EURORDIS GENERAL ASSEMBLY 2016

26 May, 2016
Edinburgh, United Kingdom
Activity Report 2015:
EURORDIS KEY ACHIEVEMENTS
Terkel Andersen, President

26 May, 2016 Edinburgh, UK
FOCUS ON FEW KEY MESSAGES
WHEN LOOKING BACK AT 2015

• EURORDIS is growing in membership, networking and outreach
• Priority on Improving Patient Access to Medicines to address growing concerns with innovative ideas and boldness
• Pressure is increasing on EU Regulation on Orphan Medicinal Products and there is a risk of growing gap with the USA
• Priority on European Reference Networks for Rare Diseases with high bet and high investment for potential high return
• International initiatives led by Europe to carry a long term vision, reach a wider critical mass, build a stronger ecosystem
Growth of membership & network & outreach
2015 EURORDIS KEY FIGURES

- 695 member patient organisations (as of Dec 2015)
- 63 countries (26 EU countries)
- 32 national alliances (in Europe)
- Over 1000 other patient groups represented indirectly
- 40 staff members
- 366 volunteers
MEMBERSHIP: 32 NATIONAL ALLIANCES
MEMBERSHIP: 52 EUROPEAN FEDERATIONS
MEMBERSHIP

- EURORDIS Membership:
  - 695 members in 63 countries including **65 new members**
  - New Alliances launched in **Serbia and Macedonia**

- The **18th Workshop of the Council of National Alliances**
  (32 National Alliances for Rare Diseases)

- The **8th Workshop of the Council of European Federations**
  (52 rare diseases-specific networks)

- EURORDIS Membership Meeting 2015 Madrid with 250 participants from 43 countries
  - 27 patient representatives received fellowships

=> **We are experimenting new ways to work together through more frequent consultations, surveys, working groups, webinars**
Priority on Access to Medicines
Patients’ access to orphan medicines across Europe has deteriorated due to the economic crisis and resulting impact on healthcare budgets.

Throughout 2015 EURORDIS sustained its advocacy work in favour of European-level initiatives aimed at improving access for patients:
- European parallel scientific advice by EMA and HTA agencies
- Exchange on HTA reports and future European common HTA assessment reports.
- Medicines Adaptive Pathways to Patients (MAPPs)
- Mechanism of Coordinated Access to Orphan Medicinal Products (MOCA)

We continue to promote European collaboration between Member States on pricing and structured market access that is based on value, volume and post-marketing authorisation data generation, also exploring managed entry agreement, fair pricing, differential pricing, pricing based on outcomes and discount for uncertainties:
- EURORDIS –EPF Call to Payers in 2015
- Active dialogue at European / national levels
- Papers on Differential Prices, on Shortages of Medicines
The Challenge: Patient Access

- Despite great progress in few years’ time, **scientific advances are not being translated rapidly enough** into approved therapies effectively reaching the patients in need:

  If a medicine is approved but does not reach those who need it, it **is a failure of its primary purpose**.

  We need to **close the gap between innovation and access**.
Is a “New Deal” possible?

All stakeholders – regulators, health authorities, industry – need to **rethink radically** how new treatments reach patients faster and more affordably.

A more structured patient access/market access, guaranteeing better value for money of medicines, requires to tackle **two main “drivers”**:

**Science**
- Pre-requisite for OMPs: recognise that evidence generation has to be all along the lifecycle of medicines
- Assessment of safety, efficacy / effectiveness for small populations requires post-MA research activities agreed and aligned at European level between companies, EMA, HTA and payers
- Current system is siloed and no longer suited to today’s scientific reality

**Economy**
- Current value demonstration models generally avoid the “elephant in the room” and only result in a disconnect between supposed value and final price
- Industry and payers together must agree upfront on how to approach value (e.g. in situations of small populations and limited knowledge), how to ensure more robust value assessment over time, and how to better link value and price

**MAPPs and seamless EMA-HTA-payers dialogue at the European level**
Priority on European Reference Networks
EUROPEAN REFERENCE NETWORKS

Developing European Reference Networks of Centres of Expertise and Healthcare Providers was our strategy and work since 2006 and an though advocacy battle and it’s coming! A huge success & in progress!

• Throughout 2015, EURORDIS continued paving the way for the next steps in ERN implementation to take place in 2016 by:
  – Supporting the implementation of the EU policy on European Reference Networks => CEG RD Recommendations on RD Groupings and on Patient Engagement + Active Dialogue with Commission and Board of MSs
  – Preparing our members and stakeholders to be involved in ERN development
  – Undertaking a re-structuration of our membership around the ERN grouping of rare diseases and forming the European Patient Advocacy Groups (ePAGs).

• EURORDIS led the PACE-ERN Consortium together HOPE and Accreditation Canada to develop the overall process of the call, application submission and evaluation based on the Assessment Manual & Tools => We dared to take high risks to apply and be selected as a provider with EC to help shape a policy based on patient perspective and clinical excellence
<table>
<thead>
<tr>
<th>Thematic Grouping</th>
<th>Coordinating HCP</th>
<th>Name of Coordinator</th>
</tr>
</thead>
<tbody>
<tr>
<td>Rare Bone Diseases (BOND)</td>
<td>Istituto Ortopedico Rizzoli, ITALY</td>
<td>Luca Sangiorgi</td>
</tr>
<tr>
<td>Rare Cancers and Tumours (Paediatric)</td>
<td>St. Anna Kinderkrebsforschung e.V. – AUSTRIA</td>
<td>Ruth Ladenstein</td>
</tr>
<tr>
<td>Rare Cancers and Tumours (Adult)</td>
<td>General Cancer Centre Léon Bérard – FRANCE</td>
<td>Jean-Yves Blay</td>
</tr>
<tr>
<td>Rare Cardiac Diseases (GUARD-HEART)</td>
<td>Academic Medical Centre - THE NETHERLANDS</td>
<td>Arthur Wilde</td>
</tr>
<tr>
<td>Rare Connective Tissue and Musculoskeletal Diseases (ReCONNET)</td>
<td>Rheumatology Unit, Azienda Ospedaliero Universitaria Pisana, ITALY</td>
<td>Marta Mosca</td>
</tr>
<tr>
<td>Rare Craniofacial and ENT</td>
<td>TBC</td>
<td></td>
</tr>
<tr>
<td>Rare Endocrine Diseases (ENDO-ERN)</td>
<td>Leiden University Medical Centre – THE NETHERLANDS</td>
<td>Alberto Pereira</td>
</tr>
<tr>
<td>Rare Eye Diseases (ERN-EYE)</td>
<td>Hôpitaux Universitaires de Strasbourg – FRANCE</td>
<td>Hélène Dollfus</td>
</tr>
<tr>
<td>Rare Gastrointestinal Diseases (ERN-CAM)</td>
<td>Erasmus Medical Centre, Rotterdam – THE NETHERLANDS</td>
<td>Rene Wijnen</td>
</tr>
<tr>
<td>Rare Gynaecological &amp; Obstetric Diseases</td>
<td>TBC</td>
<td></td>
</tr>
<tr>
<td>Rare Haematological Diseases (eurobloodnET)</td>
<td>Hospital Clinic of Barcelona – SPAIN</td>
<td>Joan-Lluis Vives Corrons</td>
</tr>
<tr>
<td>Rare Hepatic Diseases (ERN-LIVER)</td>
<td>Newcastle Upon Tyne Hospitals – UNITED KINGDOM</td>
<td>David Jones</td>
</tr>
<tr>
<td>Rare Hereditary Metabolic Diseases (MetabERN)</td>
<td>Centre for Rare Diseases (ZSE) Helios Dr. Schmidt Kliniken Wiesbaden – GERMANY</td>
<td>Maurizio Scarpa</td>
</tr>
<tr>
<td>Rare Immunological and Auto Inflammatory Diseases (RITA)</td>
<td>NUTH, UNITED KINGDOM</td>
<td>Andrew Cant</td>
</tr>
<tr>
<td>Rare Malformations and Developmental Anomalies and Rare Intellectual Disabilities (ITHACA)</td>
<td>Central Manchester University Hospitals, UNITED KINGDOM</td>
<td>Jill Clayton-Smith</td>
</tr>
<tr>
<td>Rare Multi-systemic Vascular Diseases</td>
<td>TBC</td>
<td></td>
</tr>
<tr>
<td>Rare Neurological Diseases* (ERN-RND)</td>
<td>University Hospital Tübingen – GERMANY</td>
<td>Holm Graessner</td>
</tr>
<tr>
<td>Rare Neuromuscular Diseases (EURO-NMD)</td>
<td>John Walton Muscular Dystrophy Research Centre, Newcastle Upon Tyne Hospitals UNITED KINGDOM</td>
<td>Kate Bushby (Teresinha Evangelista)</td>
</tr>
<tr>
<td>Rare Pulmonary Diseases (ERN LUNG)</td>
<td>Klinikum Goethe University Frankfurt – GERMANY</td>
<td>Thomas Wagner</td>
</tr>
<tr>
<td>Rare Renal Diseases (ERKNET)</td>
<td>Heidelberg University Hospital – GERMANY</td>
<td>Franz Schaefer</td>
</tr>
<tr>
<td>Rare Skin Diseases (ERN on Rare and Undiagnosed Skin Disorders) ***</td>
<td>MAGEC, Service de Dermatologie, Hôpital Necker Enfants Malades – FRANCE</td>
<td>Christine Bodemer</td>
</tr>
<tr>
<td>Rare Urogenital Diseases</td>
<td>Sheffield Teaching Hospitals NHS Foundation Trust – UNITED KINGDOM</td>
<td>Chris Chapple (Michelle Battye)</td>
</tr>
</tbody>
</table>

Note: TBC indicates that the name of the Coordinator is not specified.
Priority on Europe while keeping an international perspective
**RareConnect**

- Expanded to 83 global communities in collaboration with 680 patient organisations worldwide and 280 volunteer moderators

- New feature: RareConnect discussion groups covering both specific rare diseases as well as topics which go across diseases - 200 discussion groups to date

- New logo and visual identity
Rare Disease Day 2015

- Organised in collaboration with partners in over 80 countries worldwide.

- Rare Disease Day video translated into 27 languages with 1.5 million views on EURORDIS social media.

- Over 1000 events including a Rare Disease Day European Policy Event in Brussels entitled ‘Rare but Real: Talking Rare Diseases’.
Rare Disease Day official video
Published in 34 languages
RARE DISEASES INTERNATIONAL

• Officially launched in Madrid in May 2015 with over 80 participants

• Joint Declaration on “Rare Diseases as an International Public Health Priority” adopted

• Website, social media & RareConnect discussion group launched

• Governance structure and related documents developed and adopted by the PFG
UN NGO Committee on Rare Diseases

“Substantive Committee of the Conference of Non-Governmental Organisations (CoNGO) in Consultative Status with the United Nations’ Economic and Social Council (ECOSOC)”

Timeline:

- 2014: Received mandate for the creation of the UN Committee on Rare Diseases thanks to Agrenska Foundation - unanimously approved by all 30+ members of the CONGO present

- **October 2015**: First inception meeting held in New York at the CONGO

- **Early 2016**: Adoption of by-laws & start composition of Committee

- **8 November 2016**: 1st Event of UN Committee in New York UN Buildings
2015 EURORDIS KEY ACHIEVEMENTS

For a full description of the activities that took place in 2015 and the Workplan 2016, please refer to the Activity Report in your bags!

You can also download it from the website...and disseminate it to your members and key contacts.
EURORDIS Board of Directors

EURORDIS Staff
Financial Report 2015
& Auditor’s Report
Dimitrios Synodinos - Treasurer
26 MAY, 2016 EDINBURGH, UK
EURORDIS 2015 REVENUE AND EXPENSES

REVENUE BY ORIGIN 2015
5,099 K€

- 25% Health Sector Corporates
- 36% Patient Organisations and Volunteers
- 29% European Commission
- 3% Other Corporates
- 5% Other
- 2% Foundations and NPOs

EXPENSES BY TYPE 2015
5,049 K€

- 45% Staff Costs
- 22% Services
- 20% Volunteers
- 11% Travel and Subsistence
- 2% Others

EURORDIS GENERAL ASSEMBLY - MAY 2016 – EDINBURGH
Thank you for your support
Auditor’s Special Report on regulated conventions & renewal of mandate
Dimitrios Synodinos - Treasurer

26 May, 2016 Edinburgh, UK
Candidates to the Board of Directors
Avril Daly, Vice-President

26 May, 2016 Edinburgh, UK
CANDIDATES 2016

- Terkel Andersen, Danish Haemophilia Society, Denmark
- John Dart, DEBRA International, UK
- Anne-Sophie Lapointe, Vaincre les Maladies Lysosomales, France
- Oliver Timmis, AKU Society, UK
- Lex van der Heijden, CMTC-OVM, The Netherlands
- Geske Wehr, Selbshilfe Ichthyose e.V., Germany
- Vlasta Zmazek, Croatian Alliance for Rare Diseases, Croatia
Workplan 2016
Yann Le Cam, Chief Executive Officer

26 May, 2016
FOCUS ON FEW KEY MESSAGES WHEN LOOKING AHEAD AT 2015

• Revisiting our basics and adapting for our Strategy 2016-2020
• Becoming a more agile networking organisation
• Powering our Advocacy capacities & Evidence based Patient Perspectives for Patient Perspective based Policy
• It’s all about Patient Empowerment & Patient Engagement
• It’s all for Improving Patient Access & Patient Health Outcomes
• A daring budget with a clear and accepted risk, an investment into new programmes which in time will catalyse new resources
Revisiting our basics and adapting for our Strategy 2016-2020
The value EURORDIS creates for People Living With a Rare Disease

- Catalyse transformational change
- Act as a knowledge broker
- Connect members and PLWRD
- Empower members and individual advocates
- Elevate the patient perspective
- Provide a cohesive voice

Alliance of over 700 rare disease organisations from more than 60 countries
EURORDIS’ distinctive characteristics

<table>
<thead>
<tr>
<th>Patients first</th>
<th>Authentic</th>
<th>Authoritative</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Legitimate</td>
<td>• Credible</td>
<td>• Professional</td>
</tr>
<tr>
<td>• Holistic</td>
<td>• Trustworthy</td>
<td>• Leader</td>
</tr>
<tr>
<td>• Humanistic</td>
<td>• Genuine</td>
<td>• Well-informed</td>
</tr>
<tr>
<td>• Solidarity</td>
<td>• Representative</td>
<td>• Evidence-based</td>
</tr>
<tr>
<td>• Balanced use of emotion</td>
<td>• «Finger on the pulse»</td>
<td>• Intellectual</td>
</tr>
<tr>
<td>• Grass roots</td>
<td></td>
<td>• Institutional</td>
</tr>
<tr>
<td>• Independent</td>
<td></td>
<td>• Purist</td>
</tr>
<tr>
<td></td>
<td></td>
<td>• Serious</td>
</tr>
<tr>
<td></td>
<td></td>
<td>• Reserved</td>
</tr>
<tr>
<td>Courageous</td>
<td>Collaborative</td>
<td>Innovative</td>
</tr>
<tr>
<td>• Straightforward</td>
<td>• Respectful</td>
<td>• Visionary</td>
</tr>
<tr>
<td>• Blunt</td>
<td>• Collegiate</td>
<td>• Enterprising</td>
</tr>
<tr>
<td>• Brave</td>
<td>• Inclusive</td>
<td>• Creative</td>
</tr>
<tr>
<td>• Relentless</td>
<td>• Non-competitive</td>
<td>• «Can-do» culture</td>
</tr>
<tr>
<td>• Persistent</td>
<td>• Consultative</td>
<td>• Open-minded</td>
</tr>
<tr>
<td>• Consistent</td>
<td>• Pragmatic</td>
<td>• Pioneering</td>
</tr>
<tr>
<td>• No «pussy footing»</td>
<td>• Modest</td>
<td></td>
</tr>
<tr>
<td></td>
<td>• Humble</td>
<td></td>
</tr>
</tbody>
</table>
ADAPTING TO OUR STRATEGY 2016-2020

• Governance: revise Statutes & create EURORDIS By-Laws

• Refresh Logo & Identity

• Revisit communication basics rare diseases & Eurordis

• Upgrade, redesign and reorganise content of website

• Bring RareConnect to the core of Eurordis and the fore of our work process as a platform to integrate RD patient organisations, patient advocates, volunteers and staff around diseases, disease groupings, disease features, topics linked to our work priorities => to streamline our activities, enhance direct participation, promote horizontal communication, share more and learn more from each other
EURORDIS is becoming a more agile networking organisation
A NETWORK ORGANISATION

- Members – 700 to 1 000
- Network of National Alliances - 32 +
- Network of European Federations & Informal Networks - 52 +
- Network of Members of Rare Diseases International – 35 to 100
- European Patient Advocacy Groups for each Rare Disease Grouping mirroring the ERNs and future clinical research networks or Community Advisory Boards: each ePAG is a network and all ePAGs are a network – 22 ePAGs, 77 to 100 reps

- Volunteers – different diseases, different cultures, different activities, different depth of commitment – 300 to 1 000
- Staff – different status (self-employed, interns), distributed
- Alumni of Trainings – 250 to 1 000
- RareConnect – its 600+ partnering patient organisations, its volunteer moderators, its 20 000+ members
VOLUNTEERS AND STAFF

320 + volunteers

40 + staff members

Paris
Brussels
Barcelona
Zagreb
Belgrade
London
Geneva
## ePAG Patient Representatives as of May 2016

<table>
<thead>
<tr>
<th>Disease Grouping</th>
<th>ePAG</th>
<th>Total Responses</th>
<th>Elected patient reps</th>
<th>Elected reps (Non-members)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Neurological</td>
<td>119</td>
<td>6</td>
<td>6</td>
<td>1</td>
</tr>
<tr>
<td>Malformations</td>
<td>88</td>
<td>11</td>
<td>7</td>
<td>1</td>
</tr>
<tr>
<td>Hereditary Meta</td>
<td>72</td>
<td>6</td>
<td>6</td>
<td>1</td>
</tr>
<tr>
<td>Skin</td>
<td>72</td>
<td>6</td>
<td>6</td>
<td>1</td>
</tr>
<tr>
<td>Neuromuscular</td>
<td>64</td>
<td>10</td>
<td>2</td>
<td>2</td>
</tr>
<tr>
<td>Immuno</td>
<td>56</td>
<td>7</td>
<td>5</td>
<td>0</td>
</tr>
<tr>
<td>Haematological</td>
<td>56</td>
<td>9</td>
<td>5</td>
<td>3</td>
</tr>
<tr>
<td>Con Tissue</td>
<td>53</td>
<td>3</td>
<td>3</td>
<td>2</td>
</tr>
<tr>
<td>Cancer/Tumours</td>
<td>47</td>
<td>7</td>
<td>5</td>
<td>2</td>
</tr>
<tr>
<td>Lung</td>
<td>41</td>
<td>9</td>
<td>5</td>
<td>1</td>
</tr>
<tr>
<td>Bone</td>
<td>32</td>
<td>3</td>
<td>3</td>
<td>0</td>
</tr>
<tr>
<td>Vascular</td>
<td>29</td>
<td>5</td>
<td>5</td>
<td>2</td>
</tr>
<tr>
<td>Eye</td>
<td>27</td>
<td>5</td>
<td>5</td>
<td>1</td>
</tr>
<tr>
<td>Endocrine</td>
<td>23</td>
<td>2</td>
<td>3</td>
<td>0</td>
</tr>
<tr>
<td>Renal</td>
<td>17</td>
<td>2</td>
<td>2</td>
<td>0</td>
</tr>
<tr>
<td>Urogenital</td>
<td>14</td>
<td>2</td>
<td>2</td>
<td>1</td>
</tr>
<tr>
<td>Cardiac</td>
<td>10</td>
<td>0</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>Paediatric Cancer</td>
<td>9</td>
<td>3</td>
<td>3</td>
<td>2</td>
</tr>
<tr>
<td>Hepatic</td>
<td>9</td>
<td>2</td>
<td>2</td>
<td>0</td>
</tr>
<tr>
<td>Craniofacial</td>
<td>5</td>
<td>1</td>
<td>1</td>
<td>1</td>
</tr>
<tr>
<td>Gastro</td>
<td>4</td>
<td>1</td>
<td>1</td>
<td>1</td>
</tr>
<tr>
<td>Gynae</td>
<td>1</td>
<td>0</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td><strong>Total</strong></td>
<td>848</td>
<td>100</td>
<td>77</td>
<td>29%</td>
</tr>
</tbody>
</table>
EURORDIS’ REPRESENTATION
IN EXTERNAL NETWORKS, ORGANISATIONS AND INSTITUTIONS IN 2016

Member of European Networks:
- E-Rare
- EuroBioBank
- ECRIN
- BBMRI Stakeholders Forum
- Treat NMD
- RD-Connect
- SCOPE Joint Action (Advisory Board)
- OpenMedicine
- IMI EUPATI
- IMI ADAPT-SMART
- MoCA: Mechanism of Coordinated Access

Partnership Learned Societies:
- European Federation of Internal Medicine (EFIM)
- European Hospital & Healthcare Federation (HOPE)
- International Federation of Social Workers Europe (IFSW-Europe)
- European Society of Human Genetics (ESHG)
- International Society for Pharma co-economics and Outcomes Research (ISPOR)

European Commission

Commission Expert Group on
Rare Diseases

Commission Expert Group on
Cancer Control

EU Health Policy Forum

Health Technology Assessment (HTA)

HTA Network

EUnetHTA Stakeholder Forum

Scientific Early European Dialogue (SEED)

Joint Research Center
EU Platform Rare Diseases Registration (JRC)

European and International
Not-for-Profit Organisations:
- DIA: Drug Information Association
- EFPIA Think Tank: European Federation of Pharmaceutical Industries and Associations
- EUROPABIO Patients Advisory Group
- EUCOPE
- EPF: European Patients’ Forum
- EFGCP: European Forum for Good Clinical Practice
- FIPRA – International Policy Advisors
- Friends of Europe
- NEWDIGS: New Drug Development Paradigm
- IAPO: International Alliance of Patients’ Organizations
- IRDiRC: International Rare Disease Research Consortium
- ICORD: International Conference on Rare Diseases and Orphan Drugs
- Rare Cancer Europe
- Social Platform
- Maladies Rares Info Service (French Helpline for RDs)
- Rare Disease Platform in Paris

European Medicines Agency

EMA

Committee for Orphan Medicinal Products

COMP

Paediatric Committee

PDCO

Committee for Advanced Therapies

CAT

Patients’ & Consumers’ Working Party

PCWP

Scientific Advice Working Party

SAWP

Committee for Medicinal Products for Human Use

CHMP

EU clinical trials portal and Union database stakeholders group

EU clinical trials portal

EMAtask Force on Registries

Member of European Networks:

- E-Rare
- EuroBioBank
- ECRIN
- BBMRI Stakeholders Forum
- Treat NMD
- RD-Connect
- SCOPE Joint Action (Advisory Board)
- OpenMedicine
- IMI EUPATI
- IMI ADAPT-SMART
- MoCA: Mechanism of Coordinated Access

Partnership Learned Societies:

- European Federation of Internal Medicine (EFIM)
- European Hospital & Healthcare Federation (HOPE)
- International Federation of Social Workers Europe (IFSW-Europe)
- European Society of Human Genetics (ESHG)
- International Society for Pharma co-economics and Outcomes Research (ISPOR)
Powering our Patient Advocacy
Evidence based Patient Perspective
Patient Perspective based Policy
POWERING OUR ADVOCACY

• European & International Advocacy team is strengthened:
  • Public Affairs Directors: Valentina, head, and Jean Louis
  • Public Affair Managers & Junior: Ariane, Annette (1/2), Jenny

• Improving the way we work:
  • Integrate more European level with national & international
  • Involve most managers in the team
  • Integrated in projects eg Joint Actions, InnovCare
  • Link more to dedicated group of volunteers or members
  • Collaboration for faster change: CEGs, partners, panel experts
  • Consult members and call webinars linked to specific actions
  • More direct work with CNA, CEF, ePAGs, RDI

• Stronger capacities in:
  • Overall EU policy, national policies, international policy
  • Research, HealthCare, Public Health, Social, Regulatory, Access

• Rare Barometer:
  • An innovative programme to go beyond and faster EurordisCare
  • Produce Evidence based Patient Perspective
  • Advocate for Patient Perspective based Policy
eurordis.org/voices
PATIENT ENGAGEMENT

• EURORDIS strategy has always been to participate, to facilitate rare disease patient advocate active participation

• Patient Engagement in:
  • Research
  • Healthcare
  • Product life cycle

• We have created the opportunities, now we need to make sure we have the people and capacities to engage!
  • Healthcare: ERN, CoE, Registries & cohorts, Best Practice of diagnosis & care
  • Product: EMA, HTA & EUnetHTA, MOCA & Payers; academia; industry… a change of scale
New Framework for Patient Engagement at EURORDIS

• Hiring of Patient Engagement Manager – Elisa
  also François, Virginie, Jean Louis, Nancy, Anja

• Patient Engagement in EMA & HTA activities
  – Protocol Assistance, Scientific Advice
  – Scientific Advisory Groups
  – European Early Dialogue HTA/Scientific Advice, and // advice
  – Pilot on B/R assesment at CHMP
  – EMA/IMI-related project (ADAPT-SMART, WEB-RADAR)

• Patient Engagement in activities outside EMA
  – MoCA
  – Ad hoc requests
  – Future opportunities & challenges
New Framework for Patient Engagement

EURORDIS

EMA Protocol assistance

HTA

MoCA

Ad hoc activities

Patient Engagement Manager

TAG meets Board 2016, Barcelona, 01 Apr 2016
PATIENT EMPOWERMENT

• Website & eNews & Members News
• Working Groups, Task Forces, etc
• Workshops F2F & Eurordis Membership Meeting
• Webinars
• Trainings: Summer School & EUPATI + Social Media + new trainings
• eLearnings & Tutorials
• RareConnect communities per topics, group of diseases, working groups or activities
Patient Access & Patients Health Outcomes
PATIENT HEALTH OUTCOMES

• Therapy:
  • Patients Relevant Outcome Measures (PROM / PCOM)
  • Patients Reported Outcomes
  • Outcome based pricing & reimbursement

• European Reference Networks & Centres of Expertise & Healthcare Pathways:
  • Application and evaluation based on Assessment Manual & Tool, is leading toward clinical excellence and improved patient health outcomes
  • Patient registrations, cohorts, registries to collect data and measure outcomes, compare outcomes..
  • Best practice of Diag & Care to improve outcomes

• Social:
  • Innovative social services for measurable outcomes
Budget 2016
EURORDIS Funding from 2002 to 2016

- ECRD (+29%)
- Event fees
- NonProfit Org.
- Other Corp.
- Pharma. Cies
- European Commission
- Volunteers
- Mem.
- AFM-Téléthon

Years: 2002 to 2016

Funding levels: 0 k€ to 7,000 k€