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SHAPING THE NEXT 10 YEARS OF RARE DISEASE POLICIES: EUROPE'S ACTION PLAN FOR RARE DISEASES

### #30millionreasons

### Programme at a glance:

Day MAY 12 parallel workshops

- 09.30-13.00 WORKSHOP 1 EURORDIS 101: What can EURORDIS do for you?
- **09.30-13.00** WORKSHOP 2 Living in a virtual world: Online conferences and fundraisers
- **14.00-16.00 NETWORKING SESSION** Networking and learning from each other's innovative strategies and initiatives

# **Day @** MAY 13

09.30-13.30 PLENARY SESSION Shaping the next 10 years of rare disease policies: Europe's Action Plan for Rare Diseases

# Day 3 MAY 14 PARALLEL WORKSHOPS

**09.30-13.00WORKSHOP 3**<br/>Moving forward: Advocating at national and EU level**09.30-13.00WORKSHOP 4**<br/>Moving forward: Advocating on a specific issue



# WORKSHOP 1: EURORDIS 101 WHAT CAN EURORDIS DO FOR YOU?

Moderator: Alba Ancochea, FEDER and EURORDIS, Spain

09.30-09.45 Icebreaker

### 09.45-10.15 EURORDIS: A community of people living with a rare disease

- EURORDIS' vision, mission, and values
- How is EURORDIS governed?
- Who does what on the Board and across the team?
- What does it mean to be a volunteer and what are volunteers doing?
- How can you make the most of our networks, information, and training? **Yann Le Cam**, Chief Executive Officer, EURORDIS
- 10.15-10.35 Get the best out of EURORDIS' resources: New website, eNews, Member News, webinars, Rare Disease Day resources. **Stanislav Ostapenko**, Communications Manager & **Davor Duboka**, Web Technology Manager, EURORDIS
- 10.35-10.45 Comfort break

### How can you engage with EURORDIS?

- 10.45-11.05 Learn: Open Academy: EURORDIS' training programme Raquel Castro, Open Academy Director, EURORDIS & Ilaria Galetti, FESCA, Italy
- 11.05-11.25 Share: Rare Barometer Voices: Make your voice heard! Sandra Courbier, Social Research Director, EURORDIS
- **11.25-11.30** *Comfort break*
- 11.30-11.50 Represent: European Patient Advocacy Groups (ePAGs) Ines Hernando, ERN and Healthcare Director, EURORDIS & Anne-Laure Aslanian, Ariane Weinman and Lenja Wiehe, ePAG Engagement Managers, EURORDIS
- **11.50-12.10** Therapeutic Action Group (TAG) **Virginie Hivert**, Therapeutic Development Director, EURORDIS & TAG members
- 12.10-12.35 Participate: Rare Disease Day. Simona Bellagambi, UNIAMO and EURORDIS, Italy
- 12.35-13.00 DITA (Drug Information, Transparency and Access Task Force) & HTA (Health Technology Assessment) Task Force
  François Hoüyez, Director of Treatment Information and Access & Matteo Scarabelli, Patient Engagement Manager HTA, EURORDIS, and Task Force members



# **Day 1** MAY 12

# **WORKSHOP 2:** WORKING IN A VIRTUAL WORLD: ONLINE CONFERENCES AND FUNDRAISERS

Moderator: Geske Wehr, ACHSE and EURORDIS, Germany

09.30-09.45	Icebreaker
09.45-10.15	How to organise engaging meetings and conferences online: practical tips, platforms, and budgets. Sharon Ashton, Events Director, EURORDIS
10.15-10.45	Holding a successful fundraising sports event online Elvira Martinez, FEDER, Spain
10.45-10.55	Comfort break
10.55-11.10	Q&A
11.10-11.30	Creating an online auction. Saskia de Vries, ACHSE, Germany
11.30-11.50	Organising an online conference for cystinosis Claudia Sproedt, Cystinosis Network Europe
12.00-12.15	Comfort break
12.15-12.20	Introduction to the Digital School Marta Campapadal, RareConnect Manager, EURORDIS
12.20-12.35	Engaging with the media and celebrities <b>Catherine Fowler</b> , Aortic Dissection Charitable Trust, UK
12.35-12.50	Launching projects in a virtual world <b>Dorica Dan</b> , RONARD and EURORDIS, Romania
12.50-13.00	Q&A

### **NETWORKING SESSION**

**14.00-16.00** Moderator: **Gerrit Heijkoop**, Live Online Events, Netherlands Networking and learning from each other's innovative strategies and initiatives





# PLENARY SESSION: SHAPING THE NEXT 10 YEARS OF RARE DISEASE POLICIES: EUROPE'S ACTION PLAN FOR RARE DISEASES

Moderator: Maria Montefusco, Rare Diseases Sweden and EURORDIS, Sweden

Yann Le Cam EURORDIS It is time to reset Europe's rare disease focus for the next decade: we need a policy framework in Europe adapted to today's realities, to embed excellence and bring policies in line with new technologies, values and infrastructures. Europe's efforts since 2009 have shown how much progress can be made when national plans and strategies for rare diseases are coordinated across countries and integrated at EU level. We cannot lose momentum now: the Rare 2030 recommendations set the direction we need to go."

**09.30-10.00** Welcome and opening remarks: We are the 30 million people asking for Europe's Action Plan for Rare Diseases **Terkel Andersen**, President and **Yann Le Cam**, Chief Executive Officer, EURORDIS

#### 10.00-10.40 Why do we need to advocate together for a new policy framework for rare diseases?

20 years of progress made for Duchenne patients thanks to national and European policies **Elizabeth Vroom**, World Duchenne Organization and EURORDIS, Netherlands

The young patient's perspective: Challenges for the familial hypercholesterolemia community (Global Call for Action) Athanasios Pallidis, FH Europe, Greece

Stronger together **Rebecca Tvedt Skarberg**, Norwegian National Advisory Unit on Rare Disorders and OIFE, Norway

**10.40-11.00** Small group discussions

**11.00-11.15** *Comfort break* 





- **11.15-11.30** Rare 2030 Recommendations: The roadmap for the next decade **Anna Kole**, Public Health Policy Director, EURORDIS
- **11.30-11.55** State of the art of rare diseases across Europe: National plans **Vicki Hedley**, Newcastle University, UK
- 11.55-12.15 Advocating for change at national level: Considering Rare 2030 in the Italian national plan
   Annalisa Scopinaro, UNIAMO, Italy & Stefano Bienvenuti, Fondazione Telethon, Italy
- 12.15-12.45 Panel Discussion: How can the Rare 2030 Recommendations help your national plan? Alba Ancochea, FEDER and EURORDIS, Spain Dorica Dan, RONARD and EURORDIS, Romania Nick Meade, Genetic Alliance UK Vesna Aleksovska, National Alliance for Rare Diseases of North Macedonia

### Advocating for change at European level

- 12.45-12.55 Learning from EPF's Access Campaign: "Taking Action A Roadmap to Achieving Universal Health Coverage for All Patients by 2030"
  Michele Calabro, Policy Advisor, European Patients' Forum (EPF)
- **12.55-13.10** How we can work together for change: Grassroots advocacy for a new policy framework **Jenny Steele**, Campaign Manager, EURORDIS

#### Live launch of the EU campaign platform

**13.20-13.30** Closing remarks: The end goal: Measurable social and health outcomes **Avril Daly**, Retina International and EURORDIS, Ireland





# **WORKSHOP 3:** MOVING FORWARD: THE RARE 2030 CAMPAIGN

This hands-on workshop is designed to set you up with the tools you need to advocate during the upcoming Rare 2030 campaign at the local and national level. Moderator: **Anna Arellanesová**, Rare Diseases Czech Republic

- 09.30-10.15 Rare 2030 Campaign for a new policy framework: Why we need your support and how this will help you! Anna Kole, Public Health Policy Director & Jenny Steele, Campaign Manager, EURORDIS
- 10.15-10.45 How to advocate with your MP/MEPs Tell a story Kostas Aligiannis, Public Affairs Manager, EURORDIS & Dominique Sturz, Pro Rare Austria
- **10.45-10.55** *Comfort break*
- **10.55-12.25** Breakouts and Discussion: Developing your campaign strategy
- **12.25-12.30** *Comfort break*
- 12.30-13.00 International policies for rare diseases: UN Resolution on addressing the challenges of persons living with a Rare Diseases
  Clara Hervas, Public Affairs Manager, EURORDIS





# **WORKSHOP 4:** MOVING FORWARD: ADVOCATING FOR A SPECIFIC ISSUE

This workshop is designed to help you make progress in an area where you want to see change. Moderator: **Alain Cornet**, Lupus Europe and EURORDIS, Belgium

- **09.30-10.00** Welcome & Introduction to the breakout sessions Using EURORDIS' position papers and Rare 2030 recommendations to advocate for diagnosis, treatment and care **Valentina Bottarelli**, Public Affairs Director & Head of European and International Advocacy, EURORDIS
- 10.00-12.30 Breakout 1 parallel session Access to potentially curative therapies Moderator: Karolina Hanslik, Senior Project Officer, EURORDIS
- **10.00-10.30** Rare 2030 recommendation 8 Available, Accessible and Affordable, Treatments **Simone Boselli**, Public Affairs Director, EURORDIS

**10.30-10.45** *Comfort break* 

- 10.45-11.15 Case study: Access to curative treatment across borders: a Swedish/Italian success story Maria Montefusco, Rare Diseases Sweden and EURORDIS, Sweden & Simone Boselli, EURORDIS
- 11.15-11.30 Questions & Answers
- **11.30-11.40** *Comfort break*
- 11.40-12.30 Discussion
- 10.00-12.30 Breakout 2 parallel session Newborn Screening & innovative diagnosis Moderator: Simona Bellagambi, UNIAMO and EURORDIS, Italy
- **10.00-10.20** Rare 2030 Recommendation 2: Earlier, Faster, more accurate diagnosis **Antoni Montserrat**, ALAN, Luxembourg





- **10.20-10.40** NBS in Healthcare (Next Generation Sequencing for NBS) Nick Meade, Genetic Alliance UK
- **10.40-10.55** Presentation of Key Principles for Newborn Screening and roadmap to implementation **Gulcin Gumus**, Research & Policy Project Manager, EURORDIS
- 10.55-11.10 Case study: Newborn Screening for SMA Marie Christine Ouillade, European Alliance of NBS for SMA
- **11:10-11:30** *Comfort break*
- **11.30-12.30** Co-creation of the roadmap
- 10.00-12.30 Breakout 3 parallel session Taking action on ERNs at national and European level Moderator: Matt Bolz-Johnson, ERN and Healthcare Advisor, EURORDIS
- **10.00-10.20** Tour de table and introduction moderator
- 10.20-10.50 Taking action at national level Inés Hernando, ERN and Healthcare Director, EURORDIS
- **10.50-11.10** *Comfort break*
- 11.10-11.30 Best practices. Taking action at national level: Connecting patient organizations to Centres of Expertise by a legal role in the designation process.
   Cor Oosterwijlk, Director National Patient Alliance for Rare and Genetic Diseases (VSOP), The Netherlands
- 11.30-11.50 Case study Taking action at European Level Improving access to care and treatment for Huntington's Disease patients and families Astri Arnesen, European Huntington Association, Norway
- **11.50-12.30** Questions and Answers Listening to you
  - Identify together other best practices have you organised or participated in any action on ERNs at national or at European level?
  - Is the toolkit useful or are we missing something?
- 12.30-13.00 Reporting back from Breakouts & Closing remarks Alain Cornet, Lupus Europe and EURORDIS, Belgium





#### EMM 2021 Programme Committee:

Konstantinos Aligiannis, EURORDIS Alba Ancochea, FEDER Sharon Ashton, EURORDIS Valentina Bottarelli, EURORDIS Lara Chappell, EURORDIS Dorica Dan, Romanian National Alliance For Rare Diseases

Anja Helm, EURORDIS Clara Hervás, EURORDIS Anna Kole, EURORDIS Yann Le Cam, EURORDIS Maria Montefusco, Rare Diseases Sweden Jenny Steele, EURORDIS Geske Wehr, ACHSE



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