-Based Medicine data analysis, conclusions, and reporting will be thoroughly discussed.

# Key topics

- Orphan Drugs EU and US regulatory guidance
- Legal basis for approvals (Regulatory pathways) & Working Groups
- Trial concept, design and conclusions
- The Importance of Precision and Confidence Intervals
- Study designs and methodology considerations
- What constitutes convincing evidence?

# Your trainers

**Dr. Simon Day**
CEO
Clinical Trials Consulting & Training

Simon has spent 30 years working in clinical trials, mostly in the pharmaceutical industry but also including five years at the UK and European regulatory agencies. He now works as a statistical and regulatory consultant to pharmaceutical and biotechnology companies around the world. He specialises in training and consulting on drug development programmes, scientific advice/ end of Phase II meetings and preparations for oral explanations and advisory committees. He is particularly well known for his work in the area of developing treatments for rare diseases.
He is a former president of the International Society for Clinical Biostatistics. He is joint editor of Statistics in Medicine, on the editorial board of Translational Sciences of Rare Diseases, and has previously served on many other editorial boards. In 2012 he was elected a Fellow of the Society for Clinical Trials. He has published widely in statistical and medical journals, is author of one book "Dictionary for Clinical Trials" and is joint editor of the "Textbook of Clinical Trials", both published by Wiley.
Simon has served on a variety of data monitoring committees both for industry- and government-sponsored trials. He is chairman of the External Advisory Panel for the Department of Statistics at Oxford University and an Associate on the faculty at Johns Hopkins University in Baltimore. He formerly served as vice-Chairman of the West London Research Ethics Committee. He has given numerous lectures and courses on statistics and clinical trials all around the world, including courses at the FDA on development and regulatory assessment of orphan drugs.

# Who should attend?

- Rare Disease Specialists
- Clinical Research Associates
- Clinical Operations Analysts
- Clinical Diagnostic Specialists
- Pharmacovigilance Scientists
- Regulatory Managers and Specialists
- Process Science Research & Development
- Drug Product Development Scientists
- Orphan Drug Products Formulators
- Quality & Compliance Associates

# Key takeaways

- ✓ EMA and FDA expectations for Orphan Drugs clinical studies
- ✓ The Importance of avoiding Bias and Regression to the Mean
- ✓ Working with Evidence Based Medicine and Clinical trials
- ✓ How to design a study and which methodology to use?
- ✓ Practical implementation of regulatory pathways
- ✓ Where do we need randomised trials?
- Training materials will be provided in printed and digital form.
- All delegates will receive printed and LinkedIn certificates.
- 5 hours of networking with industry peers


# In-house training

Would you like to bring this training to your company? In-House Training Courses are the most cost-effective and efficient way to train your team.

Contact us

# Day 1

8 October 2020

**08:30** *Registration*

**08:45** *Meet & Greet session*

**09:00** **An introduction to Evidence Based Medicine and Clinical Trials**

**09:45** **Summary of Orphan Drugs EU and US regulatory guidance documents, the thinking behind them and their practical implementation**

**10:30** *Morning coffee*

**11:00** **Summary of Legal basis for approvals, Working Groups and Sources of Information**

- Legal basis for approvals (Regulatory pathways)
  - Full
  - Conditional
  - Exceptional circumstances
  - Accelerated approval
- Summary of recent Working Groups
  - InSPiRe
  - ASTERIX
  - IDEAL
  - IRDiRC
  - FDA Science of Small Clinical Trials
- Useful sources of information
  - IRDiRC
  - EURORDIS
  - Orphanet
  - GARD

**12:00** *Lunch*

**13:30** **The Importance of Avoiding Bias and Regression to the Mean**

- Trial concept
- Trial design
- Data analysis
- Conclusions and reporting

**15:00** *Afternoon coffee & snacks*

**15:30** **The Importance of Precision and Confidence Intervals**

- Selection of endpoints
- Use of stratification
- Covariates and analysis strategies

**17:00** *Networking drink*

**18:00** *End of Day 1*

# Day 2

**9 October 2020**

**09:00** **Study designs and methodology considerations**

- Single arm; control groups
- Historical controls and natural history studies
- Group sequential and adaptive designs
- Multiple endpoints and multiplicity; composite endpoints
- N-of-1 studies
- Standards of evidence (P-values; 1- and 2-sided)
- Bayesian approaches
- Registries

**10:30** ***Morning coffee***

**11:00** **What constitutes convincing evidence?**

- The problems of not starting randomised trials
- Levels of evidence
- Grades of recommendation
- Where do we (and don't we) need randomised trials?

**12:00** **Closing discussion / Q&A session with examples from delegates**

**12:30** ***Lunch & Networking***

**14:00** ***End of Day 2***

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Click here for FAQ

# BOOKING FORM

To register your place, complete this form and email it to: **trainings@symmetric.events**

**OR CLICK HERE TO REGISTER ONLINE**

**BOOKING LINE: +421 2222 005 43**

SYM 030

| TRAINING TITLE: | DATES | NUMBER OF DELEGATES | | |
|---|---|---|---|---|
| Orphan Drugs Clinical Trials | 8. - 9. 10. 2020 | | **PUBLIC TRAINING** (EUR) **2.098,- €** | ☐ |
| | | | **ONLINE TRAINING** (EUR) **1.350,- €** | ☐ |

## DELEGATE DETAILS:

| Name | Job Title | Email contact |
|---|---|---|
| 1. | | |
| 2. | | |
| 3. | | |

## COMPANY/ORGANISATION DETAILS

Company Name: _____

Street Address: _____

City, Postal Code: _____

Country: _____

Contact person: _____

VAT: _____

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## AUTHORIZATION AND ACCEPTANCE OF CONTRACT & TERMS & CONDITIONS

Name: _____

Date: _____

Signature: _____

Booking is invalid without a signature

## PAYMENT METHOD

☐ Wire/Bank transfer
- Send me the invoice

☐ Credit/Debit card
- Send me the payment instructions

On behalf of Symmetric Ltd., I hereby declare Symmetric Ltd. will adhere to this sales contract & terms & conditions

Mliekarenska 9, 821 09 Bratislava, Slovak republic
Registration. no. : 47068124, VAT no.: SK2023741973

Attila Molnár, Director

## TERMS AND CONDITIONS:

These terms and conditions represent an integral part of this contract between SYMMETRIC, s.r.o. representing of Industry 4.0 Insights brand and a client, which is specified above, and who by signing this Registration Form fully and without any doubt or uncertainty accepts all following terms and conditions:

1. **Payment Terms.** SYMMETRIC, s.r.o. (hereinafter only "SYMMETRIC") requires the full payment of the invoiced amount within 14 days from the issue date of the invoice. In case your internal policy does not allow you to sign contract with a 2 week due date, you can ask your key account manager for an individual exception. Exception needs to be made in a written form. SYMMETRIC reserves the right to refuse entry to any client who does not pay the invoice in full and on time. The registration fee includes: training documentation, admission to all training sessions, lunches and refreshments. The registration fee does not include travel, hotel accommodation, transfers or insurance.
2. **Hotel Accommodation.** Overnight accommodation is not included in the registration fee. Registered delegates will be provided with a list of accommodation options near the event. Assistance is provided by SYMMETRIC upon request.
3. **Cancellation by The Client.** The client has the right to cancel his/her participation in the event. Cancellation must be received by SYMMETRIC in writing, either by post or email.
   - One month before the event commencement date, SYMMETRIC shall be entitled to retain and charge 50% of the total invoiced amount.
   - If the client cancels less than one month before the commencement date, or fails to attend the event, then the client shall not be entitled to any refund nor discount.
   - The client's failure to attend the event does not cancel, decrease or in any matter waives the client's obligation to fully pay the fee invoiced to the client by SYMMETRIC.
   - The client can transfer once on a "space available" basis at no extra cost, until 4 weeks prior to the event, to another course held within one year of the original course date.
4. **Cancellation by Symmetric.** If SYMMETRIC must cancel a course due to exceptional circumstances, SYMMETRIC will issue a credit note or a full refund. However, SYMMETRIC will not be responsible for any airfare, hotel or other costs incurred by registrants.
5. **Force Majeure.** Neither party shall be liable for any costs or damages due to delay or changes under the following terms & conditions. These include, but are not limited to, acts of terrorism, acts of war, illness, natural disasters and strikes. Under these circumstances SYMMETRIC reserves the right to postpone dates, substitute trainers, change venue or move the training into online format. SYMMETRIC will not be responsible for any airfare, hotel or other costs incurred by registrants.
6. **Governing Law.** This contract shall be governed and construed in accordance with the laws of the Slovak Republic (not including its conflict of laws provisions).
7. **Participant Names.** Names of participants can be changed by the Client up to 48 hours prior to the event beginning, at no additional cost.