

*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 CROS 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

Sponsorship opportunities

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- **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 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 12th) 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.

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.

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For any additional information, please contact:

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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.

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