NEW HORIZONS IN FABRY DISEASE

INTERNATIONAL CONFERENCE ON ADVANCES IN THE TREATMENT OF FABRY DISEASE

PRAGUE, CZECH REPUBLIC
NOVEMBER 24–25, 2017

HELD UNDER THE AUSPICES OF THE CZECH MEDICAL ACADEMY

WWW. HORIZONS-FABRY.COM
CONFERENCE PROGRAMME
ORGANIZED BY GALÉN-SYMPOSION
UNDER THE AUSPICES OF THE CZECH MEDICAL ACADEMY

ENDORSED BY
CENTER FOR FABRY DISEASE
GENERAL UNIVERSITY HOSPITAL
PRAGUE
CZECH REPUBLIC

FRENCH REFERENCE CENTER FOR FABRY DISEASE
UNIVERSITY OF VERSAILLES
FRANCE

MEETING FACULTY:
MEETING CHAIR: ALEŠ LINHART (CZECH REPUBLIC)
MEETING CO-CHAIR: DOMINQUE GERMAIN (FRANCE)

ULLA FELDT RASMUSSEN (DENMARK), PERRY ELLIOTT (UNITED KINGDOM),
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DAVID KASPER (AUSTRIA), JEFFREY MEDIN (CANADA),
GERE SUNDER-PLASSMANN (AUSTRIA), ANDREAS SCHAAF (GERMANY),
RAPHAEL SCHIFFMANN (USA), PETER NORDBECK (GERMANY),
OLIVIER MORAND (SWITZERLAND)
FRIDAY, NOVEMBER 24TH

12:00 – 13:00  GET-TOGETHER LUNCH

13:00 – 15:30  SESSION 1
DIAGNOSTIC DILEMMAS IN FABRY DISEASE
Chairpersons: Carla Hollak, Ales Linhart

MAKING DIAGNOSIS OF FABRY DISEASE – WHERE DO WE STAND - MUTATIONS, GLA ACTIVITY AND LYSO GB3
David Kasper (Austria)

GENOTYPE – PHENOTYPE CORRELATIONS – WHAT NEXT – BIOMARKERS, BIOPSY, CLINICAL STAGING?
Albina Nowak (Switzerland)

X-CHROMOSOME INACTIVATION – READY FOR CLINICAL USE?
Dominique Germain (France)

CARDIAC LATE-ONSET VARIANTS VS. CLASSICAL PHENOTYPE – PROGNOSIS AND TREATMENT ISSUES
Perry Elliott (United Kingdom)

CRITICAL REVIEW OF CURRENT TREATMENT RECOMMENDATIONS – DO WE ALL SPEAK THE SAME LANGUAGE?
Carla Hollak (The Netherlands)

15:30 – 16:00  COFFEE BREAK

16:00 – 18:00  SESSION 2
ENZYME REPLACEMENT THERAPY – CURRENT KNOWLEDGE AND FUTURE PERSPECTIVE
Chairpersons: Ana Jovanovic, Perry Elliott

ENZYME REPLACEMENT THERAPIES – WHAT DO WE KNOW AFTER 16 YEARS OF CLINICAL USE

AGALSIDASE ALFA
Christoph Kampmann (Germany)

AGALSIDASE BETA
Dominique Germain (France)

ENZYME REPLACEMENT THERAPIES IN DEVELOPMENT – PRECLINICAL AND CLINICAL DATA AND EXPERIENCE WITH PEGUNIGALSIDASE ALFA
Raphael Schifffmann (USA)

MECHANISMS OF ENZYME UPTAKE FROM CIRCULATION – MOSS-AGAL
Andreas SchaaF (Germany)

19:00 – 21:00  COCKTAIL DINNER
Old Town Hall, Staromestske namestí 1/3, Prague 1
(group departure – walk – at 18:40 from the hotel)
**SATURDAY, NOVEMBER 25TH**

**SESSION 3**

**SMALL MOLECULES IN THE TREATMENT OF FABRY DISEASE**

**Chairpersons:** Ulla Feldt-Rasmussen, Pablo García Pavia

**Chaperones – Mechanisms of Action and Identification of Amenable Mutations**

Ana Jovanovic (United Kingdom)

**Migalastat – Pivotal Clinical Trials Results – Clinical Implications**

Ulla Feldt-Rasmussen (Denmark)

**Migalastat in Clinical Practice – Single Center Experience**

Peter Nordbeck (Germany)

**Substrate Reduction Therapy with Lucerastat for Fabry Disease**

Olivier Morand (Switzerland)

**Screening Studies – Newborn Screening vs. High Risk Population Screening Strategy**

Pablo García Pavia (Spain)

11:20 – 11:50 **Coffee Break**

11:50 – 13:30 **SESSION 4**

**What Next**

**Chairpersons:** Albina Nowak, Mehdi Namdar

**Substrate Reduction Therapies – GZ/SAR402671 and Combination Therapies – What Do We Know So Far...**

Aleš Linhart (Czech Republic)

**Gene Therapy for Fabry Disease**

Jeffrey Medin (Canada)

**Concluding Remarks**

Aleš Linhart (Czech Republic)

13:30 – 14:30 **Lunch**
PLATINUM PARTNER:

Amicus Therapeutics®

PARTNERS:

SANOFI GENZYME

Shire

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