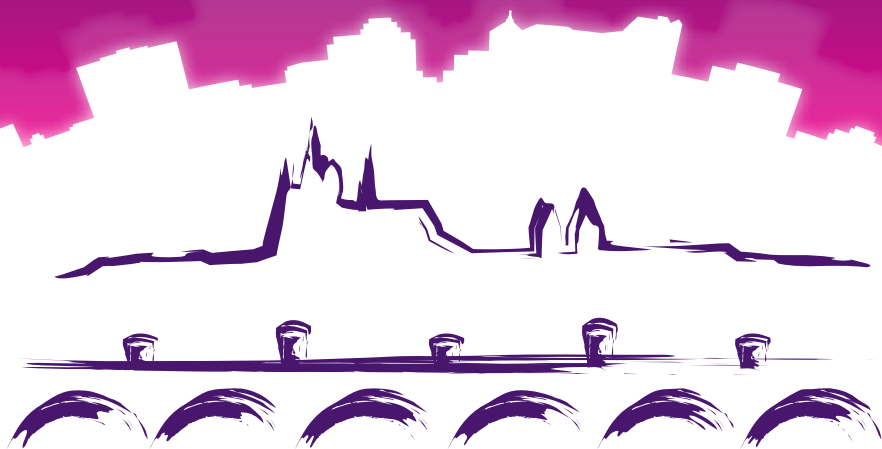


NEW HORIZONS IN FABRY DISEASE

INTERNATIONAL CONFERENCE ON ADVANCES
IN THE TREATMENT OF FABRY DISEASE



PRAGUE, CZECH REPUBLIC
NOVEMBER 1–2, 2019

HELD UNDER THE AUSPICES OF THE CZECH SOCIETY OF CARDIOLOGY



CZECH SOCIETY
OF CARDIOLOGY

CONFERENCE PROGRAMME

ORGANIZED BY GALÉN-SYMPOSIUM
UNDER THE AUSPICES OF THE CZECH SOCIETY OF CARDIOLOGY



CZECH SOCIETY
OF CARDIOLOGY

THE CONFERENCE IS HELD UNDER THE AUSPICES
OF THE MAYOR OF PRAGUE

ENDORSED BY

CENTER FOR FABRY DISEASE
GENERAL UNIVERSITY HOSPITAL
PRAGUE
CZECH REPUBLIC

FRENCH REFERENCE CENTER
FOR FABRY DISEASE
UNIVERSITY OF VERSAILLES
FRANCE



VFN PRAHA



SCIENTIFIC AND ORGANIZING COMMITTEE:

ALEŠ LINHART

(GENERAL UNIVERSITY HOSPITAL AND FIRST FACULTY OF MEDICINE, CHARLES
UNIVERSITY PRAGUE, CZECH REPUBLIC)

DOMINIQUE P. GERMAIN

(FRENCH REFERENCE CENTER FOR FABRY DISEASE UNIVERSITY OF VERSAILLES, FRANCE)

PERRY M. ELLIOTT

(UNIVERSITY COLLEGE OF LONDON, LONDON, UNITED KINGDOM)



European
Reference
Networks

ICON

International Cardiomyopathy
Network

FRIDAY, NOVEMBER 1ST

9:00 – 10:00 REGISTRATION

10:00 – 10:10 WELCOME AND INTRODUCTION

ALEŠ LINHART (CZ), DOMINIQUE P. GERMAIN (FR), PERRY M. ELLIOTT (GB)

10:10 – 10:40 KEYNOTE LECTURE

CHAIR: ALEŠ LINHART

**FROM LYSOSOMAL BIOGENESIS TO LYSOSOMAL STORAGE
DISORDERS**

JIRÍ ZEMAN (CZ).....30'

10:40 – 12:20 SESSION 1

UNDERSTANDING THE PATHOPHYSIOLOGY I

CO-CHAIRS: BOJAN VUJKOVAC, PERRY M. ELLIOTT

THE ROLE OF INFLAMMATION

SANDRO FERIOZZI (IT).....20'

THE ROLE OF VASCULAR AND MICROVASCULAR INVOLVEMENT

ALEŠ LINHART (CZ).....20'

ELECTROPHYSIOLOGY OF FABRY HEART

MEHDI NAMDAR (CH).....20'

LYSO GB3 – PATHOPHYSIOLOGICAL ROLE

JOHANNES AERTS (NL).....20'

Q & A.....20'

12:20 – 13:40 LUNCH

13:40 – 15:00 SESSION 2

UNDERSTANDING THE PATHOPHYSIOLOGY II

CO-CHAIRS: ELENA BIAGINI, ALBINA NOWAK

**IMPACT OF CARDIOVASCULAR DISEASE ON QOL AND
PROGNOSIS IN LATE ONSET VARIANT AFD PATIENTS**

PERRY M. ELLIOTT (GB).....20'

X-INACTIVATION – READY FOR CLINICAL USE?

DOMINIQUE P. GERMAIN (FR).....20'

BIOMARKERS

ALBINA NOWAK (CH).....20'

Q & A.....20'

15:00 – 15:30 COFFEE BREAK

SATURDAY, NOVEMBER 2ND

15:30 – 17:50 SESSION 3 DIAGNOSTIC TOOLS

CO-CHAIRS: PERRY M. ELLIOTT, DAVID WARNOCK

TI MAPPING

SABRINA NORDIN (GB).....20'

SCREENING METHODS IN FABRY DISEASE

DAVID KASPER (AT).....20'

SCREENING IN HIGH RISK POPULATIONS AND PEDIGREE DRAWING

DOMINIQUE P. GERMAIN (FR)20'

GENOTYPE – PHENOTYPE CORRELATION

JOÃO OLIVEIRA (PT).....20'

DOES ENDOMYOCARDIAL BIOPSY HELP IN SOLVING THE CONTROVERSY OF VARIANTS OF UNKNOWN SIGNIFICANCE - A CASE OF D313Y VARIANT?

FERNANDO DOMINGUEZ (ES).....20'

KIDNEY BIOPSY

CAMILLA TØNDEL (NO).....20'

Q & A 20'

17:50 CLOSING REMARKS DAY 1

19:00 CONFERENCE COCKTAIL DINNER
THE LORD MAYOR'S RESIDENCE (REZIDENCE PRIMÁTORA)
MARIÁNSKÉ NÁMĚSTÍ 1/98, PRAGUE 1
(GROUP DEPARTURE – WALK – AT 18:40 FROM THE HOTEL
LOBBY)

9:00 – 10:30 SESSION 4 TARGETED THERAPIES I

CO-CHAIRS: BOJAN VUJKOVAC, DAVID WARNOCK

REGISTRY DATA – AGALSIDASE BETA

DOMINIQUE P. GERMAIN (FR)20'

INTERPRETING EFFECTIVENESS DATA IN FABRY DISEASE – AGALSIDASE ALFA

CHRISTOPH KAMPMANN (DE).....20'

IMMUNE RESPONSE TO ENZYME REPLACEMENT THERAPIES

MALTE LENDERS (DE).....20'

PREVENTION IS THE BEST THERAPY: THE GENETICIST'S APPROACH

GHEONA ALTADESCU (IL).....20'

Q & A10'

10:30 – 11:00 COFFEE BREAK

11:00 – 13:10 SESSION 5 TARGETED THERAPIES II

CO-CHAIRS: GHEONA ALTADESCU, ALBERTO ORTIZ

AMENABILITY TESTING

MALTE LENDERS (DE).....20'

RESPONSE TO MIGALASTAT – ENZYMATIC LEVELS AND BIOMARKERS

PETER NORDBECK (DE)20'

UBSTRATE REDUCTION THERAPIES

MARKUS CYBULLA (DE)20'

CHAPERONES

DERRALYNN HUGHES (GB).....20'

NEW ENZYMES

DAVID WARNOCK (US)20'

GENETIC TREATMENTS

DERRALYN HUGHES.....20'

Q & A10'

13:10 – 14:00 LUNCH

14:00 – 15:40 **SESSION 6**
SYMPTOMATIC TREATMENTS

CO-CHAIRS: DOMINIQUE P. GERMAIN, GHEONA ALTADESCU

PAIN MANAGEMENT

ROCCO LIGUORI (IT)20'

**RENIN-ANGIOTENSIN SYSTEM INHIBITORS –
WHAT CAN BE EXPECTED**

ALBERTO ORTIZ (ES)20'

**ARRHYTHMIA MANAGEMENT AND SUDDEN DEATH
PREVENTION**

MAURIZIO PIERONI (IT)20'

**VALVULAR DISEASE – WHAT IS KNOWN AND HOW TO
MANAGE THEM**

ALES LINHART (CZ)20'

FUTURE TRENDS IN AFD

PERRY M. ELLIOTT (GB)20'

15:40 **CLOSING REMARKS AND ADJOURN**





THE MEETING IS SUPPORTED BY EDUCATIONAL GRANTS FROM
AMICUS THERAPEUTICS, TAKEDA (SHIRE),
SANOFI GENZYME AND PROTALIX / CHIESI
BUT ITS CONTENT IS FULLY INDEPENDENT.

CONFERENCE VENUE:
HILTON PRAGUE OLD TOWN
V CELNICI 7, 110 00 PRAHA 1
CZECH REPUBLIC

ORGANIZING AGENCY:
GALÉN-SYMPOSIUM S.R.O.
BŘEŽANSKÁ 10, 100 00 - PRAGUE 10
CZECH REPUBLIC