



# EMM 2017 Budapest

19 & 20 May 2017

Danubius Hotel HELIA, Budapest, Hungary

## Agenda

### Thursday 18 May – Satellite Meetings

14.00-18.00 ePAG representatives meeting & Leadership workshop *Helia Room*

09.30-18.00 Council of National Alliances (CNA) meeting *Panorama Room*

### Friday 19 May

09.00-13.00 EURORDIS General Assembly (EURORDIS Members only) *Helia Room*

Business agenda

Election of five Board members

Discussion: *Taking Rare Disease Policies to the next level*



## EMM 2017 Plenary: EURORDIS 20 years' anniversary

*Building on success of 20 years of advocacy for rare disease*

*Helia Room*

EURORDIS' members can look back on 20 years of advocacy, which has brought many positive changes. In this plenary session, we will discuss in three panels what have been the key success factors, our strengths and weaknesses, how we can build on these successes while addressing the challenges ahead.

14.30-14.40 Welcome speech, *Terkel Andersen, President, EURORDIS*

14.40-14.50 Opening remarks, *Hungarian Health Ministry (tbc)*

14.50-15.00 Introductions and Interactive welcome, *Geske Wehr, European Network for Ichthyosis & EURORDIS*

**15.00-15.50**

### **I Building an empowered and engaged community of patient advocates**

*Moderator: Lise Murphy, Svenska Marfanföreningen, Sweden*

#### **Achievements over the past 20 years (10 min), *Dorica Dan, Romanian Alliance for Rare Diseases & EURORDIS, Romania***

*Access to information on rare diseases was scarce and now it is wide. The level of information and knowledge shared has increased in a spectacular way, with websites, on line tools and help lines. Policy makers awareness is increasing, public awareness is emerging. Thousands of people in patient organisations have been empowered through information, experience sharing, networking. Hundreds of patient representatives have been trained over the past two decades, in the Summer school, EMM & ECRD workshops and webinars to become European rare disease patient advocates. Today, many of them represent patients at the different EMA committees, HTA bodies, the Commission Expert Groups, task forces or working groups and now in the ePAGs.*

#### **Panel discussion: Way forward: goals, objectives and means (40 min)**

*Gabor Pogany, Rare Diseases Hungary*

*Lesley Greene, COMP / EURORDIS, UK*

*François Houyez, EURORDIS, France*

**15.50.16.40**



## **II Building an open and respected movement of patient organisations and advocates**

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*Moderator: Lene Jensen, Rare Diseases Denmark*

### **Achievements over the past 20 years (10 min) *Avril Daly, Retina International & EURORDIS, Ireland***

*We have seen steady growth in the number patient organisations across diseases and countries. National Alliances now federate rare disease patient organisations in every European country and beyond. European and International Federations for rare disease have sprung up to enhance collaboration across borders. With the creation of European Reference Networks, patient organisations have come together in therapeutic groupings and represent their best interests in ePAGS. Rare Diseases International speaks for patients globally. Patient organisations have engaged in partnerships with a multitude of stakeholders to make their voice heard.*

### **Panel discussion: *Way forward: goals, objectives and means (40 min)***

*Claudia Crocione, HHT Europe, Italy*

*Julian Isla, Dravet Syndrome Foundation, Spain*

*Olivier Timmis, AKU Society, UK*

16.40-17.10 Coffee break

**17.10-18.00**

## **III Building a strong European & international advocacy voice & agenda to achieve Goals**

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*Moderator: Russel Wheeler, LHON Society, UK*

### **Achievements over the past 20 years and future goals in advocacy (10 min) *Yann Le Cam, Chief Executive Officer, EURORDIS***

The rare disease community has achieved much: The Orphan Medicine Regulation, other regulations on paediatric medicines and advanced therapies, the Commission Communication and Council Recommendation, the National Plans for Rare Diseases, the Directive on Patient's Right to Cross-Border Healthcare. What are the next advocacy goals? A new step in national actions for rare diseases and in European structured collaboration between countries? A 2<sup>nd</sup> Council Recommendation? A Joint Programme for Research on Rare Diseases? A Joint Action of European Reference Networks for Rare Diseases? A Resolution for Rare Diseases at the UN General Assembly? Impact oriented goals meaningful for patients, families and the public?

### **Panel discussion: *Way forward: goals, objectives and means (40 min)***

*Lieven Bauwens, IF Global, Belgium*

*Juan Fuertes, Pulmonary Hypertension Association Europe*

*Kathy Oliver, International Brain Tumour Alliance, UK*



19.00

EURORDIS 20 Years celebration dinner, Danubius Hotel

Saturday 20 May (sessions are open to all)

## Workshops (09.30-16.00)

Workshop 1: European Reference Networks – Hopes & Dreams:  
Let us make it happen!

*Chairs: Lenja Wiehe, Matt Bolz-Johnson, EURORDIS*

*Helia Room*

09.30 **Welcome & Introductions, *Matt Bolz-Johnson***

09.45-11.00 **Session 1: ‘Turning ERNs into a Reality: A New Era for Rare Diseases’**

**ERN Presentations:**

- Diagnosis: Rare Malformation ERN, *Sofia Douzgou*
- Treatment: Rare Metabolic ERN, *Maurizio Scarpa*
- Research: Rare Bone ERN, *Luca Sangiorgi*

**Ask the Experts Roundtable Discussion, *Chair: Matt Bolz-Johnson, EURORDIS***

- *Maurizio Scarpa, Rare Metabolic European Reference Network*
- *Luca Sangiorgi, Rare Bone European Reference Network*
- *Sofia Douzgou, Rare Malformations European Reference Network*

11.00-11.30 Coffee break

**Session 2 ‘Will ERNs live up to our hopes and dreams?’**

11.30-12.00.. **Three ePAG Case Studies**

- Rare Renal ePAG, *Claudia Sproedt, FEDERG*
- Rare Lung ePAG, *Courtney Coleman, European Lung Foundation*
- Haematology ePAG, *Amanda Bok, European Haemophilia Consortium*

12.00-12.30 **Panel discussion, *Chair: Lenja Wiehe, EURORDIS***

- *Claudia Sproedt, FEDERG*
- *Courtney Coleman, European Lung Foundation*



- *Amanda Bok, European Haemophilia Consortium*

12.30-13.00 **The operationalisation of an ERN, *Victoria Hedley, University of Newcastle, RD-Action, UK***

13.00-14.00 Lunch

14.00-15.30 **Session 3: ERN Specific Focus Groups**

**Focus Group 1: Rare Malformation ERN**

Lead: Sofia Douzgou, Network Coordinator ITHACA / Co-lead ePAG: Dorica Dan, Romanian Alliance for Rare Diseases

Rapporteur : Virginie Bros-Facer, EURORDIS

**Focus Group 2: Rare Bone ERN**

Lead: Luca Sangiorgi, Network Coordinator / Co-lead ePAG: Ines Alves, ANDO, Portugal

Rapporteur: Lenja Wiehe, EURORDIS

**Focus Group 3: Rare Metabolic ERN**

Lead: Maurizio Scarpa, Network Coordinator / Co-lead ePAG: Anne-Sophie Lapointe, VML & EURORDIS France

Rapporteur: Matt Johnson

15.30-16.00 Panel Discussion, **Chair: Victoria Hedley & Matt Bolz-Johnson**

- *Maurizio Scarpa*
- *Luca Sangiorgi*
- *Sofia Douzgou*
- *Victoria Hedley*
- *Claudia Sproedt*
- *Courtney Coleman*
- *Amanda Bok*

## *Workshop 2: Hope & Happiness are part of the treatment*

### *Part 1: Natural, Alternative, Traditional, Complementary therapies*

*Chair: François Houyez, EURORDIS*

*Uranus Room*

For lack of treatment, RD patients might resort to NATC Treatments (Natural, Alternative, Traditional, and Complementary) hoping for a positive impact on their disease. However, in the absence of clinical research and of evidence-based data, these patients risk disappointment, adverse effects, decline in health, interactions with medicines and financial burden. Yet, patients who purchase NATC products have the right to know if they are safe, effective, and high quality, as for pharmaceuticals. The impact of these products on patient's health and quality of health needs to be determined, and the



information needs to be made available in an understandable manner. This session will share experience on the use of NATC in rare diseases and explore which initiatives are of interest, which information do we need from patients, and finally which project could derive from our reflection.

Part 1 (morning) will focus on products: vitamins, herbal medicinal products, food supplements, trace elements and other substances for which health claims exist, even if not validated.

- 09.30-09.40 Introduction : Reflection on NATC, *François Houÿez, EURORDIS*
- 09.40-10.00 Case study: a mushroom to treat muscles? *Tuy Nga Brignol, AFM-Telethon, France*
- 10:00-10.20 A Research Roadmap for Complementary and Alternative Medicine – What We Need to Know by 2020, *Felicity Bishop, University Southampton, UK*
- 10.20-10.30 Presentation of the table discussions
- 10.30-11.00 Table discussions  
Participants will work at their table, with other participants, review and discuss documents in relation with NATC products. One of the participants will volunteer to summarise the discussion and present for 5 minutes after the coffee break.
- 11.00-11.30 Coffee break
- 11:30-12:00 Reports from tables (5 minutes each)
- 12:00-12:20 The role of the Herbal Medicinal Product Committee at the EMA, *Rob Camp, DITA Task Force*
- 12.20-12.40 Patient questionnaires: standards and requirements, *Sieta de Vries, University Groningen, The Netherlands*
- 12.40-13.00 Other possible actions and next steps
- 13.00-14.00 Lunch
- 14.00-16.00:

## Workshop 2: Part 2 -The quest for a holistic approach to being well

*Yann le Cam, Chief Executive Officer, EURORDIS*

*Helia Room*

EURORDIS strategy and actions so far promote evidence based medicines; from evidence based medicinal products assessment to best practice of care; from translational research to evidence based clinical expertise assessment. In recent years, EURORDIS has promoted innovative approaches to address the high heterogeneity of the clinical expression of our diseases; from adapted design of clinical trials to medicine adaptive pathways; from observational studies to real world evidence. EURORDIS and its members are increasingly working on the integration of social and medical care, a transformative approach far beyond the specialised social services eg resource centres, respite services, therapeutic recreational programme.



A holistic approach to patients and family needs to live up to the optimal of their potential, simply trying to be well, if not reaching well-being as ambitious by WHO and the UN Sustainable Development Goals, calls for the full variety of options beyond hospital care, genetic diagnostic, surgery, medicines, NATC, to include all para-medical therapeutic and rehabilitation services, all ancestral traditional to cutting edge innovative therapeutic or healing approaches. It is time to unveil the wide spectrum of practices actually used regularly or in some exceptional circumstances by patients and their families.

The discussion is expected to be highly inter-active based on the RareBarometer survey as well as your experience, and structured around 4 main questions:

- What are the approaches used or known to participants? Which ones are the most often used? Which ones may have the highest interest? Which ones represent the higher risks?
- Evidence based medicine will never encompass the reality of our life and care practices. Do EURORDIS members think it is important to collect more patient perspective and patient qualitative anecdotal reports? Why? How? Is it part of patient generated knowledge?
- How to navigate the knowns and unknowns? How to help each other across our community? Should we develop a code of conduct with tips to guide patients and families? Is it part of patient empowerment?
- Should we integrate this topic in our patient advocacy? If so, with which objectives?

## Workshop 3: Survival Kit for small Patient Organisation

*Chair: Claudia Crocione, HHT Europe*

*Mercure Room*

Many patient organisations, in particular newly established and small organisations, struggle with the same issues in terms of raising funds, optimizing human resources, raising awareness, triggering research and collaborating with other patient organisations for their disease across borders. This capacity-building workshop will show case examples of patient organisations' solutions, who will share their experience in a peer-to-peer session.

09.30-10.00 Interactive Introduction, *Claudia Crocione, HHT Europe,*

Is your mission clear?

Are you setting and reaching our goals?

Do your goals correspond to the needs of your patients?

Are you measuring your return on investment?

Does your Board of directors share your same vision?

10.00-10.30 Take a look on the inside – is it time to start up again?

Case study HHT Onlus, *Claudia Crocione, HHT Europe*

10.30-11.00 Training volunteers, *Claudia Crocione, HHT Europe*

Make the best use of training opportunities (Summer School, local or national free classes for NGOs, capacity-building workshops at your meetings)

11.00-11.30 Coffee break



- 11.30-12.15 Turning members into active volunteers and hiring staff: People raising and strategies associated, *Oliver Timmis, AKU Society, UK*
- 12.15-13.00 Communication: creating a positive image and impact, *Lara Chappell, EURORDIS*
- 13.00-14.00 Lunch
- 14.00-15.00 Fundraising strategies, *Claas Röhl, NF Kinder, Austria*  
Fundraising basics for small organisations, project based fundraising, events, sponsors, in-kind and the pitfalls of crowdfunding
- 15.00-16.00 Feedback from the audience, exchange of best practices and examples

## Workshop 4: Social Revolution

*Chairs: Raquel Castro, EURORDIS; Ildiko Vajda, VSOP Netherlands*      *Panorama Room*

«One voice can start a revolution», Elaina Marie. We all agree that the confrontation with a rare disease is a life changing event and that rare diseases' impact on daily life is significant, leading patients and families to situations of serious psychological, social, economic and cultural vulnerability. But how are we getting that message across to those making decisions, to all those who can be of support, to society at large? How can we make social revolution happen for people with a rare disease? Through this interactive workshop we will, together, build on each other's experiences and ideas.

### Session 1: Balancing care and daily life

*Chair: Ildiko Vajda*

*Presentation of the results of the INNOVCare/RareBarometer Survey on the impact of rare diseases in daily life & small group discussions on the results: What recommendations can we do based on the results and our experience?*

- 09.30-10.15 Survey results: Impact of rare disease on daily life and related needs, *Sandra Courbier, EURORDIS*
- 10.15-11.30 Survey results: The challenge of coordination of care, *Raquel Castro, EURORDIS*
- 11.00-11.30 Coffee break
- 11.30-12.15 Survey results: The challenge of working while living with a rare disease, *Sandra Courbier, EURORDIS*
- 12.15-13.30 Survey results: Impact of rare diseases on well-being and mental health, *Erwan Berjonneau, EURORDIS*



13.00-14.00 Lunch

**Session 2: Presenting our needs with impact**

*Chair: Raquel Castro*

*Capacity building & learning from each other*

14.00-14.30 Inspirational campaigns using humour to raise awareness, *Martina Fuga, CoorDown Board, delegate for communication, Italy*

14.30-15.00 Presenting surveys with impact, *tbc*

15.00-15.15 Disseminating survey results on social media, *Eva Bearryman, EURORDIS*

15.15-15.45 How can we disseminate the survey to different publics at European and national level? *Discussion in small groups: share your experiences & ideas*

15.45-16.00 Debrief from small group discussions

## 16.00-17.00 Plenary session

*Chair: John Dart, Debra International, UK*

*Helia Room*

16.00-16.30 **Feedback from the four workshops**

16.30-17.00 **Soap Box session: Short presentation & discussion of topics proposed by participants**

- SWAN Europe: Empower families affected by syndromes without a name; Nick Meade
- Call to Action to improve organ donation and transplant across the EU, Juan Fuentes, PHA Europe
- RareFlying project, Xun !ji, Chinese Organisation for Rare Diseases
- Patient Monitoring Project, Selcuk Kayan, MPS Turkey
- Patient-centered research promotion, Rainald von Gizycki, Pro Retina, Germany



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