PROGRAMME AT A GLANCE

All times listed are Central European Summer Time (CEST)

Wednesday 13 May 2020

10:00 - 13:15	EURORDIS Annual General Assembly (Members only)

Thursday 14 May 2020

10:30 - 11:30	Virtual Platform Navigation & Online Networking					
11:30 - 13:00	Opening & Plenary Session					
13:00 - 14:00	Lunch & Browsing Posters, Exhibit Hall and Networking					
14:00-14:45	Plenary Session: Rare2030 Foresight scenarios					
Themes	1/ The future of diagnosis: new hopes, promises and challenges	2/ Our values, our rights, our future: shifting paradigms towards inclusion	3/ Share, Care, Cure: Transforming care for rare diseases by 2030	4/ When therapies meet the needs: enabling a patient-centric approach to therapeutic development	5/ Achieving the triple As by 2030: Accessible, Available and Affordable Treatments for people living with a rare disease	6/ The digital health revolution: hype vs. reality
14:45-16:15	Session 0101	Session 0201	Session 0301	Session 0401	Session 0501	Session 0601
16:15 - 16:45	Comfort Break & Browsing Posters, Exhibit Hall and Networking					
16:45-18:15	Session 0102	Session 0202	Session 0302	Session 0402	Session 0502	Session 0602
18:15 - 19:30	Global Commission to End the Diagnostic Odyssey for Children with a Rare Disease: Progress Update					
18:15 - 19:30	Facilitated networking, Browsing Posters & Exhibit Hall					

Friday 15 May 2020

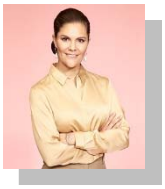
10:00-11:00	Plenary Session & Poster Winners Presentations					
11:00-11:30	Comfort Break & Browsing Posters, Exhibit Hall and Networking					
Themes	1/ The future of diagnosis: new hopes, promises and challenges	2/ Our values, our rights, our future: shifting paradigms towards inclusion	3/ Share, Care, Cure: Transforming care for rare diseases by 2030	4/ When therapies meet the needs: enabling a patient-centric approach to therapeutic development	5/ Achieving the triple As by 2030: Accessible, Available and Affordable Treatments for people living with a rare disease	6/ The digital health revolution: hype vs. reality
11:30 - 13:00	Session 0103	Session 0203	Session 0303	Session 0403	Session 0503	Session 0603
13:00 - 14:00	Lunch Break & Browsing Posters, Exhibit Hall and Networking					
14:00-15:30	Session 0104	Session 0204	Session 0304	Session 0404	Session 0504	Session 0604
15:30 - 16:00	Comfort Break & Browsing Posters, Exhibit Hall and Networking					
16:00 - 18:00	Closing Plenary Session					
18:00 - 19:00	Facilitated networking, Browsing Posters & Exhibit Hall					

OPENING SESSION AGENDA

Thursday, 14 May 2020 - 11:30 - 13:00

MODERATOR

Lise Murphy, Patient Advocate, Marfanföreningen (Swedish Marfan Association)



HRH Crown Princess Victoria of Sweden

WELCOME ADDRESS & OPENING REMARKS

Terkel Andersen, President, EURORDIS-Rare Diseases Europe

Ana Rath, Director, Orphanet

Maria Montefusco, President, Rare Diseases Sweden

KEYNOTE ADDRESSES

Lena Hallengren, Swedish Minister of Health & Social Affairs, Sweden

Helena Dalli, European Commissioner for Equality, Malta

Mariya Gabriel, Commissioner for Innovation, Research, Culture, Education & Youth, Bulgaria

INSPIRATIONAL SPEAKER

'Love, Support, Encouragement and Demands – Being a Policy Maker with a Rare Disease'

David Lega, Member of the European Parliament, Sweden

PLENARY SESSION

Thursday, 14 May 2020 - 14:00 - 14:45

RARE 2030 FORESIGHT SCENARIOS

Rare 2030 Overview

Prof. Milan Macek, Motol University Hospital and Charles University, Prague

Video: Rare 2030 "What If" Scenarios

Personal Perspective

Rebecca Skarberg, Osteogenesis Imperfecta Federation Europe (OIFE), Norway

Audience Voting

PLENARY SESSION

Friday, 15 May 2020 - 10:00 - 11:00

MODERATOR

Lise Murphy, Patient Advocate, Marfanföreningen (Swedish Marfan Association)

KEYNOTE ADDRESSES

'Discoveries for the Benefit of Man: Lessons from the Past and Hope for the Future'

Professor Anna Wedell, Member and Former Chair of the Nobel Committee for Physiology or Medicine, Sweden

'Life languages and red flags in the red sand'

Dr Gareth Baynam, Clinical Geneticist, Genetic Services of Western Australia

POSTER WINNERS

Introduced by:

Dr. Violeta Stoyanova-Beninska, Chair, Committee for Orphan Medicinal Products, EMA

1st Place: P 163 – A collaborative and patient-centric effort to find the first effective treatment for alkaptonuria. (**Nick Sireau**)

2nd Place: P 267 - 'Patient Journeys' : Personal experiences shaping clinical priorities (**Olivia Spivack**)

3rd Place: P 268 - Quality of life (QoL) for people with rare diseases: Recruitment challenges and consequences in a study pilot-testing the UK-PSC-QoL, a provisional QoL tool for people with primary sclerosing cholangitis (PSC) in the UK (**Elena Marcus**)

THEME 1

THE FUTURE OF DIAGNOSIS: NEW HOPES, PROMISES AND CHALLENGES

THEME LEADERS:

Prof. Christine Patch, Clinical Lead for Genetic Counselling, Genomics England, UK

Virginie Bros-Facer, Scientific Director, EURORDIS

THEME SUPPORT :

Gulcin Gumus, Research and Policy Project Manager, EURORDIS

THEME DESCRIPTION:

Recent scientific and technological developments have meant that the diagnosis of rare diseases has improved considerably over the last few years.

In this theme we will closely examine the current landscape and also debate future trends and scenarios. We will present the current state of play in several national Newborn Screening (NBS) programmes, and discuss challenges to expanding NBS across Europe, highlighting impacts for patients and families. This theme will also explore how new technologies can be applied to accelerate and improve access to diagnosis, taking into account the implications, opportunities and challenges that are associated with Next Generation Sequencing and Artificial Intelligence by showcasing several platforms. The diagnostic odyssey is still very much a reality for a vast number of rare disease patients despite these recent technological advances. Relevant tools and services will be discussed to understand how to better support the undiagnosed rare disease community.

Genetic counselling represents a critical milestone in the search for a diagnosis and is integral to Genetic Health Services. A dedicated session will present how partnerships and innovative ways of working can benefit all involved and improve care delivery.

SESSION 0101: Thursday 14th May 2020, 14:45 - 16:15

Rare 2030: How can we achieve faster and more accurate diagnosis?

We anticipate that the future will hold a shortened diagnostic odyssey. A number of advances in technology - such as whole genome sequencing as a first line practice (bringing it into the clinic) - present new opportunities to achieve this.

The future of diagnostics will include new trends: Big data and AI capabilities; New innovation such as WGS in the clinic; Patient engagement in the diagnostic process; Wearable technologies; Data platforms integrating many data sources (genetic, phenotypic etc.)

And old drivers of change: Continued raising of awareness; Networking of health care professionals for more efficient diagnosis (e.g. CPMS type system).

Chair: Prof. Milan Macek, Department of Molecular Genetics and National Cystic Fibrosis Centre, Motol University Hospital and Charles University, Prague

Speakers:

Anne-Sophie Chalandon, Head of European Rare Diseases Public Affairs and Patient Advocacy, Sanofi Genzyme, France

Dr. Lucy McKay, Chief Executive Office, Medics 4 Rare Diseases, UK

Dr. Lucy Raymond, Addenbrooke's Hospital & University of Cambridge, UK

Julian Isla, Data and Artificial Intelligence Resource Manager, Microsoft; Dravet Syndrome European Federation (DSEF); Founder, Fondation 29, Spain

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SESSION 0102: Thursday 14th May 2020, 16:45 – 18:15

Newborn Screening: Now and in the Future

The session will compare differing national approaches and explore the limits and potential of current and future approaches to newborn screening, exploring technical, societal, ethical and scientific considerations.

Chair: Jayne Spink, CEO, Genetic Alliance UK

Speakers:

Dr. Richard Scott, Clinical Lead for Rare Diseases, Genomics England

Nick Meade, Director of Policy, Genetic Alliance UK

Sara Hunt, Chief Executive Officer, Alex TLC

Prof. Martina Cornel, Amsterdam University Medical Centre, The Netherlands

Simona Bellagambi, UNIAMO (Italian Federation for Rare Diseases), Italy

SESSION 0103: Friday 15th May 2020, 11:30 – 13:00

Diagnosing Undiagnosed Rare Disease Patients: Tools and Resources to strengthen the voice of the undiagnosed Rare Disease Community

Progress in the application of genomic and other technologies (including web-based), has increased the diagnostic rate of patients with rare disorders to 50%. This is a great success but still leaves unanswered questions for the other 50% of the rare disease community. This session will focus on providing updates on existing initiatives of interest to the undiagnosed rare disease community, including patients, families and healthcare professionals.

The first part focuses on the views and voices of the undiagnosed community, their expectations of and outlooks on rare patients and their families. The overall aims of the session are to empower the community with tools and resources to strengthen their voices alongside policy makers and researchers, and to support them in getting closer to finding a diagnosis.

Chair: Dr. Holm Graessner, Solve-RD and ERN-RND, Institute of Medical Genetics and Applied Genomics, University of Tübingen, Germany

Speakers:

Prof. Christine Patch, Clinical Lead for Genetic Counselling, Genomics England

Dr. Holm Graessner, Solve-RD and ERN-RND, Institute of Medical Genetics and Applied Genomics, University of Tübingen, Germany

Lauren Roberts, Director of Support, Genetic Alliance UK, SWAN UK

Prof. Olaf Riess, Head of the Institute of Medical Genetics and Applied Genomics, University of Tübingen, Germany

Stephanie Broley, Senior Genetic Counsellor and Program Coordinator of the Undiagnosed Diseases Program WA, Australia

Dr. Pablo Botas, Head of Science, Fondation 29, Spain

Prof. Peter Krawitz, Institute for Genomic Statistics and Bioinformatics, University Hospital Bonn, Germany

Vanessa Lemarié, Lead Rare Disease Initiative , Business Development Life Sciences at Ada Health, Germany

SESSION 0104: Friday 15th May 2020, 14:00 – 15:30

What's Next After the Search for a Diagnosis? The Future of Specialised Health Services

This session will explore what happens after the completion of genetic analysis from the perspective of patients and health professionals. The session aims to explore what is important to patients regarding their potential diagnosis (or lack thereof) and future care pathways; to discuss innovative ways of working

with health professionals and patients to develop interventions which support these care pathways; and to appreciate the developing role of networks in the delivery of new approaches to aspects of care for rare diseases

Chair: Prof. Christine Patch, Clinical Lead for Genetic Counselling, Genomics England

Speakers :

Dr. Alessia Costa, King's College London, UK

Prof. Glenn Robert, King's College London, UK

Dr. Holm Graessner, Solve-RD and ERN-RND, Institute of Medical Genetics and Applied Genomics, University of Tübingen, Germany

Dr. Vera Frankova, Univerzita Karlova

Dr. Sofia Douzgou, ESHG Representative, Manchester Centre for Genomic Medicine, UK

THEME 2

OUR VALUES, OUR RIGHTS, OUR FUTURE: SHIFTING PARADIGMS TOWARDS INCLUSION

THEME LEADERS:

Maria Montefusco, President, Rare Diseases Sweden, Sweden

Valentina Bottarelli, Public Affairs Director and Head of European and International Affairs, EURORDIS

THEME SUPPORT :

Clara Hervas, Public Affairs Manager, European and International Affairs, EURORDIS

THEME DESCRIPTION:

Evidence demonstrates that people living with a rare disease and their families continue to face serious every day and social inclusion challenges. Rare diseases ensue in a high level of psychological, social and economic vulnerability and are detrimental to people's active participation in society.

This interlinkage between rarity, vulnerability, inequalities and social exclusion means that there is space for the integration of rare disease strategies into the broader human rights' agenda, and the health and development efforts at global level, with clear opportunities to contribute to the United Nations Agenda 2030: the Sustainable Development Goals (SDGs). This goal-based framework was agreed in 2015 by all UN Member States with the clear principle to "leave no one behind". In this sense, the SDGs are interdependent, universal goals that aim to address global challenges (such as poverty, health and climate).

The synergies between the SDGs and rare diseases have been acknowledged in different events, reports and texts of the UN, and there is momentum for international commitments for the benefit of persons living with a rare disease. In particular, the rare disease community has voiced the need to be included in efforts to achieve Universal Health Coverage (UHC) (as part of SDG 3 which focuses on health), as well as in efforts to ensure non-discrimination on the basis of health or disability status (as part of SDG 10 on reducing inequalities, or SDG 8 on decent work for example).

This theme will look at these ongoing advances in international advocacy and discuss the significance they have for different stakeholders of the rare disease community. A key goal of the theme will be to discuss how the rare disease community can translate those global commitments and aspirations into concrete regulatory practices and policies in the national context, which will have a real impact on the daily lives of those affected. Ongoing trends like reduced funding for human rights, an increasingly challenging political and social space, a debilitation of the values of solidarity and equity, and a shrinking space for civil society will be part of the debate, but the overall objective of the theme will be to identify the best practices and potential new practices that prove the value of investing in human rights and inclusion.

PRE-RECORDED SESSION: (Available on demand)

Getting our rights 'right': An international framework for rare diseases

Why should European citizens be concerned by the decisions being taken at the United Nations? Why should international collaboration continue to be fostered in the field of rare diseases? What sort of traction and impact can international frameworks have at the national level? To explore these questions, this session will first frame rare diseases as a policy priority, showcasing the EU approach and its framework established for addressing this issue. The session will then move on to frame rare diseases within the global human rights and Sustainable Development Goals (SDGs) agendas and present the advances made so far in international advocacy in engaging the United Nations bodies and agencies (UN General Assembly, Office of the High Commissioner for Human Rights, Human Rights Council…). Finally, the session will explore how to continue the progress towards a global t agenda that addresses the needs of persons living with a rare disease and encourages enforcement nationally, through tools such as UN resolutions.

Chair: Yann Le Cam, Chief Executive Officer, EURORDIS-Rare Diseases Europe

Speakers:

Anders Olauson, Chair, NGO Committee for Rare Diseases

Dr. Durhane Wong-Rieger, Canadian Organization for Rare Disorders

Raquel Peck, Senior Advisor and Former CEO of World Hepatitis Alliance, Switzerland

Todd Howland, Chief of the Development and Economic and Social Issues Branch, UN OHCHR

SESSION 0201: Thursday 14 May 2020, 14:45 – 16:15

UHC: from political commitment to reality for all

What is the value of Universal Health Coverage? What will the impact be in Europe: in terms of population, of services provided and of percentage of coverage? How will the EU implement the international commitments on UHC? Do national strategies on rare diseases successfully ensure the rights to health of persons living with a rare disease? How are patient's rights in cross-border care implemented across Europe and do they actually ensure access to health when this is not provided at country level? This session will look at these sorts of questions during a panel discussion on our current system and whether it may be outdated and in need of a paradigm shift. Possible trends like increased social investment, early intervention and prevention and promotion and the consequences of this on the balance between primary and secondary care will be part of the discussion.

Chair: Dr. Nata Menabde, Executive Director, WHO Office at the UN

Speakers:

Ilona Kickbusch, Director of the Global Health Programme, Graduate Institute of International and Development Studies, Geneva

Matt B. Johnson, Rare Diseases International Healthcare Advisor, WHO Collaborative Global Network for Rare Diseases, Germany

Martin Seychell, Deputy Director-General for Health and Food Safety, DG SANTE, European Commission, Belgium

Dr. Vytenis Andriukaitis, Former EU Commissioner for Health and Food Safety, Lithuania

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SESSION 0202: Thursday 14 May 2020, 16:45 – 18:15

Holistic Care for People Living with Rare Diseases: The Future is Now

"The specific nature of rare diseases also calls for a holistic, comprehensive and multi-disciplinary response, deeply grounded by essence in a human rights vision" (Dainius Puras, UN Special Rapporteur on the right of everyone to the enjoyment of the highest attainable standard of health). This session looks at what holistic care means in practice for persons living with a rare disease from a human rights framework and a societal point of view, rather than solely in terms of provision. It will particularly focus on the opportunities that holistic care offers, but will also look at the risks or hurdles that may be encountered to fully implement this. The session could also be an opportunity to look at how rights are interpreted in different scenarios, and therefore enshrined into and enforced (or not) within different models of care, which may depend on the direction that society as a whole chooses to take.

Chair: Robert Hejdenberg, CEO, Agrenska, Sweden

Speakers:

Ann Nordgren MD, PhD, professor in Clinical genetics at the Karolinska Institute and senior consultant at the Karolinska University hospital

Rebecca Tvedt Skarberg, Advisor, Osteogenesis Imperfecta Federation Europe (OIFE), Norway

Dr. Encarna Guillén, Head of Unit of Medical Genetics, Paediatric ward, Hospital Clínico Universitario Virgen de la Arrixaca, Spain

Dr. Cecilia Gunnarsson, Division of Clinical Genetics, Department of Clinical and Experimental Medicine, University Hospital, Linköping University, Linköping, Sweden

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SESSION 0203: Friday 15th May 2020, 11:30 – 13:00

Rare and Equal: Ensuring Non-Discrimination on the Basis of Health and Disability

People living with a rare disease often face discrimination and stigma based on their health status or chronic condition, experiencing unequal treatment in a number of areas ranging from access to education, employment, leisure and other essential support services. This is aggravated when the illness is invisible or changing. This session will present the major figures on this issue as well as personal stories and will look at the ways of enforcing these persons' rights (CRPD articles 5, EU Charter of Fundamental Rights,

European Pillar of Social Rights), particularly by ensuring appropriate regulatory processes and making use of existing tools.

Chair: Maria Montefusco, President, Rare Diseases Sweden

Speakers:

David Lega, Member of the European Parliament (MEP), Sweden

Jana Popova, EAMDA Executive Committee, Bulgarian Association for Neuromuscular Diseases & EPF Youth Group, Bulgaria

Inmaculada Placencia Porrero, Senior Expert Social Affairs, European Commission, DG for Employment, Social Affairs and Inclusion, Unit for Disability and Inclusion

Alarcos Cieza, Unit Head, Sensory functions, Disability and Rehabilitation, World Health Organisation

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SESSION 0204: Friday 15th May 2020, 14:00 – 15:30

Reaching Future Scenarios: From Accidental Progress to Success by Design

This session will present the scenarios currently being developed under the Rare2030 project and the different policy options associated to them. The session aims to be interactive, engaging the panellists and audience in a back-casting exercise and a vote on the preferred policy options.

Chair: Hans Winberg, Secretary General, Leading Health Care, Sweden

Speakers:

Terkel Andersen, President EURORDIS-Rare Diseases Europe

Rebecca Tvedt Skarberg, Advisor, Osteogenesis Imperfecta Federation Europe (OIFE), Norway

Prof. Milan Macek, Dept of Biology and Medical Genetics, Charles University and Orphanet Czech Republic

Anna Krohwinkel, Leading Health Care, Sweden

THEME 3

SHARE, CARE, CURE: TRANSFORMING CARE FOR RARE DISEASES BY 2030

THEME LEADERS:

Prof. Alberto Pereira, Coordinator of the European Reference Network on Rare Endocrine Conditions (Endo-ERN) & Leiden University Medical Centre, The Netherlands.

Dr. Birute Tumiene, Clinical Geneticist, Coordinator for Competence Centres, Vilnius University Hospital Santaros Clinics, Lithuania.

Matt Bolz-Johnson, ERN and Healthcare Advisor, EURORDIS

THEME SUPPORT :

Ines Hernando, ERN and Healthcare Director, EURORDIS

THEME DESCRIPTION:

Fast forward 20 years, the very fabric of our national health and welfare systems will be unrecognisable, transformed by the disruptive innovation and technologies of our modern age. Even today, many of our everyday services have already been radically transformed - private hire car companies now don't own any cars (Uber), retailers without shops (Amazon) and mobile-only banks (N26) are now common place. Our healthcare and welfare systems will not be immune to these changes. The rule book for healthcare will be re-written, and the once familiar building blocks of our traditional hospitals will be transformed into a new suite of health and social care competencies, on-the-go and on-demand experts and virtual clinics.

The digital transformation of healthcare has the potential to bring great benefits to the rare disease community, but it will also create new challenges. Our thirst for knowledge and unwavering scientific advancements will conscribe the diagnostic odyssey to our history books, with most rare diseases being diagnosed at birth or within the first year of the first symptom – all culminating to improve health outcomes, and resulting in a shift in the population needs and burden of care of an ageing rare disease community, facing different challenges of living longer with increased multiple co-morbidities. This survivorship effect will translate into a shift in our demand for different health and social services and service competencies. Adoption of technology, smart sciences and increasing automated health will force an evolution in the role of both health professionals and patients. The line between health care, social care and research will become increasingly blurred, as will our expectations of how care should be delivered.

Theme 3: Share, Care, Rare 2030: transforming care for rare diseases will explore the rare disease population needs in 2030-40 and explore both the opportunities and challenges of the care provision of the future. The seeds of our future are already visible today: the five sessions will put a spotlight on the emerging trends in best practice, promising technologies and cutting-edge thinking; showcase the forward-looking services and their potential to be scaled-up; and transform the way in which our healthcare will be delivered.

SESSION 0301: Thursday 14 May 2020, 14:45 – 16:15

"Live longer, healthier lives": Rare Disease Population Needs 2030 (and beyond)

Session 1: RD Population Needs 2030 (and beyond) will present the emerging policy trends and map the future population needs of the rare disease population, as well as scenario planning on the changing demographics, health inequalities modelling, horizon scanning on the availability of evidence-based services and treatments, and pinpointing the shift in the burden on care for an ageing population and the effect of increased survival. These emerging trends will shape healthcare, hospital systems and the integration of health and social care, for the next decade and beyond.

This session will present a high-level narrative on the four 'dimensions' of our future healthcare and hospital systems, specifically:

- Healthcare provision under mature ERNs (structure)
- Organisation of care under healthcare digital pathways (processes)
- Medical advancements and technology (innovation)
- Changes in role, profile and competency of medical teams and patient community (human resources)

Chairs: Prof. Kate Bushby, Institute of Genetic Medicine & Emeritus Professor, Newcastle University, UK

Speakers:

Prof. Alberto Pereira, ENDO-ERN Coordinator; Head of the Division of Endocrinology, Chair Centre for Endocrine Tumors Leiden (CETL), Leiden University Medical Centre, The Netherlands

Dr. Birute Tumiene, Institute of Biomedical Sciences, Faculty of Medicine, Vilnius University, Lithuania

Dr. Dalia Aminoff, Head of Patient Organisation, , AIMAR Onlus, Italy

Dr. Enrique Terol, Senior Policy Officer, Seconded National Expert, European Commission, Belgium

Victoria Hedley, Newcastle University John Walton Muscular Dystrophy Research Centre, UK

SESSION 0302: Thursday 14th May 2020, 16:45 – 18:15

ERN & CoE Accreditation as Quality Improvement Framework

The EUCERD Recommendations published in 2011 on the organisation of highly specialised healthcare were and remain ambitious. Even today, they continue to be relevant and far-reaching, with many countries still only beginning the process of implementing them. Session 2 will explore these key recommendations and conduct a deep dive into national recognition of expert centres and how European Reference Networks are developing.

Identification of experts in each and every Member State (MS) is the first step towards securing an accurate diagnosis and accessing appropriate care. National processes for endorsing rare disease expert

centres continue to be developed across EU MS. Endorsement and accreditation is a dynamic quality improvement process that incrementally raises the quality thresholds services need to meet to be approved. The future trend will be two-fold - universal coverage of national accreditation of highly specialised healthcare and rare diseases centres, and the incremental step-wise maturing of the accreditation process to come to a final result that will be measured on treatment outcome.

What will ERNs look like in 10-20 years' time? Session 2 will present the opportunities, benefits and challenges foreseen in a maturity ERN System. ERNs won't mature in isolation, but need to be fully integrated into national health systems, see recent Statement of the ERN Board of Member States on Integration of the ERNs to the healthcare systems of Member States.

The need for a more robust and universal care coordination across EU-ERN-wide care pathways, that are supported by shared care arrangements between hospitals as well as between health and social care. Future sustainability of many hospital systems on creating a fine balance between centralisation of supra-specialist care and shared care arrangements for local access, where the expertise travels, not the patient.

Chair: Dr. Birute Tumiene, Institute of Biomedical Sciences, Faculty of Medicine, Vilnius University, Lithuania

Speakers:

Prof. Alberto Pereira, ENDO-ERN Coordinator; Head of the Division of Endocrinology, Chair Centre for Endocrine Tumors Leiden (CETL), Leiden University Medical Centre, The Netherlands

Anke Widenmann-Grolig, KEKS & EAT, Germany

Prof. Till Voigtländer, Austrian Representative on ERN Board of Member States & Medical University of Vienna, Austria

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SESSION 0303: Friday 15th May 2020, 11: 30 – 13:00

Clinic of the Future & Digital Care Pathways

Session 3: Clinic of the Future & Digital Care Pathways will draw together the building blocks of the 'clinic of the future' and its clinical model, where research is fully embedded in daily clinical care; and its interface with other services along 'digital care pathways'. What will care look like under the clinic of the future? How will it feel to progress along the future 'digital care pathways'?

The healthcare we receive will be connected as 'networks of networks', beyond the traditional hospital building, to wider EU and global infrastructures. Centres of Expertise will act as comprise crucial hubs: they provide quality standards and connections with all stakeholders in the national network, and they are connected to EU-wide networks (for the further development of standards and implementation of all the activities, where ERNs provide economies of speed, scale and scope).

EU Green Corridors will connect hospitals across Europe, through digital healthcare pathways that are rooted in the latest evidence-based practice and support timely access, giving the 'green light' for people with a diagnosed rare disease to access the services when they need it. These healthcare pathways will provide transparency of care quality standards and centralize care only when necessary, but also make it close to home whenever possible.

Will we have witnessed the full impact of the genetic revolution on screening, surveillance, diagnosis and personalised treatment and hold real-world experience of gene therapy and genomic editing in the next 10-20 years? The two worlds of healthcare and research will be fully integrated in our clinic of the future, as we see happening today in a few countries, enabling undiagnosed rare disease patients to be fast track to research setting for an accurate diagnosis and advancing research with real-world evidence. Despite all our hopes and the pace of scientific development, there will always be some RD without specific treatment or for which the symptomatic treatments do not minimise all the complex impairments generated by the disease. For this reason, evidence-based clinical guidelines must be ensured for every disease, as well as coordinated care between health and social care.

Chair: Prof. Alberto Pereira, ENDO-ERN Coordinator; Head of the Division of Endocrinology, Chair Centre for Endocrine Tumors Leiden (CETL), Leiden University Medical Centre, The Netherlands

Speakers:

Prof. Dr. Daniel Hommes, Leiden University Medical Centre, The Netherlands

Dr. Sofia Douzgou, ESHG Representative, Manchester Centre for Genomic Medicine, UK

Prof. Dr. Helge Hebestreit, Universitäts-klinikum Würzburg, Germany

Dr. Valter Fonseca, Director of the Department for Quality in Health, Ministry of Health, Portugal

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SESSION 0304: Friday 15th May 2020, 14:00 – 15:30

Addressing Health Workforce Challenges and training the New Generation of Rare Disease Experts

Advancement in healthcare innovation and technology will lead the way in changing the competency profiles and skill mix of the healthcare workforce. The accessibility of medical information online and private online healthcare provision has already changed the doctor-patient relationship, with patients being more informed than ever, while the volume of new research and changing knowledge that healthcare professionals need to digest seems exponential. How far are we from seeing the role of doctors and surgeons being made redundant, where automated systems and artificial intelligence will have replaced them in traditional healthcare? Many may argue that the family doctor has already been made redundant thanks to this online medical revolution.

This has been the longstanding modus operandi for those living with a rare disease because they are experts in their condition. A single family doctor has never been their primary source for information. With increased health literacy, wearable technology, and direct-to-consumer genetic testing, more people than ever are taking their health into their own hands. However, what are the risks of over-relying on technology and losing the relationship with a healthcare professional? With the changing role of patients there is sure to be a change ahead for healthcare professionals. Are generalists obsolete or do they just need to be given the tools to evolve? What are the skills that our next generation of healthcare professionals need to adjust to this new environment and complement innovation?

For those with rare diseases there cannot be any doubt that greater inter-connectivity and patient power has been beneficial. However there is potentially a fly in the ointment - there's a difference between a patient who's done a rudimentary google search about some transient symptoms and an expert patient

who is one of a handful with a specific condition in the country. How will doctors be able to distinguish between the two when they come to them holding out printed information to be read and understood in a 10-minute appointment? Is the term "expert patient" being devalued?

Will the breaking down of our traditional healthcare systems see a similar overhaul of the medical training system underpinning them? Not just moving from classroom-based education to knowledge-sharing online communities, but performing a review of what is being taught and how much emphasis is being placed on it. For example, should physical examinations still include the search for late-onset stigmata of diseases that should be caught earlier in their natural history with standard diagnostic tests? What other signs could replace these that would be more informative?

Our next generation of experts are today's medical students and doctors in training. Given the changing patient and doctor roles, what skills do future doctors require in order to deliver evidence-based and compassionate care? What will the world look like when they graduate in 10 years' time and beyond? Is current medical education moving fast enough that what a medical student has learned during their studies is irrelevant by the time they graduate? What skills and knowledge will they need to learn to work in collaboration with innovative health solutions?

People with rare diseases often share their stories about disbelieving and unsympathetic doctors. Perhaps the changing role of doctors needs to be dominated more by what has been coined as 'soft skills' such as communication skills, interpersonal skills and leadership skills, leaving the pattern matching to the machines. After all, technology is only ever as good as the information you put into it. Obtaining a thorough and accurate history, while making the patient feel listened to and at ease, is an art that needs a lot of practice.

Focusing on staffing the medical community of the future, where are the gaps in the workforce and is the ever-continuing supra-specialisation creating an imbalance in our medical workforce? What is the appropriate workforce skill-mix in an individual nation and across Europe? We need to plan today in order to have the workforce in place that will address the needs of the patient population in ten years' time. Many countries are facing a brain-drain - how can we preserve our expertise, knowledge and experience? How can we get better at sharing knowledge and expertise and finding new ways of collaborating to provide cross-border healthcare?

Potentially the changing role of the patient and healthcare innovation could work favourably to make up for challenges caused by gaps in the workforce. However, as experienced by many rare diseases, innovation doesn't always mean change and can be hindered by evolutionary lag in training and system updates. As we look to 2030 we discuss how best to ready the workforce so that the full potential of innovation can be realised.

Chairs: Dr. Lucy McKay, CEO, Medics 4 Rare Diseases, UK

Speakers:

Prof. James Buchan, Queen Margaret University Edinburgh, UK

June Rogers, Paediatric Continence Specialist, Bladder & Bowel, UK

Dr. Svetlana Lagercrantz, Chair of the ERN GENTURIS Task Force on Education & Training, Sweden

THEME 4

WHEN THERAPIES MEET THE NEEDS: ENABLING A PATIENT-CENTRIC APPROACH TO THERAPEUTIC DEVELOPMENT

THEME LEADERS:

Diego Ardigo, Global Rare Diseases R&D Head, Chiesi Farmaceutici, Italy & Chair, Therapies Scientific Committee of IRDiRC

Virginie Hivert, Therapeutic Development Director, EURORDIS and vice-Chair, Therapies Scientific Committee of IRDiRC

THEME SUPPORT:

Maria Cavaller, Patient Engagement Junior Manager, EURORDIS

THEME DESCRIPTION:

The objectives of this theme are to take stock of the experience gained so far in the development of medicines for people living with rare diseases, and to examine the evolution of the field. We will look at recent scientific innovations and clinical research, regulatory solutions, roadblocks and challenges in developing therapies that match the needs of the patients, as well as ways of embedding real life evidence into the therapeutic development processes.

PRE-RECORDED SESSION: (Available on demand)

Galaxy Guide for Rare Disease therapies development

Presenters: Dr. Diego Ardigò, Chair, Therapies Scientific Committee of IRDiRC and Global Rare Disease R&D Head, Chiesi Farmaceutici, Italy & **Dr. Virginie Hivert,** Therapeutic Development Director, EURORDIS & Vice-Chair, Therapies Scientific Committee of IRDiRC

The Orphan Drug Development Guidebook Task Force was set up within the International Rare Disease Research Consortium (IRDiRC) with the aim to create a guidebook describing the available tools and initiatives specific to rare disease development and how to best use them, in order to address the multiple challenges inherent to drug development for rare diseases for which the traditional model of development is becoming less and less suitable.

The aim of the Guidebook is to benefit the various stakeholders working in the orphan drug development environment. It includes fact-sheets describing each tool or resource (covering a large number of initiatives that are available worldwide), a series of standard use cases defining how and when to use them, and a series of practical checklists of items to consider at each step of the development. Integration of such elements within a defined drug development framework is set out to generate better data quality, shorter development timelines, and better R&D efficiency.

SESSION 0401: Thursday 14th May 2020, 14:45 – 16:15

What do patients expect from therapy development?

This session aims to look at the present and future of medicine's development and to reflect what patients are expecting for the next decade.

Building on results from the Rare Barometer Surveys (e.g. RD patients' experience with accessibility to treatments), the current work around patient engagement and a few figures illustrating the current state-of-play of therapies development for rare diseases, will also help to set the scene.

A panel of patients representing different rare disease areas, with expertise in several aspects of the medicine's life-cycle and engaging with the ecosystem in a variety of capacities (EMA, EURO-CAB, IMI PARADIGM on sustainable patient engagement, ERNs, HTA, etc.) will discuss the actual challenges, the needs and main expectations vis-à-vis the development of medicines, and the way for each stakeholder to contribute to improving RD patients' lives.

Chair: Dr. Virginie Hivert, Therapeutic Development Director, EURORDIS & Vice-Chair, Therapies Scientific Committee of IRDiRC

Speakers:

Loris Brunetta, Thalassaemia International Federation, Italy

Alain Cornet, Lupus Europe, Belgium

Veronica Popa, MCT8-AHDS Foundation, Romania

Eva Stumpe, SMA Europe, Germany

Russell Wheeler, Leber's Hereditary Optic Neuropathy Society, UK

SESSION 0402: Thursday 14th May 2020, 16:45 – 18:15

Disruptive Innovations in clinical research

This session will focus on innovative trends in clinical research, both in study design and execution, as well as innovative approaches to data collection. We will discuss the opportunities and challenges posed by these developments, together with the challenges foreseen in terms of regulatory and HTA assessment and the impact for the rare disease patients.

Attendees will leave this session with a broader view and understanding of the opportunities and challenges generated by current changes to how clinical research is conceived and executed, and the impact these changes will have on evidence generation in the future.

Chairs: Dr. Diego Ardigò, Chair, Therapies Scientific Committee of IRDiRC and Global Rare Disease R&D Head, Chiesi Farmaceutici, Italy & **Dr. Violeta Stoyanova-Beninska**, Chair, Committee for Orphan Medicinal Products, EMA

Speakers:

Dr. Simon Day, Clinical Trials Consulting & Training, UK

Dr. Nigel Hughes, Janssen Research and Development, Belgium

Prof. Armando Magrelli, Istituto Superiore di Sanità, Italy

Elizabeth Vroom, World Duchenne Organization, Netherlands

Pooja Merchant, Bayer, USA

Prof. Veronica Miller, University of California, USA

SESSION 0403: Friday 15th May 2020, 11:30 – 13:00

Innovation in Advanced Therapy

In this session, we will dive into the development and use of an ATMP from idea to approval and beyond. Using a particular product as an example, we will look at it from every angle: developer, patient, physician and regulator. Participants should leave this session with a greater understanding of the challenges relating to developing an ATMP and the subsequent use of it in clinical practice, which can be rather different from a standard product.

Chair: Dr. Kristina Larsson, Head of Orphan Medicines, European Medicines Agency

Speakers:

Tomasz Grybek, Fundacja Bohatera Borysa (Boris the Hero Foundation), Poland

Michela Gabaldo, Head of Alliance Management & Regulatory Affairs, Fondazione Telethon, Italy

Patrick Célis, European Medicines Agency, The Netherlands

SESSION 0404: Friday 15th May 2020, 14:00 – 15:30

Bringing real life into therapeutic development

The patient should be central to all aspects of drug discovery, development, regulatory approval and future evidence generation of medicines, ensuring a complete life cycle approach to patient engagement. This session showcases where patients can bring their real-life experiences into different areas of the drug development pathway. We will explore how patient engagement in clinical trial design is increasingly valued, ensuring that studies capture what is important to measure from the end user perspective.

Patient reported outcomes (PRO) measure how a patient feels and functions whilst on a therapy. Developing PRO standards ensures robust data collection and interpretation, adding value to the information available about the patient experience whilst on a therapy.

The European Reference Networks facilitate discussions on rare diseases, concentrating knowledge and allowing for the collection of real world data which can be used to learn more about rare conditions and available therapies. Drug repurposing is a hot topic and an area where rare disease groups are now often leading the way, directing the development pathway for the benefit of their patient group.

Chairs: Dr. Daria Julkowska, Assistant Director, Institute GGB; Coordinator, European Joint Programme on Rare Diseases & **Dr. Daniel O'Connor,** Medical Assessor, Medicines and Healthcare Products Regulatory Agency (MHRA)

Speakers:

Dr. Anja Schiel, Norwegian Medicines Agency

Prof. Faisal Ahmed, Endo-ERN EuRRECa, UK

Dr. Madeline Pe, EORTC, Belgium

Dr. Nick Sireau, AKU Society, UK

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

Dr. Orla Galvin, Director of Research Policy, Retina International, Dublin

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), the Netherlands

Speakers:

Prof. Hildegard Büning, European Society for Gene and Cell Therapy (ESGCT), Germany

Simone Boselli, Public Affairs Director, (EURORDIS)

Adam Hutchings, Managing Director, Dolon Ltd

Declan Noone, European Haemophilia Consortium, Ireland

Evert Jan Van Lente, AOK Health Insurance, Germany

Lonneke Timmers, Advisor, Zorginstituut Nederland (ZIN), the Netherlands

Francis Pang, Vice President, Global Access, Orchard Therapeutics, UK

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 Ltd, Italy

Toon Digneffe, Head of Public Affairs and Public Policy - Rare Disease Franchise Europe & Canada, Takeda, Belgium

Dr. Donato Bonifazi, Consorzio per Valutazioni Biologiche e Farmacologiche, Italy

Josie Godfrey, Director, JG Zebra Consulting, UK

Dr. Daria Julkowska, Assistant Director, Institute GGB; Coordinator, European Joint Programme on Rare Diseases

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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

THEME 6

THE DIGITAL HEALTH REVOLUTION: HYPE VS. REALITY

THEME LEADERS:

Julián Isla, Data and Artificial Intelligence Resource Manager, Microsoft & Chief Scientific Officer, Dravet Syndrome European Foundation, Spain.

Justina Januševičienė, Head of Healthcare Innovation Development Centre, Lithuanian University of Health Sciences, Lithuania.

Brian O'Connor, Chair, European Connected Health Alliance, UK/Ireland

THEME SUPPORT :

Denis Costello, Executive Director, CML Advocates Network, Spain.

Marta Campabadal, RareConnect Manager, EURORDIS

THEME DESCRIPTION:

This theme examines the technological innovations that are underpinning disruption in medicine and science, as well as the legal, ethical and policy foundations that can frame future outcomes in this area. The theme will also look at how technology can support the social inclusion of people living with a rare disease.

Attendees should come away from this theme with a greater understanding of the role of quality data in technologies such as Artificial Intelligence and how this impacts the development of medicines and delivery of care and other services. The theme aims to question the value of such technological innovations, as well as to show the policy frameworks and ecosystems which patient representatives can involve themselves in, in order to bring the patient's voice to the evolution of policy and ethics in this area.

SESSION 0601: Thursday 14th May 2020, 14:45 – 16:15

Do we need a New Patient Data Management Model for AI?

Artificial Intelligence seems to be the holy grail, promising to solve the many challenges faced by the current healthcare system. What do we need in order for models based on Artificial Intelligence to work? The answer: large datasets for training those models. These large datasets have usually been safeguarded by hospitals, the traditional centres of medical care.

But this centralized hospital data model is giving way to a hybrid system, where data is generated not only by the hospital but also by the patient and other sources. In addition we have the problem that certain data (such as genomic data) is too large to be stored within the traditional medical information system based in hospitals. Perhaps we need new data management models that allow the capture, processing and analysis of medical data generated by various sources. This question is particularly significant for rare disease patients as they often have to visit multiple specialists in different healthcare centres. In this session we will discuss the new data management models we will need, and potential challenges that may arise (medical, regulatory, security, privacy, ethics).

Chairs: Denis Costello, Executive Director, CML Advocates Network, Spain

Speakers:

Paul Rieger, Managing Director, Centiva Health, Austria

Peter Speyer, Head of Products, data42, Novartis, Switzerland

Dr. Marco Roos, GO FAIR, Netherlands

Nicholas Becker, AI for Good, Microsoft, USA

SESSION 0602: Thursday 14th May 2020, 16:45 – 18:15

Considerations Surrounding the Ethical and Legal Aspects of Data Governance

The current COVID-19 crisis has brought into sharp relief some of the big challenges on the ethical and legal aspects of data governance, most significantly in the debate on the use contact tracing apps as a key tool in the transition out of lockdown and the management of a 'new normal'. The use of such apps highlights the fragile and complex balance between public and private interests in data, the need to build trust in data governance systems and the need for effective mechanisms for data solidarity. In this session you will hear speakers address all of these issues, providing insights on how the balance between public and private is seen in our rare disease communities as well as about exciting developments in new approaches to making data available for research (Findata) and facilitating data solidarity (Salus Coop).

Chair: Dr. Petra Wilson, Managing Director and CEO, Health Connect Partners, UK

Speakers:

Dr. Bettina Ryll, Founder and President, Melanoma Patient Network Europe

Rosa Juuti, Senior Specialist, Findata, Finland

Sandra Courbier, Social Research Director, Rare Barometer Voices, France

Joan Guanyabens, Consultant Health IT and Innovation, Salus Coop, Spain

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SESSION 0603: Friday 15th May 2020, 11:30 – 13:00

Getting Solutions to Patients Quicker and more Effectively: Are Policy, Procurement and Patients the Enablers or the Obstacles?

What changes are necessary to get solutions to patients quicker and more effectively? The speakers will share their own experiences and express their own perspectives on whether or not policy, procurement and patients are the enablers or obstacles. This interactive session will seek consensus on concrete actions to overcome any obstacles and map out a way forward.

Chair: Brian O'Connor, Chair, European Connected Health Alliance, UK/Ireland

Speakers:

Prof. George Crooks, Digital Health & Care Institute, Scotland

Jaana Sinipuro, Project Director, IHAN – Human-Driven Data Economy, SITRA, Finland

Liz Ashall-Payne, Founder and CEO, ORCHA, UK

SESSION 0604: Friday 15th May 2020, 14:00 – 15:30

Technology for inclusion and empowerment

This session will highlight the possibilities of empowerment and inclusion presented by innovative technology. You will hear about a number of good examples around tech-tools, digital accessibility and policymaking that can be used as means and stepping stones to reach full holistic inclusion.

Chairs: Robert Hejdenberg, President, Agrenska

Speakers:

Dr. Stefan Johansson, KTH Royal Institute of Technology, Sweden

Daniel Forslund, Assistant Regional Council in the Stockholm Region, Sweden

Allison Watson, Patient Advocate, Ring20, UK

Veronica Popa, Patient Advocate, Andreas-Rareș Association, Romania

Ana Neacşu, Campus Research Institute, Romania

Tim Buckinx, Founder and CEO, Epihunter, Belgium

Michael Lovgren, CEO Assistant, Agrenska, Sweden

Fredrik Ruben, Chief Executive Offficer, Tobii Dynavox, Sweden

CLOSING PLENARY SESSION AGENDA

Friday, 15 May 2020 - 16:00 - 18:00

MODERATOR

Lene Jensen, Director, Rare Diseases Denmark

HIGHLIGHTS AND TAKE-HOME MESSAGES FROM THE PARALLEL THEMES:

- **Theme 1: Prof. Christine Patch,** Clinical Lead for Genetic Counselling, Genomics England

- **Theme 2: Maria Montefusco**, President, Rare Diseases Sweden

- **Theme 3: Dr. Birute Tumiene,** Clinical Geneticists & Coordinator for Competence Centres, Vilnius University Hospital Santaros Clinics, Lithuania

- **Theme 4: Dr. Diego Ardigò,** Chair, Therapies Scientific Committee of IRDiRC and Global Rare Disease R&D Head, Chiesi Farmaceutici, Italy

- **Theme 5: Dimitrios Athanasiou,** World Duchenne Organisation (TBC)

- **Theme 6: Denis Costello,** Executive Director, CML Advocates Network, Spain

PERSONAL TAKE-HOME MESSAGES

Jana Popova, Young Patient Advocate, Bulgarian Association for Neuromuscular Diseases; European Patients' Forum Youth Group; European Alliance of Neuromuscular Disorders

CONCLUSIONS

Yann Le Cam, Chief Executive Officer, EURORDIS

PRE-CONFERENCE DAY

Wednesday, 13 May 2020

10.00 – 13.30 CEST EURORDIS General Assembly (members only)

The EURORDIS General Assembly documents (Activity report, Finance report, Action Plan 2020 and Budget 2020, Board candidates) will be posted here:

https://www.eurordis.org/content/membership-meetings

two weeks before the General Assembly. A video presenting these documents will be posted on the same page one week before the General Assembly.

The voting and election of EURORDIS Board members will take place online, 13th May, on a dedicated voting platform.

All full members of EURORDIS who have paid their membership fee 2020 will receive a link to the voting platform with a password, allowing them to vote.

Please note, you can either:

- Register to attend the General Assembly online **only** (13th May) by filling in the form;

OR

- Register for the General Assembly as part of your registration to participate in the European Conference on Rare Diseases & Orphan Products 2020 (now taking place 100% online, 14th - 15th May)