

THEME 5

ACHIEVING THE TRIPLE AS BY 2030: ACCESSIBLE, AVAILABLE AND AFFORDABLE THERAPIES FOR PEOPLE LIVING WITH A RARE DISEASE

THEME LEADERS:

Dimitrios Athanasiou, European Medicines Agency, World Duchenne Organisation and European Patient Forum, Greece

Prof. Josep Torrent-Farnell, Universitat Autònoma de Barcelona, Spain

THEME SUPPORT:

Simone Bosseli, Public Affairs Director, EURORDIS

Ana Palma, Senior Director Global HTA & Patient Access Lead, SOBI

THEME DESCRIPTION:

There are more life-changing therapies in development for people living with rare diseases than ever before, yet at our current pace it will still take decades to cover all our unmet needs. The rare disease community still faces a number of challenges in accessing authorised therapies, which indicates that the system in its current design is not functioning to the benefit of all, particularly those people living with a rare disease.

How can we improve the functioning of the system by 2030? What are the solutions to ensure the sustainable development of therapies that are truly available to all? This theme will examine the different aspects of the system which need significant change.

SESSION 0501: Friday 15th May 2020, 14:00 – 15:30

Rare Diseases in Numbers: What do they mean?

Chair: Avril Daly, CEO, Retina International; Vice-President, EURORDIS

Speakers:

Sandra Coubier, Social Research Director – Rare Barometer Programme Lead, EURORDIS

Dr. Ana Rath, Director, Orphanet

SESSION 0502: Friday 15th May 2020, 16:30 – 18:00

Financing Rare Disease Therapies Development – Is the Current Model Fit for Purpose?

Chair: Dimitrios Athanasiou, World Duchenne Organisation

SESSION 0503: Saturday 16th May 2020, 09:00 – 10:30

New Disruptive Technologies: How can we prepare Healthcare Systems?

Gene and cell therapies (ATMPs) have the potential to bring a level of disruption to treatment for rare diseases that we have never seen before. This session will explore novel treatments for haemophilia, Spinal muscular atrophy (SMA), thalassemia and retinal disorders, and will feature work done on assessment, availability, access and affordability as part of RARE IMPACT. The panel will discuss their suggestions and potential solutions for improving access across Europe.

Chair: Dr. Mariette Driessens, Policy Officer, VSOP (Dutch Genetic Alliance)

SESSION 0504: Saturday 16th May 2020, 11:00 – 12:30

From Research to Access: Is a European Collaborative Approach Possible?

Chair: TBC

SESSION 0505: Saturday 16th May 2020, 14:30 – 16:00

Ensuring Faster Development and Equitable Access: Future Scenarios from Rare 2030

Chair: Sheela Upadhyaya, National Institute for Health and Care Excellence, UK