Towards an EU action plan on rare diseases
W kierunku unijnego planu działania dla chorób rzadkich

10 April 2025 | 09:00 - 17:30
Conference / *Konferencja*

11 April 2025 | 08:45 - 18:00
Side event / *Wydarzenie towarzyszące*

Medical University of Warsaw / *Warszawski Uniwersytet Medyczny*

PROGRAMME OF THE CONFERENCE

TOWARDS AN EU ACTION PLAN ON RARE DISEASES: NATIONAL PLANS AND EUROPEAN COOPERATION

10 APRIL 2025

Venue: 2a Księcia Trojdena Street, 02-109 Warsaw, Poland

Simultaneous interpretation provided from/to English, French and Polish.

08:00 | REGISTRATION

09:00 – 09:30 | OPENING SESSION

Welcome: Rafał KRENKE, Rector, Medical University of Warsaw, Poland

- **Izabela LESZCZYNA**, Health Minister, Poland
- **Baiba MILTOVIČA**, President of the Section for Transport, Energy, Infrastructure and the Information Society, EESC
- **Olivér VÁRHELYI**, European Commissioner for Health and Animal Welfare (tbc)
- **Adam JARUBAS**, Chair of the European Parliament Committee on Public Health

09.30 – 10.45 | SESSION 1
Towards an EU action plan on rare diseases: national plans and European cooperation – state of play and future actions

Moderator: Enrique TEROL, Health Counsellor, Permanent Representation of Spain to the EU

- **Urszula DEMKOW**, Undersecretary of State, Ministry of Health, Poland: "Poland's national strategy on rare diseases"

- **Anne-Sophie LAPOINTE**, Project Manager of Rare diseases mission, Ministry of Labour, Health, Solidarity and Families, France: "Main outcomes and lesson learned from the French national strategy on rare diseases"
- **Agnete VALE NIELSEN**, Director of Hospital Planning, Health Authority, Denmark: "The evaluation of the Danish national strategy for rare diseases" (tbc)
- **Donata MERONI,** Head of Unit, Health monitoring and cooperation, Health networks, DG SANTE, EC: "Towards an EU action plan on rare diseases: next steps"

Debate

10.45 – 11.15 | COFFEE BREAK

11:15 – 12:30 | SESSION 2
Organisation and management of healthcare systems for patients with rare diseases

Keynote Speech: **Krystyna SZYMAŃSKA**, Professor at the Medical University of Warsaw, Poland – showcase rare disease patient

Moderator: Josep FIGUERAS, Director Emeritus of the European Observatory Health Systems and Policies

- **Luca SANGIORGI**, ERN BOND Coordinator, Coordinator of the ERNs Coordinators Group: "ERNs 8 years' experience on integration into the national healthcare systems: barriers and enablers"
- **Eileen TREACY**, Clinical Professor of Medicine of Rare Disorders, Trinity College Dublin, Ireland: "Development of efficient patient pathways and referral system: the EU dimension: JARDIN (The Joint Action on Integration of ERNs into National Healthcare Systems)"
- **Juan Fernando MUÑOZ**, Secretary General of Digital Health and Innovation, Ministry of Health, Spain: "New models of national referral systems: the UNICAS approach in Spain"
- **Lori RENNA LINTON**, EuroHSP: "The rare diseases patient journey experience"

Debate

12:30 – 13:30 | SESSION 3 Access to treatments: key issues and new approaches

Moderator: Alexis ARZIMANOGLOU, Professor, Coordinator of the European Reference Network for Rare and Complex Epilepsies, ERN EpiCARE

- **Kaja KANTORSKA**, Policy Officer, Unit of Medicines: Policy, Authorisation and Monitoring, DG SANTE, EC: "Regulatory options and pull and push incentives"
- **Daria JULKOWSKA**, INSERM, ERDERA: "The example of the European partnership ERDERA to boost clinical research and innovation"
- **Eulàlia OLESTI**, Regulatory Affairs Lead, Hospital Clinic Barcelona, Spain: "Academic CAR-T Clinical Development: The Case of ARI-0001"
  Katarzyna PIOTROWSKA-RADZIEWICZ, Director, Department of Drug Policy and Pharmacy, Ministry of Health, Poland: Orphan drugs in Poland - access, new developments, key issues
  Debate

13:30 – 15.00 | LUNCH

15.00 – 15.30 | SESSION 4
Solidarity and cooperation on rare diseases with Ukraine

Moderator: Urszula DEMKOW, Undersecretary of State, Ministry of Health, Poland

- **Tacjana WILCZYŃSKA**, Head of Clinic of Children Palliative Care, Consultant for Mobile Palliative Care Unit, Kyiv City Diagnostic Centre, Ukraine
- **Tatiana MAKAREVICH,** Public Health Specialist, Representative of WHO in Poland: the initiatives and outcomes of Poland's support to Ukraine
- **Ruth LADENSTEIN**, ERN PaedCan, Vice-Chair of the ERNs Coordinators Group

  Debate

15.30 – 16.15 | SESSION 5
Towards reducing the time for an early and accurate diagnosis for patients with rare diseases

Moderators: Virginie BROS-FACER, CEO, EURORDIS

- **Maurizio SCARPA**, Coordinator of MetabERN: "Promises and challenges of genomic new-born screening"
- **Felipe GÓMEZ MARTÍN**, Ministry of Health, Spain: "Screening, Genetic Tests and Reference Centres for Patients with Rare Diseases in the Spanish National Health"
- **Bruno RODRIGUES**, Ministry of Research and Higher Education, Luxembourg "The European "1+ million genomes" initiative" (online)

  Debate

16.15 – 17.15 | SESSION 6
Patient data as a fundamental element in addressing the needs of patients with rare diseases

Moderator: Hélène LE BORGNE, Policy Officer, Unit of Health Innovations & Ecosystems, DG RTD, EC

- **Tomislav SOKOL**, Member of the European Parliament, Rapporteur on the e-Health Data Space Regulation: "Expected benefits for patients with rare diseases" (online)
- **Holm GRAEßNER**, Coordinator of ERN-RND and JARDIN WP7 leader: "Patient registries and data sharing in the ERN environment"
- **Joni KOMULAINEN**, Senior Ministerial Adviser, Ministry of Social Affairs and Health, Finland: "Implementation of the regulation at the national level in the case of rare diseases: Is there a need for a particular approach?"
- **Szymon BIELECKI,** Head of Sector - Research and Innovation, Unit of eHealth, Well-Being and Ageing, DG CNECT, EC: "AI Innovation and Policy Coordination"

  Debate

17:15 – 17:30 | CLOSING SESSION
Conclusions with the participation of Ministers from the Member States of the European Union.

- **Urszula DEMKOW**, Undersecretary of State, Ministry of Health, Poland
- **Sophie LØHDE**, Minister for the Interior and Health of Denmark
- **Ágnes CSER**, Rapporteur on rare diseases, EESC