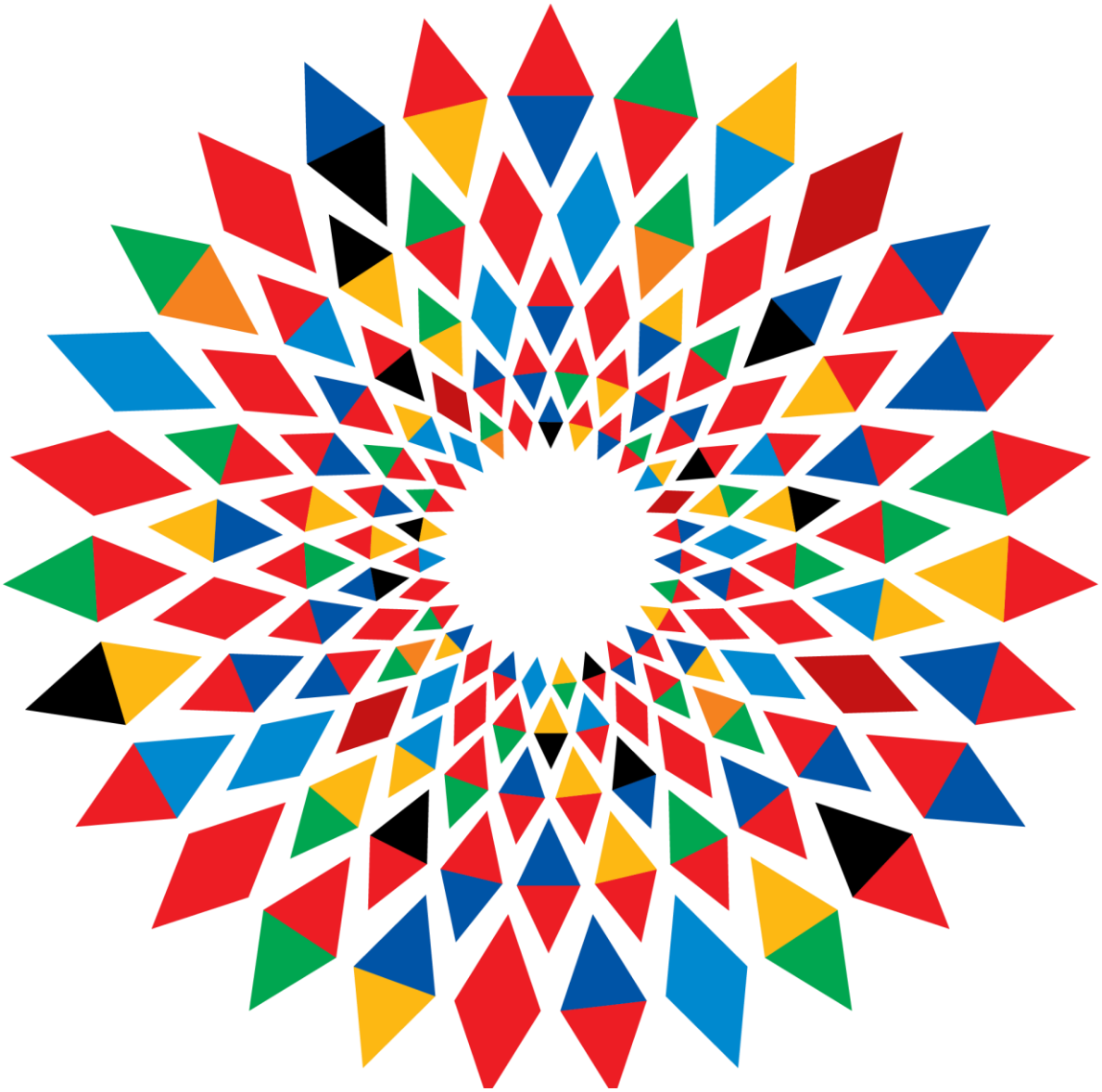
EU2022.CZ

Czech Presidency of the Council
of the European Union



Programme

Expert Conference on Rare Diseases

Towards a New European Policy Framework:

Building the future together for rare diseases

Prague, Czech Republic

Conference brochure:

Tuesday, 25. 10. 2022

Time	Programme	Speaker
13:00 – 14:00	Registration and welcome coffee	
14:00 – 14:10	Welcome remarks from the CZPRES	**Mr. Vlastimil Válek** Deputy Prime Minister and Minister of Health of the Czech Republic
14:10 – 14:20	Remarks from the European Commission	**Mr. Andrzej Rys** Principal Scientific Adviser, EC
14:20 – 14:30	Remarks from the previous French Presidency	**Ms. Anne Sophie Lapointe** Ministry for Solidarity and Health, France
14:30 – 14:40	Remarks from the upcoming Swedish Presidency	**Mr. Thomas Linden** Government Chief Medical Officer, Sweden
14:40 – 16:00	**SESSION I. – A NEW GOAL-BASED AND COORDINATED STRATEGY FOR RARE DISEASES** (Moderator Ms. Anna Arellanesová)	
14:40 – 15:15	Series of keynote presentations	
14:40	Presentation of the Call to Action and proposal for the European Action Plan for Rare Diseases	**Mr. Jakub Dvořáček,** Deputy Minister, Ministry of Health, Czech Republic
14:50	Investing in Rare Diseases Research: a European long-standing commitment	**Ms. Irene Norstedt**, Director "People", Directorate-General for Research and Innovation, European Commission

14:55	Why Europe's Action Plan for Rare Diseases is critical for our community	**Mr. Yann le Cam** CEO, Eurordis-Rare Diseases Europe
15:15 – 16:00	Moderated panel discussion: Working together with rare disease patient representatives (Moderator Ms. Anna Arellanesová)	
	A message from a young patient advocate	**Ms. Adéla Odrihocká** Rare Diseases Czech Republic
	Patient life-journey, need for holistic view	**Mr. Anders Olauson** President of Agrenska, and Chairman of "RareResourceNet" a European Network of Rare Diseases Resource Centres
	Patient advocacy - in the field of rare disorders in Norway	**Ms. Lisen J. Mohr** Representative of the Norwegian rare disease resource centre, Frambu.no
16:00 – 16:30	**Coffee break**	
16:30 – 18:00	**SESSION II. – EARLY DIAGNOSIS FOR RARE DISEASES** (Moderator Mr. Milan Macek)	
16:30 – 17:15	Series of keynote presentations	
16:30	Importance of neonatal Screening for the early diagnosis of rare diseases	**Mr. Maurizio Scarpa** Coordinator MetabERN and representative of Screen4Rare
16:45	The Rare Diseases Partnership – improving R&I potential and accelerating clinical trial readiness of the rare diseases	**Ms. Daria Julkowska** Scientific Coordinator of the European Joint Programme on Rare Diseases

17:00	Orphanet contribution to improving rare disease diagnostic path	**Ms. Ana Rath** Orphanet Director France
17:15 – 18:00	Moderated panel discussion: Early diagnosis: from newborn screening to personalized patient care (Moderator: Mr. Viktor Kožich)	
	IVDR 2017/746: quite a challenge for new tests for rare diseases to preserve the final purpose of the regulation. Call for embedding an incubation period	**Ms. Els Dequeker** Representative of European Society of Human Genetics and EuroGentest
	Key indicators of newborn screening: International context and future perspectives for cooperation	**Mr. Ondřej Májek** Representative of the National Screening Center of the Institute of Health Information and Statistics
	Newborn dried blood spot screening and follow up in Sweden	**Mr. Rolf Zetterström** Representative of Karolinska University Hospital
18:00 – 18:15	**CONCLUSION – DAY 1**	
18:00	**Summary - Session I. and II.**	**Mr. Milan Macek** National Coordination Centre for Rare Diseases UH Motol and Charles University
18:10	**Closing remarks - Day 1**	**Mr. Jakub Dvořáček** Deputy Minister, Ministry of Health of the Czech Republic
19:30	**Evening reception** (Corinthia Hotel Prague, Bellevue Hall, 24th floor)	

Wednesday, 26. 10. 2022

Time	Programme	Speaker
09:00 – 10:30	**SESSION III. – REVISION OF THE ORPHAN DRUG AND PAEDIATRIC DRUG REGULATIONS** (Moderator: Mr. Tomáš Mlčoch)	
9:00 – 09:20	Series of keynote presentations	
09:00	Title of the presentation (TBA)	**Ms. Olga Solomon** Head of Unit Medicines: Policy, Authorisation and Monitoring, DG SANTÉ
09:10	Evidence for orphan and paediatric medicines – challenges and opportunities	**Mr. Michael Berntgen** Head of Scientific Evidence Generation Department European Medicines Agency
09:20 – 10:30	Moderated panel discussion: Improved provision of innovative medicines to rare diseases patients (Moderator: Ms. Kateřina Kopečková)	
	How to make treatments for rare diseases less rare	**Ms. Nathalie Moll** Representative of the EFPIA
	The OMP Regulation review - developers perspective and reflections on the way ahead	**Ms. Vittoria Carraro** Representative of Orphan Drug Incentives / EUCOPE
	The revision of the OMP regulation - perspectives from the Rare Disease Community	**Ms. Virginie Hivert** Representative of the EURORDIS-Rare Diseases Europe
10:30 – 11:00	**Coffee break**	

11:00 – 12:00	**SESSION IV. – INSTRUMENTS FOR IMPROVING ACCESS TO RARE DISEASES TREATMENTS** (Moderator: Mr. Jakub Dvořáček)	
11:00 – 11:30	Series of keynote presentations	
11:05	Access to therapies: how to address the systemic failures with innovative tools	**Mr. Yann Le Cam** Chief Executive Officer EURORDIS-Rare Diseases Europe
11:15	United Action for Better Health; leave no-one behind	**Ms. Sarah Garner** World Health Organization
11:30 – 12:00	Moderated panel discussion: Improving access to diagnostics and treatment in rare diseases (Moderator: Mr. Jakub Dvořáček)	
	Pan-continental ERN based data sharing for solving the unsolved RD in Europe	**Mr. Holm Graesner** ERN-RND, Center for Rare Diseases, Tübingen
		Mr. Declan Noone Representative of the European Haemophilia Consortium
	Improving access to diagnostics and treatment in rare diseases	**Ms. Anna Bucsics** Representative of the Mechanism of Coordinated Access to Orphan Medicinal Products
12:00 – 13:00	**Buffet lunch**	

13:00 – 14:20	**SESSION V. – HOLISTIC HEALTHCARE PATHWAYS: INTEGRATING ERN INTO EUROPEAN HEALTH CARE AND SOCIAL SYSTEMS** (Moderator: Ms. Pavla Doležalová)	
13:00 – 13:30	Series of keynote presentations	
13:00	The European Reference Networks at the service of national EU health networks for Rare Diseases	**Mr. Alexis Arzimanoglou** Coordinator of the European Reference Network for Rare and Complex Epilepsies (EpiCARE), Chair of the ERN Coordinators Working Group
13:10	European Reference Networks: towards equity in rare diseases	**Ms. Birute Tumiene** Head of Unit, Center for Medical Genetics, Vilnius University Hospital
13:20	The future Joint Action on Integration: one key stimulus towards multifaceted and holistic healthcare pathways for Rare Diseases in Europe	**Mr. Till Voigtländer** Co-chair of the Board of Member States for ERNs
13:30 – 14:20	Moderated panel discussion: European Reference Networks for rare diseases as key hubs of research and medical / social care for rare diseases (Moderator: Ms. Pavla Doležalová)	
	Improving diagnosis for Rare Diseases in Europe – impact of European Reference Networks	**Mr. Holm Graessner** ERN-RND, Center for Rare Diseases, Tübingen
		Mr. Maurizio Scarpa Coordinator MetabERN and representative of Screen4Rare
		Ms. Anne Sophie Lapointe Ministry for Solidarity and Health, France

14:20 – 14:45	CONCLUSION – DAY 2	
14:20	**Summary of the discussion from Session III. to V.**	**Mr. Yann Le Cam** CEO, EURORDIS-Rare Diseases Europe
14:30	**Call to Action** – Invitation to support	**Mr. Jakub Dvořáček** Deputy Minister, Ministry of Health of the Czech Republic
14:35	**Closing remarks**	**Mr. Vlastimil Válek** Deputy Prime Minister and Minister of Health of the Czech Republic