EUROPE RARE DISEASE SUMMIT 2023

February 15th 2023 – Hotel Ilunion Pío XII - Madrid, Spain
IN PERSON/ +200 ATTENDANTS/ +35 SPEAKERS/ +6 HOURS NETWORKING
Language: ENGLISH

INTRODUCTION

Through a variety of panels and stand-alone presentations by industry leaders, the objective of the European Rare Disease Summit is to uncover the key trends and topics relating to diagnostics and treatments for Rare Diseases in Europe, as well as the challenges to timely and equitable access to orphan drugs, and European initiatives for access, research and innovation programs to improve the lives of patients with rare diseases.

Topics to be covered:

- Government Plans
- Access & Financing
- Clinical Research and Innovation
- Artificial Intelligence and Data
- Social & Patient

TARGET AUDIENCE

Local and European government authorities, members of the European Commission and the European Parliament, large public and private hospital groups, research centres, pharmaceutical and patient associations, regulatory agencies and scientific societies and healthcare professionals from across Europe.

AGENDA

(*) speaker pending confirmation, subject to alterations

08:00 **PRE-EVENT RECEPTION AND NETWORKING**

09:00 **Welcome & Opening Remarks:**

- **Ignacio Para Rodriguez-Santana,** Presidente de la Fundación Bamberg
- TBD

09:15 **1st Panel: National Plans, Policies and Government Initiatives Against Rare Diseases**

- **Gianni D'Errico,** Vice-Chair of ICPerMed – International Consortium for Personalised Medicine (Italy)
- **Lucy McKay,** CEO Medics4RareDiseases (UK)
- **Ana Rath,** Director of Orphanet at INSERM (France)
- **Alexis Nolte**, Head of Human Medicines European Medicines Agency (EMA) (Netherlands)*
- **Dolors Montserrat,** Parlamentaria europea. Ponente del Informe: Una estrategia farmacéutica para Europa (Belgium)*

Moderator: **TBD**

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10:00 **2nd Panel: Addressing Rare Diseases: Research Networks and Reference Centres**

- **Madeleine Durbeej-Hjalt**, Secretary General for Medicine and Health at the Swedish Research Council and Expert in Rare Muscle Diseases (Sweden)
- **Lucia Mazzolai,** Director Centre of Malformations and Rare Vascular Diseases CHUV, Centre Hospitalier Universitaire Vaudois, Lausanne (Switzerland)*
- **Alain Verloes** Professor, Department of Genetic, Hospital Robert Debré and Coordinator of ERN ITHACA (Intellectual Disability and Congenital Malformations) (France)*
- **Olve Moldestad,** Head of the Centre for Precision Medicine for Rare Diseases, Oslo University Hospital (Sweden)*
- **Claudio Carta,** Researcher, National Centre for Rare Diseases, Istituto Superiore di Sanità (Italy)*

Moderator: TBD

10:45 **NETWORKING BREAK**

11:15 **3rd Panel: The Role of the Pharmaceutical and Biotechnology Industries**

- **Alexander Natz,** Secretary General EUCOPE (Belgium)
- **Claudio Carini,** Faculty at King's College School of Medicine. Member of FNIH Biomarkers Consortium (UK) *
- **Veronika Jekerle,** Head of Pharmaceutical Quality European Medicines Agency (EMA) (Germany)*
- **Xavier de Cuyper,** CEO Federal Agency for Medicines and Health Products Belgium*
- **Lars Bo Nielsen,** Director General Danish Medicines Agency *

Moderator: **TBD**

12:00 **Industry presentation** (TBD)

12:15 **4th Panel: Innovation and Access to Orphan Drugs: Challenges and Opportunities**

- **Tina Taube,** Director Market Access & Orphan Drug Policy Lead EFPIA - European Federation of Pharmaceutical Industries and Associations (Belgium)
- **Jordi Faus,** Founding Partner, Faus Moliner Abogados (Spain)
- **Thomas Bols,** Head of Government Affairs and Patient Advocacy, EMEA & APAC, PTC Therapeutics (Switzerland)
- **Kristina Larsson,** Head of Orphan Medicines European Medicines Agency (EMA) (Sweden)*
- **Maria José Sanchez,** Presidente de la Asociación española de laboratorios de medicamentos huérfanos y ultra huérfanos, AELMHU (Spain)*

Moderator: **TBD**

13:00h **LUNCH AND NETWORKING**

14:00h **5th Panel: The Role of AI & Data in Overcoming the Challenges of Rare Diseases Specific Registry and Diagnostic**

- **Josep Samitier Martí**, Director, Institute for Bioengineering of Catalonia (IBEC), European Project THERACAT (Spain)

- **Bogi Eliasen,** Director of Health, Copenhagen Institute for Futures Studies (Denmark)
- **Dr. Sebastian Köhler,** Bioinformatician, Berliner Institut für Gesundheitsforschung. Co-Founder of Human Phenotype Ontology (HPO) (Germany)*
- **Sergi Beltran,** Head of Bioinformatics Unit and Data Analysis Team, Centro Nacional de Análisis Genómico (Spain)*
- **Karl Hamilton,** Head of Digital Change European Medicines Agency (EMA) (Ireland)*

Moderator: **TBD**

14:45 **6th Panel: Advances in Rare Disease Diagnosis and Treatment: Gene Therapies**

- **Jose Luis Zamorano,** Jefe de Servicio de Cardiología Hospital Ramón y Cajal (Spain)
- **Milan Macek**, Head of the Institute of Biology and Medical Genetics of Charles University and Motol University Hospital (Czech Republic)*
- **Victor Volpini**, Director of Molecular Genetics Diagnosis Centre Idibell, Biomedical Research Institute (Spain)*
- **Alain Verloes,** Professor, Department of Genetic, Hospital Robert Debré and Coordinator of ERN ITHACA (Intellectual Disability and Congenital Malformations), (France)*
- **Catherine Nguyen,** Director of the Thematic Institute of the Genetics, Genomics and Bioinformatics (France)*

Moderator: **TBD**

15:30 **7th Panel: Viral Vector & mRNA Technologies: Implementation and Future Development Against Rare Diseases**

- **María de la Fuente,** Directora de la Unidad de Nano-Oncología del Instituto de Investigación Sanitaria de Santiago de Compostela (IDIS) (Spain)
- **Christian Kubisch**, Head of Undiagnosed Disease Program (UDP), Martin Zeitz Centrum for Rare Diseases, UKE Hamburg (Germany)*
- **Prof. Dr Stefan Mundlos**, Director, Institut für Medizinische Genetik und Humangenetik, Charité Universitätsmedizin Berlin (Germany)*
- **Lourdes Ruiz Desviat,** Directora del Centro de Biología Molecular Severo Ochoa (Spain)*
- TBD, Moderna

Moderator: **TBD**

16:15 **8th Panel: The Patient's Voice as a Driver of Access and Drug Approvals**

- **Yann Le Cam,** CEO of EURORDIS- Rare Diseases Europe (France) *
- **Manuela Stier,** President and Founder of Kinder Mit Seltenen Krankheiten (KMSK) (Switzerland)*
- **Marie-Christine Ouillade**, French Muscular Dystrophy Association, AFM-Téléthon (France) *
- **Marco Greco,** President of the Board European Patients Forum (EPF) (Belgium)*
- **Mike Morrissey,** CEO European Cancer Organisation (Belgium)*

Moderator: **TBD**

17:00 **CLOSING REMARKS**

17:15 **END OF THE EVENT AND NETWORKING**

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CONFIRMED SPEAKERS

- **Gianni d'Errico,** Vice-Chair of ICPerMed – International Consortium for Personalised Medicine (Italy)
- **Lucy McKay,** CEO Medics4RareDiseases (UK)
- **Ana Rath,** Director of Orphanet at INSERM (France)
- **Madeleine Durbeej-Hjalt**, Secretary General for Medicine and Health In Sweden and Expert in Rare Muscle Diseases (Sweden)
- **Alain Verloes** Professor, Department of Genetic, Hospital Robert Debré and Coordinator of ERN ITHACA (Intellectual Disability and Congenital Malformations), France
- **Alexander Natz,** Secretary General European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)
- **Thomas Bols,** Head of Government Affairs and Patient Advocacy EME & APAC PTC Therapeutics (Switzerland)
- **Tina Taube,** Director Market Access & Orphan Drug Policy Lead EFPIA - European Federation of Pharmaceutical Industries and Associations (Belgium)
- **Josep Samitier Martí,** Director, Institute for Bioengineering of Catalonia (IBEC), European project THERACAT (Spain)
- **Milan Macek,** Head of the Institute of Biology and Medical Genetics of Charles University and Motol University Hospital (Czech Republic)
- **Jordi Faus,** Founding Partner, Faus Moliner Abogados (Spain)
- **Bogi Eliasen,** Director of Health, Copenhagen Institute for Futures Studies (Denmark)
- **Michael Schlander**, Head of Division of Health Economics at German Cancer Research Center (DKFZ), Founder and Chairman of InnoVal, Prof. of Health Economics, University of Heidelberg (Germany)
- **Jose Luis Zamorano,** Professor of Cardiology and Head of Department of the Hospital Ramón y Cajal (Spain)
- **María de la Fuente,** Director of the Nano-Oncology Unit of the Health Research Institute of Santiago de Compostela (IDIS) (Spain)

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