

*The European Statistical Forum  
is pleased to announce its 8th Annual Conference:*

# Statistical Methods for rare diseases and special populations



Barcelona, Spain  
**H10 Marina Barcelona**  
Av. Bogatell, 64-68 - Barcelona

Monday,  
November 13th, 2017

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## Statistical Methods for rare diseases and special populations

The development of treatments for rare diseases has been largely boosted by several initiatives from regulatory agencies. For example, the FDA has initiated its rare diseases program with the objective “to facilitate, support and accelerate the development of drug and biologic products for the benefit of patients with rare disorders”. In September 2016, an EU-US collaboration to boost medicine development for rare diseases was announced to achieve a common understanding of each Agency’s regulatory approaches to rare diseases drug development.

Furthermore, special populations like most prominently pediatrics have been promoted as a field of unmet medical need and for further investigations. Also on this topic agencies have initiated programs to enhance research. The EMA initiated the Pediatric regulation in 2007 which led to the establishment of the Pediatric Committee with the objective “to ensure that medicines for use in children are of high quality, ethically researched and authorized appropriately and improving the availability of information on the use of medicines for children”.

One common feature for both areas is the limited availability of patients. Therefore, there is an increasing burden in defining a proper trade-off between the feasibility of these trials and the application of traditional statistical approaches for confirmatory regulatory purposes. This challenge has an increased demand on innovative methods and approaches. Techniques to be mentioned in this context are adaptive designs, meta-analysis, enrichment, randomized withdrawal, Bayesian statistics and use of historical data. Also, decision making under uncertainty and in particular extrapolation play a key role here.

The eighth edition of the European Statistical Forum will be dedicated to the topic of “Statistical Methods for Rare Diseases and Special Populations”. Presentations will focus on

- Innovative statistical methods for rare diseases and special populations
- Clinical trials designs, endpoints as well as approaches to evidence synthesis and extrapolation
- Regulatory views on statistical approaches
- Practical examples and case studies

### European Statistical Forum

(ESForum) aims to face statistical topics focusing on the most advanced methodological, regulatory and operational characteristics to support the drug and medical devices development

Thereby, the conference will promote the exchange between experts coming from statistics, medicine, regulatory and academia interested in the field of rare diseases and special populations.

### Scientific board

**Jens-Otto Andreas** - Head Global Statistical Sciences - Bone & New Diseases at UCB Biosciences GmbH

**Lisa Comarella** - Director Biostatistics at CROST NT Group

**Frank Fleischer** - Expert Statistician Methodology at Boehringer-Ingelheim

**Giacomo Mordenti** - Global Biometrics Director at Livaanova

### Who should attend the conference?

The conference is addressed to statisticians, pharmacometricians, physicians, regulators, academia and other experts interested in the field belonging to:

- Pharmaceutical, and Biotechnology companies
- CROs
- Universities/Hospitals
- Academic Research

# Statistical Methods for rare diseases and special populations

## Agenda

- 08:20 - 08:50 **Registration and Welcome Coffee**
- 08:50 - 09:00 **Welcome from the Chair**  
**Jens-Otto Andreas** - Head Global Statistical Sciences - Bone & New Diseases at UCB Biosciences GmbH  
**Lisa Comarella** - Director Biostatistics at CROST NT Group  
**Frank Fleischer** - Expert Statistician Methodology at Boehringer-Ingelheim  
**Giacomo Mordenti** - Global Biometrics Director at Livanova
- 09:00 - 09:40 **Nigel Stallard** - Professor of Medical Statistics, Head of the Statistics and Epidemiology Group and Deputy Head of the Division of Health Sciences at Warwick Medical School  
**Clinical trials in rare diseases; should we do them differently?**
- 09:40 - 10:20 **Yolanda Barbachano** - Senior Statistical Assessor at MHRA  
**Benefit – risk assessments in small populations**
- 10:20 - 10:50 **Coffee break**
- 10:50 - 11:30 **Andy Grieve** - Head of Statistical Innovation Centre of Excellence, Development and Patient Value Practices at UCB  
**Ros Walley** - Statistician at UCB  
**The Use of Historical Data in Assessing the Efficacy of Drugs in Rare Diseases and Paediatric Populations**
- 11:30 - 12:10 **Simon Wandel** - Expert Statistical Methodologist at Novartis  
**Meta-analysis of two studies in rare diseases: what works (and what does not)**
- 12:10 - 13:10 **Networking Lunch**
- 13:10 - 13:50 **Stephen Senn** - Head of Competence Center for Methodology and Statistics (CCMS) - Competence Center for Methodology and Statistics - General Management at Luxembourg Institute of Health  
**Purpose is everything: approaches to analysing n of 1 trials**
- 13:50 - 14:30 **Christopher Jennison** - Professor of Statistics at University of Bath  
**Optimising group sequential designs**
- 14:30 - 15:10 **Gillian Armstrong** - Senior Statistical Scientist at Roche  
**Designing two confirmatory seamless studies for a rare disease clinical development program**
- 15:10 - 15:40 **Coffee break**
- 15:40 - 16:20 **Hendrik Schmidt** - Team Leader of Statistics at Boehringer Ingelheim  
**Statistical considerations in medicines development for orphan diseases**
- 16:20 - 17:00 **Round Table: Current challenges and future of statistics in rare disease and special population**  
**With the contribution of:**  
Gillian Armstrong, Andy Grieve, Christopher Jennison, Stephen Senn, Ros Walley, Simon Wandel
- 17:00 - 17:10 **Conclusions**



## How to reach the conference venue

Venue | **H10 Marina Barcelona** | Av. Bogatell, 64-68 - Barcelona, Spain



H10 Marina Barcelona is 10 minutes walk from the beach and 5 minutes away you have the Olympic Village. Closer to the Metro stop, Bogatell: it is just 2 minutes behind the hotel and can take you into the city centre in 15 minutes, or you could even walk in about 30-40 minutes.

### By taxi

A taxi from the airport should take about 25 minutes. Taxi Fare: €30.00 - €35.00

### By train

The RENFE train leaves the airport every half an hour and will take you to Passeig de Gràcia station, in about 25 minutes. Then you have to change at Passeig de Gràcia for the Yellow Metro for Bogatell and your hotel is 3 minute walk from the metro stop.

RENFE Train Ticket Prices Single ticket: €4.10

### By bus

The Aerobus will also take you from the Airport to Catalunya station. The journey time is around 30 minutes. From Catalunya you need to change for the Green line Metro to Passeig de Gràcia; at Passeig de Gràcia you need to change again for the for the Yellow Metro for Bogatell and your hotel is 3 minute walk from the metro stop. Aerobus Ticket Prices Single ticket: €5.90

### By tube

The closer metro station is Bogatell.

### By car

Coordinates: +2° 11' 32.85" +41° 23' 34.48"

## Pre-Conference Dinner

The dinner will provide an opportunity for you to network informally with your industry peers:

**Barcelona** - Sunday, November 12th, 7:30 PM (the meeting is set for 7:15 PM at the restaurant)

**Restaurant: La Fonda del Port Olímpic** - Moll de Gregal, 7-10 08005 - Barcelona

The price is € 35,00 in addition to the fee for the conference.

Please confirm your interest on the registration form or during the registration online

For further information please visit the [website](#) or contact the organization offices:

**Stefania Sarga** Phone: +39 (0)35.4123594 | Mail: [info@esforum.eu](mailto:info@esforum.eu)

## Sponsorship opportunities

Sponsor the event and take the chance to network with all attendees. Exclusive benefits and high impact branding before, during and after the conference.

If you are interested in sponsoring the event, please write an email to [events@LSacademy.it](mailto:events@LSacademy.it)  
or call us: +39(0)35.515684

[Download the Events Summary 2017](#)

## TWO WAYS TO REGISTER:

- **Online:** please register here <http://esf-2017.eventify.it/orders/new>  
In this case, you can choose to pay by credit card or by bank transfer.
- **By email or fax:** please fill the registration form below for each attendee and send it by email:  
[info@esforum.eu](mailto:info@esforum.eu) or by fax: [+39\(0\)35.4501262](tel:+390354501262) In this case, you can pay by bank transfer.



### REGISTRATION FEE FOR THE CONFERENCE

- **€ 610,00\* Early Bird fee extended until October 30th, 2017**
- € 705,00\* Ordinary fee (after October 30th, 2017)
- € 550,00\* Patronage members fee (Association\_\_\_\_\_)
- € 370,00\* for Academy, Public Administration, Freelance (please, specify\_\_\_\_\_)

\*For Italian companies: + 22% VAT

The fee includes: seat at the conference, copy of presentations of Speakers who allow the distribution, informative literature for the day, welcome coffee, networking lunch, coffee break, organisational office assistance, certificate of attendance.

### REGISTRATION FEE FOR THE CONFERENCE + PRE-CONFERENCE DINNER

- **€ 645,00\* Early Bird fee extended until October 30th, 2017**
- € 740,00\* Ordinary fee (after October 30th, 2017)
- € 585,00\* Patronage members fee (Association\_\_\_\_\_)
- € 405,00\* for Academy, Public Administration, Freelance (please, specify\_\_\_\_\_)

\*For Italian companies: + 22% VAT

The fee includes: pre-conference dinner (Sunday November 12<sup>th</sup>) seat at the conference, copy of presentations of Speakers who allow the distribution, informative literature for the day, welcome coffee, networking lunch, coffee break, organisational office assistance, certificate of attendance.

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### Terms of payment

The registration fee must be paid at the time of registration. Confirmation of event admission will be given on receipt of payment. EasyB reserves the right to refuse late registrations or additional registrations above the maximum accepted number of participants or registrations of roles that are not included in the target of the event.

#### Cancellation

Please note that refunds (70% refund of the registration fee) will only be given if cancellation is received at least one week before the event date. Cancellations will only be valid if made in writing (by email). Transfer of registrations (or name changes) are allowed and should be made in writing within 7 days prior to the event. EasyB reserves the right to postpone or cancel an event, to change the location of an event or to alter the advertised speakers for an event. EasyB is not responsible for any loss or damage as a result of substitution, alteration, postponement or cancellation of an event due to causes beyond its control including without limitation, acts of God, natural disasters, sabotage, accident, trade of industrial disputes, terrorism, or hostilities.

#### Information collection and use

Participants information is collected and utilised by EasyB S.r.l. and sponsor companies in accordance with Italian Legislative Decree 196/2003. Data collected will be used and communicated to third parties for the purposes of event organisation and may be used to communicate future similar initiatives. Participants may at any time verify the accuracy of the information and request changes or deletion. During the event, the LS Academy Staff may take pictures, that could be shared on our Social Media pages.

#### Payment by bank transfer

The full amount must be paid on registration to EasyB S.r.l. by bank transfer. If you pay by bank transfer, please attach proof of payment to the registration form.

Bank transfer payable to:

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