



3rd ELA Scientific Congress

Paris, June 23-26, 2015

Advances in molecular pathogenesis, diagnosis and management of the leukodystrophies

PRELIMINARY PROGRAM

Advisory Scientific Committee

Chair: Charles French-Constant (UK)

Francesca ALOISI (Italy); Patrick AUBOURG (France); Anne BARON (France); Enrico BERTINI (Italy); Raul ESTEVEZ (Spain); Robin FRANKLIN (UK); Volkmar GIESELMANN (Germany); Wolfgang KÖHLER (Germany); Alfried KOHLSCHÜTTER (Germany); Jean-Louis MANDEL (France); David ROWITCH (USA); Frédéric SEDEL (France); Jacqueline TROTTER (Germany); Ronald WANDERS (The Netherlands)

Local organization: The ELA Research Foundation

June 23, 2015

8-10 pm

WELCOME COCKTAIL - Hotel Ibis Bercy

June 24, 2015

9-9:30 am

Amphitheater, Brain and Spine Institute (ICM)

CONGRESS OPENING

Welcome

*Chair of the ELA Research Foundation Supervisory Board
Guy Alba (France)*

*Chair of the ELA Scientific Committee
Charles French-Constant (UK)*

ELA: 20 years of calls for research proposals

Jean-François Dhainaut, Marie-Josée Duran, ELA

9:30-11 am

Session 1 – PATHOGENESIS OF LEUKODYSTROPHIES

Chair: Jacqueline TROTTER, Mainz University, Germany

Aicardi Goutières syndrome clinical trials

Yanick J. Crow, Imagine Institute, Paris, France

Megalencephalic leukoencephalopathy with subcortical
cysts: intracellular trafficking of MLC1 in astrocytes

Elena Ambrosini, Istituto Superiore di Sanita, Rome, Italy

Clinical spectrum of polymerase 3-hypomyelinating
leukodystrophies

Geneviève Bernard, McGill University, Montreal, Canada

11-11:30 am

COFFEE BREAK

11:30 am-1 pm

Session 2 - ADVANCES IN ANIMAL MODELS

*Chair: Anne BARON-VAN EVERCOOREN, ICM, Paris,
France*

New insights from the mouse model for Vanishing White
Matter disease

Orna Elroy-Stein, University of Tel Aviv, Israel

An improved mouse model for X-linked adrenoleukodystrophy

*Stephan Kemp, Academic Medical Center, University of
Amsterdam, The Netherlands*

Canavan mice models

*Matthias Eckhardt, Institut für Biochemie und
Molekularbiologie, Bonn, Germany*

1-2 pm

POSTER SESSIONS & LUNCH

2-4 pm

Session 3 – IMAGING MYELIN DISEASES

Chairs: David ROWITCH, UCSF, San Francisco, CA, USA & Wolfgang KÖHLER, Saxonian State Hospital Hubertusburg, Wermsdorf, Germany

Diffusion Tensor Imaging Techniques in Assessment of Leukodystrophies: Importance of Analysis Methods

James Provenzale, Duke University Medical Center, Durham, NC, USA

Brain FDG-PET in adrenoleukodystrophy

Ettore Salsano, Neurology Institute, Milano, Italy

White matters abnormalities – DTI and functional imaging

Denis Ducreux, Bicêtre Hospital, Paris, France

Imaging central nervous system myelin by positron emission tomography

Bruno Stankoff, Saint-Antoine Hospital, Paris, France

4-4:30 pm

COFFEE BREAK

4:30-6 pm

WORKSHOP - TRAINING FOR YOUNG INVESTIGATORS & CLINICIANS “What is a leukodystrophy?”

Caroline Sevin, Bicêtre Hospital, Le Kremlin-Bicêtre, France

June 25, 2015

Amphitheater, Brain and Spine Institute (ICM)

9-10:30 am

Session 4 – MOLECULAR DIAGNOSIS

Chair: Marjo van der KNAAP, VuMC, Amsterdam, The Netherlands

New genetic approaches in leukodystrophies

Marjo van der Knaap, VuMC, Amsterdam, The Netherlands

Identification of novel leukodystrophies

Imen Dorboz, ELA, Paris, France (+ INSERM ?)

Targeted high-throughput sequencing for diagnosis of leukodystrophies

Claire Redin, Institute of Genetics and Molecular and Cellular Biology (IGBMC), Strasbourg, France

10:30-11 am

COFFEE BREAK

- 11 am-1:30 pm **Session 5 – ADVANCES TOWARDS CELL & REMYELINATING THERAPIES**
Chair: Robin FRANKLIN, University of Cambridge, UK
 Signaling pathways involved in myelination
Bernard Zalc, ICM, Paris, France
 Signaling in neural stem and progenitor cells
Terri Wood, Rutgers New Jersey Medical School, Newark, NJ, USA
 Transplantation of glial precursor cells in Vanishing White Matter
Vivi Heine, VuMC, Amsterdam, The Netherlands
 Stem cell therapy for PMD
Anne Baron-Van Evercooren, ICM, Paris, France
 Induced neuronal reprogramming
Marius Wernig, Stanford University, CA, USA
- 1:30-2:30 pm **POSTER SESSIONS & LUNCH**
- 2:30-5 pm **WORKSHOP**
“Optimizing clinical data to biobank & omics data”
Chairs: Charles ffrench-CONSTANT, University of Edinburgh, UK & David ROWITCH, UCSF, San Francisco, CA, USA

Speakers:
Odile Boespflug-Tanguy, Robert-Debré Hospital, Paris, France
Adeline Vanderver, Children’s Hospital, Washington D.C., USA
Patrick Aubourg, Bicêtre Hospital, Le Kremlin-Bicêtre, France
Industry representative
- 5-6 pm **POSTER SESSIONS**
- 8 pm **BANQUET** - Hotel Ibis Bercy

June 26, 2015

Amphitheater, Brain and Spine Institute (ICM)

9-11 am

Session 6 – PHARMACOLOGICAL THERAPIES

Chair: Patrick AUBOURG, Bicêtre Hospital, Le Kremlin-Bicêtre, France

Neuroprotective and promyelinating actions of Olesoxime in hypomyelinating leukodystrophies

Odile Boespflug-Tanguy, Robert-Debré Hospital, Paris, France

Identification of drug targets in X-ALD: translation to the clinics

Aurora Pujol, IDIBELL, Barcelona, Spain

Cholesterol-based therapy for Pelizaeus-Merzbacher Disease

Gesine Saher, Max Planck Institute for Experimental Medicine, Göttingen, Germany (pending)

MD-1003 clinical trial in adrenomyeloneuropathy

Frédéric Sedel, MedDay Pharmaceuticals, Paris, France

11-11:30 am

COFFEE BREAK

11:30 am-1pm

Session 7 - GENE THERAPIES

Chair: Enrico BERTINI, Ospedale Bambino Gesù, Rome, Italy

Long-term follow-up of gene therapy for cerebral adrenoleukodystrophy

Patrick Aubourg, Bicêtre hospital, Le Kremlin-Bicêtre, France

Update on the HSC gene therapy trial for the treatment of metachromatic leukodystrophy

Alessandra Biffi, San Raffaele Telethon Institute for Gene Therapy, Milano, Italy

Major improvement in lifespan and pathology following BMT plus iv injection of AAVrh10-GALC in twitcher mice

David Wenger, Thomas Jefferson University, Philadelphia, PA, USA.

1 pm

CONGRESS CLOSING & AWARD CEREMONY

LUNCH
