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 to make it 'fit-for-purpose' now and for our future needs.

SESSION 0501: Thursday 14th May 2020, 14:45 – 16:15

Rare Diseases in Numbers: What do they mean?

There is a growing need for accurate baseline numbers to enable effective evidence-based advocacy for the rare disease community. Recent initiatives have addressed this need for data, with regard to the key issues of access, the economic burden of rare diseases, and the budgetary impact of therapies. This session will discuss recent studies and methodologies related to these issues.

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

Speakers:

Dr. Ana Rath, Director, Orphanet

Orla Galvin, Retina International, Ireland

Alexander Natz, Secretary-General of EUCOPE, Belgium

SESSION 0502: Thursday 14th May 2020, 16:45 – 18:15

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)

Speakers:

Mr. Declan Noone, European Haemophilia Consortium, Ireland

Evert Jan Van Lente, AOK Health Insurance, Germany

SESSION 0503: Friday 15th May 2020, 11:30 – 13:00

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

Bearing in mind technological advances as well as the need to increase the number of therapies available, can we realistically imagine one seamless European approach from development to access? What elements would this require? Can it be established in the next 10 years? This session will look at the existing successful model of partnership.

Chair: Dimitrios Athanasiou, World Duchenne Organisation

Speakers:

Dr. Elena Nicod, Dolon, Italy

SESSION 0504: Friday 15th May 2020, 14:00 – 15:30

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

We are seeing emerging narratives emphasising the strain that people living with a rare disease place on the overall healthcare system; yet at the same time, the general public continue to respond in their thousands to crowdfunding appeals, demonstrating an unprecedented sense of solidarity. Crowdfunding is, however, an unsustainable approach. How much is society willing to pay in 2030 for people living with a rare disease? Do we need a solidarity pact? Which future trends in rare disease therapies need to be taken into consideration?

Chair: Sheela Upadhyaya, HST and Topic Selection Specialist Centre for Health Technology Evaluation, National Institute for Health and Care Excellence, UK

Speakers:

Dimitrios Athanasiou, World Duchenne Organisation

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

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

Ana Palma, Senior Director Global HTA & Patient Access Lead, Swedish Orphan Biovitrum BVBA/SPRL, Belgium