

## Satellite Symposium to the WCD 2019:

### Advances in EB and Ichthyoses



**9.-11.June, 2019**

**Venue: Grand Hotel Bristol**

**Stresa, Italy**

#### **Programme committee (tentative):**

Matthias Schmuth (Innsbruck), Johann Bauer (Salzburg), Eli Sprecher (Tel Aviv), Judith Fischer (Freiburg), Keith Choate (New Haven), Leena Bruckner-Tuderman (Freiburg), Christine Bodemer (Paris), Alain Hovnanian (Paris), M Akiyama (Nagoya), MarcelJonkman (Groningen), FrancisPallison (Santiago de Chile), PeterMarinkovich (Palo Alto), John McGrath (London), Dedee Murrell (Sydney), KatsutoTamai (Osaka), H Traupe (Muenster), Yong Yang (Peking)

1. Measuring Therapeutic Success

Outcome Measures EB – Overview

D. Murrell, Sidney

Outcome Measures Ichthyoses – Overview

K. Choate/L. Milstone, New Haven

Trial Design for Rare Diseases - Statistics

N.N.

Trial Design for Rare Diseases – Toxicology, FDA/EMA-regulations

N.N.

2. Therapeutic advances – New pathomechanisms as a source of novel therapeutic targets

Theme 1

N.N.

Theme 2

N.N.

Metabolic aspects: Benitah S, Barcelona

Lipid therapies

P. Elias, San Francisco

3. Therapeutic advances – clinical trials

Small molecules

V. Wally, Salzburg; L. Bruckner-Tuderman, Freiburg

Proteins/Peptides

P. Marinkovich

Gene replacement

M. De Luca, Modena

Nucleases

J. Reichelt, Salzburg

Biologics: Paller, Chicago