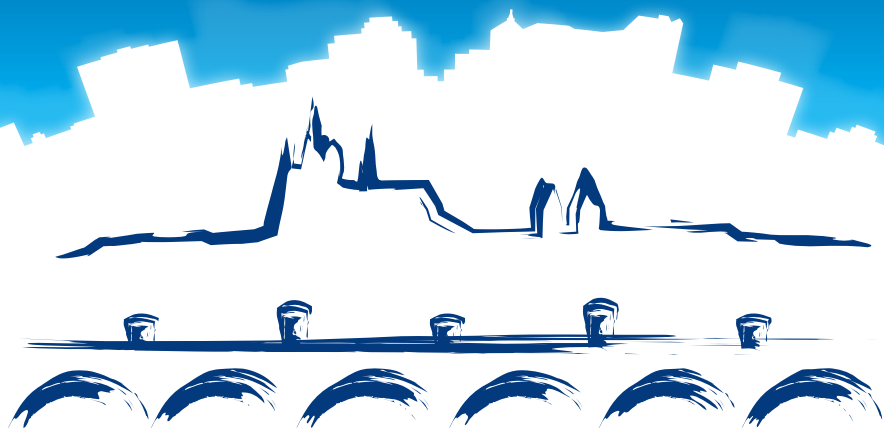


NEW HORIZONS IN FABRY DISEASE

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-SYMPOSIUM
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: DOMINIQUE GERMAIN (FRANCE)

ULLA FELDT RASMUSSEN (DENMARK), PERRY ELLIOTT (UNITED KINGDOM),
ANA JOVANOVIĆ (UNITED KINGDOM), CARLA HOLLAK (THE NETHERLANDS),
MEHDI NAMDAR (SWITZERLAND), ALBINA NOWAK (SWITZERLAND),
PABLO GARCÍA PAVÍA (SPAIN), CHRISTOPH KAMPMANN (GERMANY),
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, ALEŠ 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 JOVANOVIĆ, 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 SCHIFFMANN (USA)

MECHANISMS OF ENZYME UPTAKE FROM CIRCULATION – MOSS-AGAL

ANDREAS SCHAAF (GERMANY)

19:00 – 21:00 **COCKTAIL DINNER**

OLD TOWN HALL, STAROMĚSTSKÉ NÁMĚSTÍ 1/3, PRAGUE 1
(GROUP DEPARTURE – WALK – AT 18:40 FROM THE HOTEL)

SATURDAY, NOVEMBER 25TH

9:00 – 11:20

SESSION 3

SMALL MOLECULES IN THE TREATMENT OF FABRY DISEASE

CHAIRPERSONS: : ULLA FELDT-RASMUSSEN, PABLO GARCÍA PAVÍA

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 PAVÍA (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:



PARTNERS:

SANOFI GENZYME 

The text 'SANOFI GENZYME' is displayed in a blue, sans-serif font. To the right of the text is a circular logo icon with a blue and green gradient, resembling a stylized 'S' or a wave.

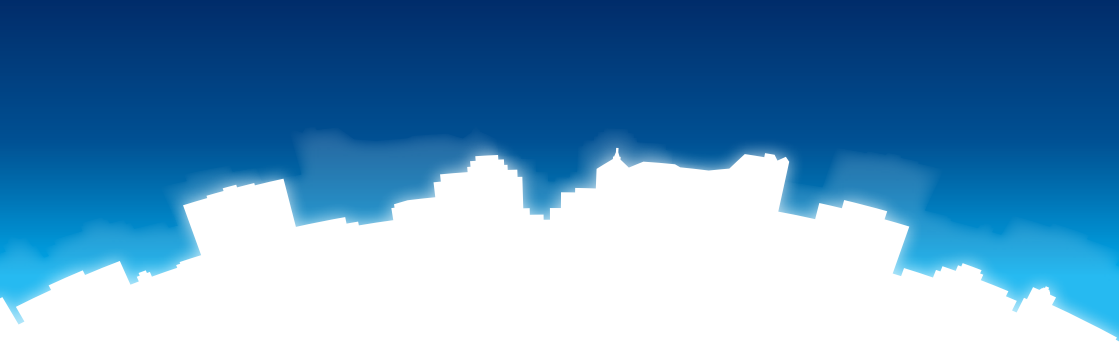
 **Shire**

The logo for Shire features a stylized blue 'S' icon on the left, composed of two overlapping curved shapes. To the right of the icon, the word 'Shire' is written in a bold, blue, sans-serif font.

IDORSIA

PROTALIX

GREENOVATION



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