



EURORDIS  
Rare Diseases Europe

# MEMBERSHIP MEETING 2015

Madrid ✈ 28-30 May

[eurordis.org](http://eurordis.org) #EMM2015Madrid



## Programme Committee

### Alba Ancochea Díaz

FEDER Spanish National  
Alliance for Rare Diseases, Spain

### Amanda Bok

European Haemophilia Consortium, Belgium

### Valentina Bottarelli

EURORDIS

### Yann Le Cam

EURORDIS

### Dorica Dan

RONARD Romanian National  
Alliance for Rare Diseases, Romania

### Anja Helm

EURORDIS

### Birthe Holm

Rare Diseases Denmark, Denmark

### Lene Jensen

Rare Diseases Denmark, Denmark

### Matt Johnson

EURORDIS

### Anne Sophie Lapointe

Vaincre les Maladies Lysosomales, France

### Christoph Nachtigäeller

ACHSE, German National Alliance  
for Chronic Rare Diseases, Germany

## EURORDIS Membership Meeting 2015 Madrid Hotel Rafael Atocha

### Satellite meetings

#### THURSDAY 28 MAY

09.30–13.30 **Council of National Alliances (CNA) Meeting** CIBELES ROOM  
Closed meeting



09.30–12.30 **Rare Connect & Social Media** CASTELLANA ROOM  
Open meeting



08.00–12.30 **EuMGA Meeting** EL PRADO ROOM  
Closed meeting

14.30–17.30 **Rare Diseases International Launch Meeting** CIBELES ROOM  
Closed meeting



RARE  
DISEASES  
INTERNATIONAL

14.00–17.30 **Cognitive disorders – results from the Gencodys project and future outlook to ERNs**  
EL PRADO ROOM  
Closed meeting

16.00–17.00 **Asociación Asanol Meeting** RECOLETOS ROOM  
Closed meeting

#### SUNDAY 31 MAY

08.30–16.00 **FEDER General Assembly** ATOCHA ROOM



14.00–18.00 **Annual Assembly European Federation EURO-HSP and the Spanish Association AEPEF**  
RECOLETOS ROOM  
Closed meeting



# Friday 29 May, EURORDIS Membership Meeting

## EURORDIS GENERAL ASSEMBLY (MEMBERS ONLY, ATOCHA ROOM)

🔊 ENGLISH – SPANISH

|             |  |
|-------------|--|
| 08.00–09.00 | Registration   |
| 09.00       | Opening remarks, <i>Terkel Andersen, President</i>   |
| 09.05–09.15 | Activity Report 2014, <i>John Dart, General Secretary</i>  |
| 09.15–09.25 | Financial Report 2014 & Auditor's Report, <i>Dimitrios Synodinos, Treasurer</i>  |
| 09.25–09.30 | Auditor's special report on regulated conventions, <i>Dimitrios Synodinos</i>  |
| 09.30–10.00 | Presentation of candidates for the Board of Directors elections  |
| 10.00–10.15 | Action Plan 2015 & budget 2015, <i>Yann Le Cam, Chief Executive Officer</i>  |
| 10.15–10.30 | Membership criteria, <i>Yann Le Cam</i>  |
| 10.30–10.45 | Questions & answers  |
| 10.45–11.15 | Presentation of strategy 2015 – 2020, <i>Yann Le Cam</i>   |
| 11.15–11.30 | <b>Coffee break</b> PLAZA DE ORIENTE ROOM  |
| 11.30–12.30 | 4 break-out sessions: discussion of strategy 2015–2020<br><i>Terkel Andersen</i> (Atocha room, only room with Spanish translation)<br><i>Yann Le Cam</i> (Cibeles room, ground floor)<br><i>John Dart</i> (Castellano room, first floor)<br><i>Dorica Dan</i> (El Prado room, first floor) |
| 12.30–12.55 | Plenary session (Atocha room): synthesis of breakout session discussions by the rapporteurs  |
| 12.55–13.00 | Results of resolutions vote & Board of Directors election, <i>John Dart</i>  |
| 13.00–14.00 | <b>Lunch</b> PLAZA DE ORIENTE ROOM   |

## PLENARY SESSION (ATOCHA ROOM) 🔊 ENGLISH – SPANISH

|             |  |
|-------------|--|
| 14.00–15.00 | <b>European Reference Networks for rare diseases (ERN)</b><br><b>Moderator: Avril Daly, CEO Fighting Blindness, Ireland &amp; EURORDIS</b> <ul style="list-style-type: none"><li>&gt; A short overview of the long and winding road to the current position, and of future challenges, <i>Yann Le Cam</i></li><li>&gt; The state of the art on ERNs at EU level, <i>Matt Johnson, Healthcare and Research Director, EURORDIS</i><ul style="list-style-type: none"><li>▪ Legal base to create ERNs</li><li>▪ Progress so far (Board of EU MS, Manual Tool Kit, assessment bodies)</li><li>▪ How the call for ERN will be set up by the European Commission in late 2015 and 2016</li></ul></li><li>&gt; What can patients expect from European Reference Networks? Why is it important for patients to be involved? <i>Birthe Holm, Rare Disorders Denmark &amp; EURORDIS</i></li><li>&gt; "Ideals vs reality" panel discussion with <i>Birthe Holm, Rare Diseases Denmark &amp; EURORDIS</i>; <i>Dorica Dan, Rare Diseases Romania &amp; EURORDIS</i>; and <i>Matt Johnson</i></li></ul> |
| 15.00–16.30 | <b>Breakout sessions: preparing for patient engagement in ERN applications</b><br><b>Moderators: Yann Le Cam (Atocha room); Amanda Bok (Cibeles room); Matt Johnson (Castellana room); John Dart (El Prado room)</b> <ul style="list-style-type: none"><li>&gt; Opportunity for networking</li><li>&gt; Discuss the process of patient representation</li><li>&gt; Discuss how best to collaborate with specialists</li></ul>  |
| 16.30–17.00 | <b>Coffee break</b> PLAZA DE ORIENTE ROOM  |
| 17.00–17.45 | <b>Plenary session – outcomes of breakout sessions</b> ATOCHA ROOM<br><b>Moderator: Avril Daly. Each moderator will report on their break-out session</b>  |
| 17.45–18.00 | <b>Closing remarks</b><br><b>Maravillas Izquierdo, Subdirectora General de Cartera Básica del SNS y Fondo de Cohesión, Spain</b>   |
| 19.30       | <b>Welcome dinner at the Hotel Rafael Atocha</b><br>CIBELES, PLAZA DE ORIENTE AND NEPTUNO ROOMS  |

## Saturday 30 May

### 09.30–10.30 **PLENARY SESSION** *ATOCHA ROOM*

Inspiration from national conferences: an overview of successes in national rare disease strategies

- > What are the overall outcomes of the national conferences on national plans?
- > What are the existing or promising positive elements included in national plans or strategies that could be used to inspire other countries?

Panel discussion with EUROPLAN– EURORDIS advisors: *Lene Jensen, Avril Daly, Dorica Dan, Vlasta Zmazek*

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### 10.30–11.00 **Coffee break** *PLAZA DE ORIENTE ROOM*

### 11.00–13.00 **WORKSHOPS – PART 1:**

State of play, taking stock of national experiences, national conferences and rare disease strategies

#### Workshop **RESEARCH** *CASTELLANA ROOM*

**Moderators: John Dart; Valentina Bottarelli, Public Affairs Senior Advisor, EURORDIS**

- > Inspiring actions and good measures from national plans/strategies and EUROPLAN national conferences *Valentina Bottarelli*
- > Positive examples of initiatives at a national level
  - France: *Roseline Favresse, French Foundation for Rare Diseases, France*
  - Spain: *Dr. José María Millán, CIBERER, Spain*
  - UK: Rare Diseases Translational Research Collaboration, *Dr. Marijcke Veltman, Operational Director, NIHR Rare Diseases Translational Research Collaboration*
- > From patient empowerment to an open scientific community, the experience of "Determinazione Rara" in Italy, *Sara Casati, UNIAMO, Italy*

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#### Workshop **ACCESS TO ORPHAN MEDICINAL PRODUCTS, TREATMENT AND CARE**

*CIBELES ROOM*

**Moderators: Flaminia Macchia, Public Affairs Director; Yann le Cam**

- > Inspiring actions and good measures from national plans/strategies and EUROPLAN national conferences, *Flaminia Macchia*
- > First results of Access campaign survey, *Rob Camp, DITA Task Force*
- > How patients are now consulted as experts at the time of risk/benefit assessment by CHMP/EMA? How patients are consulted on HTA at European level through early scientific dialogue, *François Houÿez, Treatment Information and Access Director, Health Policy Advisor, EURORDIS*

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#### Workshop **CENTRES OF EXPERTISE** *ATOCHA ROOM*

**Moderators: Lene Jensen, Chief Executive Officer, Rare Diseases Denmark; Matt Johnson**

- > Inspiring actions and good measures from national plans/strategies and EUROPLAN national conferences, *Matt Johnson*
- > European perspective: review of quality of Centres of Expertise undertaken in WP4 of the Joint Action, *Francesc Palau, CIBERER, Spain*
- > Diversity of national landscapes for Centres of Expertise, 3 case studies:
  - Denmark: *Lene Jensen, Rare Diseases Denmark, Denmark*
  - France: *Paul Gimenes, Alliance Maladies Rares, France*
  - Portugal: *Marta Jacinto, Aliança Portuguesa de Associações das Doenças Raras, Portugal*

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#### Workshop **SOCIAL POLICY** *EL PRADO ROOM*

**Moderators: Dorica Dan; Raquel Castro, Social Policy Manager, EURORDIS**

- > How member states and the Commission Expert Group are addressing social challenges in rare diseases, *Raquel Castro*
- > Social policy proposals at the Spanish national EUROPLAN conference, *Alba Ancochea Díaz, FEDER*
- > Care pathways: the experience of Sweden, *Veronica Wingstedt de Flon, NFSD – Swedish National Agency for Rare Diseases, Sweden*

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### 13.00–14.30 **Lunch** *PLAZA DE ORIENTE ROOM*

## 14.30–16.00 **WORKSHOPS – PART 2**

What's next? How to move forward in order to sustain the process of developing national and European rare disease policies?

### Workshop **RESEARCH** CASTELLANA ROOM

**Moderator: John Dart; Valentina Bottarelli**

- > E-Rare-3 – Project presentation, role of EURORDIS, opportunities for members, *Mathieu Boudes, Operations and Projects Manager, EURORDIS*
- > Good examples and presentation of scenarios of coordination between POs and national funding agencies, *Stephanie Robertson, Canadian Institutes of Health Research (CIHR), Canada*
- > Open discussions and feedback from members

### Workshop **ACCESS TO ORPHAN MEDICINAL PRODUCTS, TREATMENT & CARE**

CIBELES ROOM

**Moderators: Flaminia Macchia; Yann le Cam**

- > Actions to address the substitution, for economic considerations, of an orphan medicine by an unauthorised product or off-label use? Action to address shortage of medicines? *François Houyez*
- > EURORDIS advocacy main messages to improve access: key concepts & new European processes integrating national and European levels, *Yann le Cam, EURORDIS*
- > Discussion on our common priorities for national and European advocacy

### Workshop **CENTRES OF EXPERTISE** ATOCHA ROOM

**Moderators: Lene Jensen; Matt Johnson**

- > Patient organisations as part of the accreditation process of Centres of Expertise, *Cor Oosterwijk, VSOP, Netherlands*
- > Patient organisations involvement in the development of care pathways, guidelines, transition from childhood to adult care, *Amanda Bok, European Haemophilia Consortium*
- > A national perspective: experience of a national network from the UK on Centres of Expertise for neurofibromatosis, *Dr Susan Huson, National Lead and Geneticist, UK*

### Workshop **SOCIAL POLICY** EL PRADO ROOM

**Moderators: Dorica Dan; Raquel Castro**

- > The role of patient organisations: case study of the Croatian Alliance for Rare Diseases, *Vlasta Zmazek*
- > The role of case managing teams: case study of PRIOR, *Prof. Dominique Bonneau, PRIOR Maladies Rares, France*

## 16.00–16.30 **Coffee Break** PLAZA DE ORIENTE ROOM

## 16.30–17.30 **PLENARY SESSION: NEXT STEPS** ATOCHA ROOM

**Moderator: Terkel Andersen**

Panel discussion with *John Dart, Yann Le Cam, Dorica Dan, Lene Jensen*

## 17.30–18.30 **Farewell drinks** PLAZA DE ORIENTE ROOM

## Sunday 31 – Social programme

- 09.30–11.30 Combined walking tour and panoramic visit by bus of Madrid (wheelchair accessible), in English  
For pre-registered attendees  
Pick up and drop off at the Hotel Rafael Atocha

## Notes

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Handwriting practice lines consisting of 20 horizontal dotted lines on a white background.







**Conference Venue:**

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