EUROPE RARE DISEASE SUMMIT

Madrid, February 14th, 2024

Date and Location of the meeting

- February 14th of 2024
- Madrid

With the Institutional support of the **Fundación Bamberg**

Objectives:

The European Rare Disease Summit aims to explore important developments and issues in diagnosing and treating Rare Diseases in Europe. This will be done through a series of discussions and individual talks led by experts in the field. The event will also address the obstacles faced in ensuring prompt and fair availability of specialized medications, known as orphan drugs. Additionally, it will highlight European efforts in facilitating access, advancing research, and promoting innovative programs to enhance the well-being 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 centers, pharmaceutical and patient associations, regulatory agencies and scientific societies and healthcare professionals from across Europe.

Agenda

(*subject to confirmation)

08:30 RECEPTION AND NETWORKING
Registro de asistentes y café de bienvenida

09:00 OPENING AND WELCOME REMARKS:

- ***Ignacio Para Rodríguez-Santana,*** *Presidente de la Fundación Bamberg*
- ***Stelios Kympouropoulos,*** *Member of the European Parliament, Greece**
- ***Fátima Matute Teresa,*** *Consejera de Sanidad de la Comunidad de Madrid**

09:15 1ST PANEL: NATIONAL PLANS, POLICIES AND GOVERNMENT INITIATIVES AGAINST RARE DISEASES

- ***Ana Rath,*** *Director of Orphanet at INSERM**
- ***Lars Bo Nielsen,*** *Director General Danish Medicines Agency**
- ***Domenica Taruscio,*** *Director Istituto Superiore di Sanitá (ISS) (Italy)**
- ***Alexander Natz,*** *Secretary General European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)**
- ***Carmen Laplaza Santos,*** *Head of Health Innovations, Directorate-General for Research and Innovation, DG RTD, European Commission**

10:00 2º PANEL: THE PATIENT'S VOICE AS A DRIVER OF ACCESS AND DRUG APPROVALS

- ***Jean-Philippe Plançon,*** *Vice-President Alliance Maladies Rares**
- ***Annelis Ronnow,*** *Representative Federation of European Scleroderma Associations at EURORDIS**
- ***Souad Mazari,*** *Founder NMO France**
- ***Juan Carrión Tudela,*** *President FEDER**
- ***Begonya Nafría Escalera****, Patient Engagement in Research Coordinator, Sant Joan de Déu Children's Hospital**

10:45 COFFEE BREAK AND NETWORKING

11:15 3º PANEL: INNOVATION, FUNDING AND ACCESS TO ORPHAN DRUGS: CHALLENGE AND OPPORTUNITIES

- ***Kristina Larsson,*** *Head of Orphan Medicines European Medicines Agency (EMA) (Sweden)**
- ***María Jesús Lamas Díaz*** *Executive Director Agencia Espanola de Medicamentos y Productos Sanitarios. Spain**

- ***Violeta Stoyanova-Beninska,*** *Chair of Committee for Orphan Medicinal Products at European Medicines Agency (EMA)**
- ***Tina Taube,*** *Director Market Access & Orphan Drug Policy Lead EFPIA - European Federation of Pharmaceutical Industries and Associations**
- ***Maria Cavaller-Bellaubi,*** *Patient Engagement Senior Manager, EURORDIS and MoCA Steering Committee Members**

12:00 4º PANEL: THE ROLE OF EMERGING TECHNOLOGIES TECHNOLOGIES IN OVERCOMING THE CHALLENGES OF RDS

- ***Josep Samitier Martí,*** *Director, Institute for Bioengineering of Catalonia (IBEC), European project THERACAT (Spain)**
- ***Marjon Pasmooij,*** *Member of Big Data Steering Group, Medicines Evaluation Board**
- ***Irene Norstedt*** *Director of the People Directorate, DG Research and Innovation, European Commission**
- ***Zoltan Galaz****, Leading Researcher in Data Science Brain Diseases Analysis Laboratory*
- *Sergi Beltran, Head of Bioinformatics Unit and Data Analysis Team at Centro Nacional de Análisis Genómico**

12:45 5º PANEL: ADVANCES IN RARE DISEASE DIAGNOSIS AND TREATMENT. CELL & GENE THERAPY

- ***Alastair Kent*** *Former Executive Director of Genetic Alliance UK. Chair of the UK Rare Disease Forum. Chair of the Rare Disease Advisory Group (RDAG) for NHS England. Member of the Genomics England (GeL) Ethics Advisory Committee (England)**
- ***M.ª José Calasanz Abinzano****. Directora Genética de Enfermedades Hematológicas. Centro de Investigación CIMA. Navarra (Spain)**
- ***Caroline Pothet,*** *Head of Advances Therapies, European Medicines Agency (EMA)**
- ***Pilar Gayoso Diz****, Subdirectora General de Terapia Celular y Medicina Regenerativa. Insitituto de Salud Carlos III (Spain)**
- ***Philip J. (P.J.) Brooks,*** *Acting Director Division of Rare Diseases Research Innovation, National Center for Advancing Translational Sciences**

13:30 LUNCH AND NETWORKING

14:30 6º PANEL: FOSTERING RARE DISEASE RESEARCH AND INNOVATION AGAINST RD's in EUROPE

- ***Lourdes Ruiz Desviat,*** *Directora del Centro de Biología Molecular Severo Ochoa (Spain)**
- ***Virginia Arechavala-Gomeza.*** *Group Leader Neuromuscular Disorders Group, Biocruces Bizkaia Health Research Institute (Spain)**
- ***Alexis Arzimanoglou,*** *Coordinator European Reference Network on Rare and Complex Epilepsies ERN EpiCARE chez ERN EpiCARE**
- ***Paolo Morgese,*** *Head of Public Affairs, Europe, Alliance for Regenerative Medicine**
- ***Adrien Samson,*** *Healthcare Biotechnology Manager EUROPABIO**

15:15 7º PANEL: ADDRESSING MANAGEMENT OF RARE DISEASES. RESEARCH NETWORKS & REFERENCE CENTERS

- ***Claudio Carta,*** *Researcher, National Centre for Rare Diseases, Istituto Superiore di Sanità, Italy**
- ***Maurizio Scarpa****, Director Regional Coordinating Center for Rare Diseases, Azienda Sanitaria Universitaria Friuli Centrale, Udine, (Italy)**
- ***Lucia Mazzolai,*** *Director Centre of malformations and rare vascular diseases CHUV / Centre Hospitalier Universitaire Vaudois, Lausanne (Switzerland)**
- ***Eva Bermejo-Sánchez,*** *Director of the Institute for Rare Diseases Research (IIER), Instituto de Salud Carlos III (ISCIII). Scientific Director of the Spanish National Biobank of ISCIII (Spain)*.*
- **Francesc Palau Martínez.** Scientific Coordinator of the Strategy for Rare Diseases of the Sant Joan de Déu Children's Hospital. Member of Ciberer and ERN Ithaca (Spain)*

16:00 CLAUSURA

16:15 FIN DEL ACTO

PONENTES CONFIRMADOS

Patrocinadores

Colaboradores

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