EURORDIS Summer School

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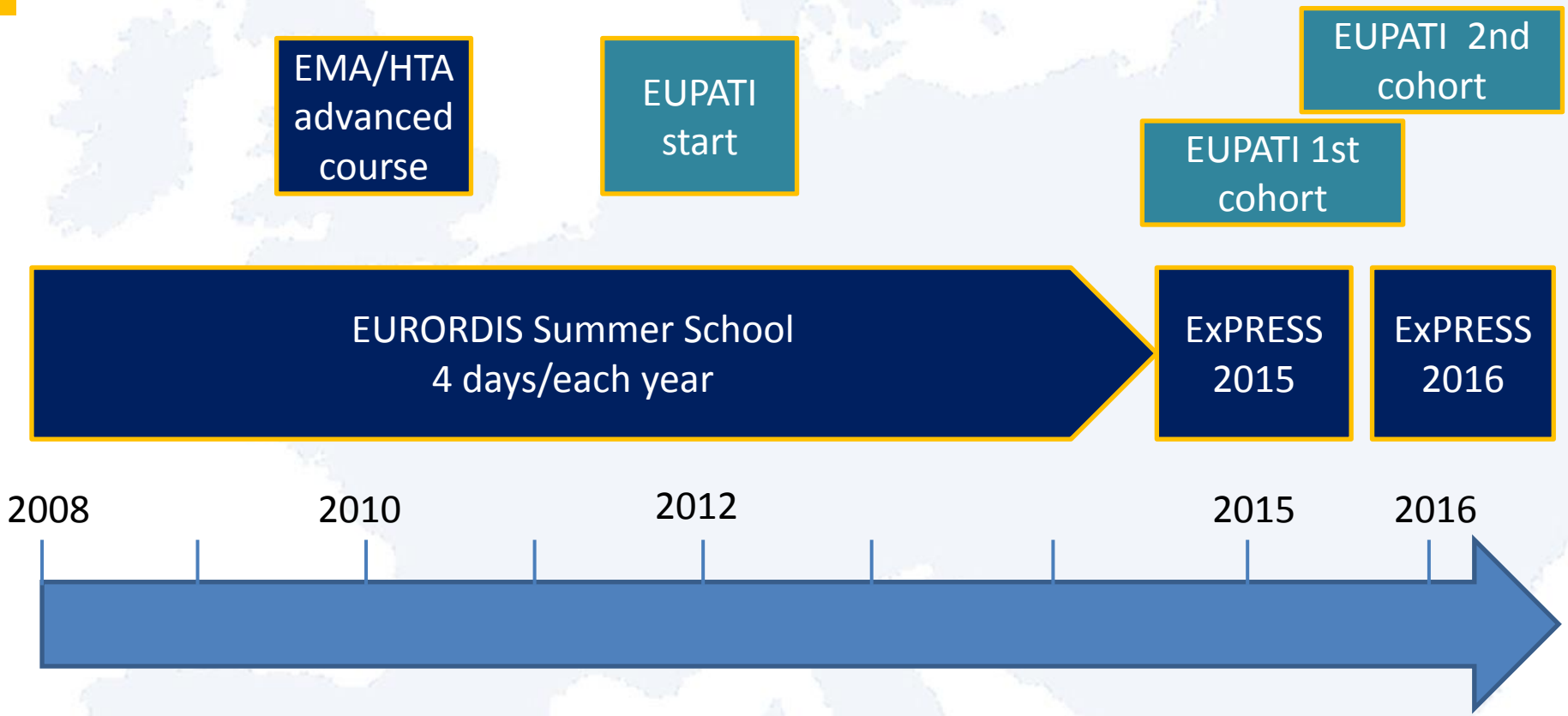
PCWP meeting, 26th November, EMA, London, UK

Who are we?

An international non-profit, non-governmental umbrella rare disease patients' organisation representing an estimated 30 million individuals in Europe. Our Mission:

- To build a strong **pan-European community** of patient organisations and people living with rare diseases
- To be **their voice** at the European level
- To directly or indirectly fight against the impact of rare diseases on their lives

Training programmes at EURORDIS



Objectives of the Summer School

- Capacity building programme
- Patient engagement all along the life cycle of product development
- Content is continuously adapted in line with evolution in the field of regulation and processes

Format & Content

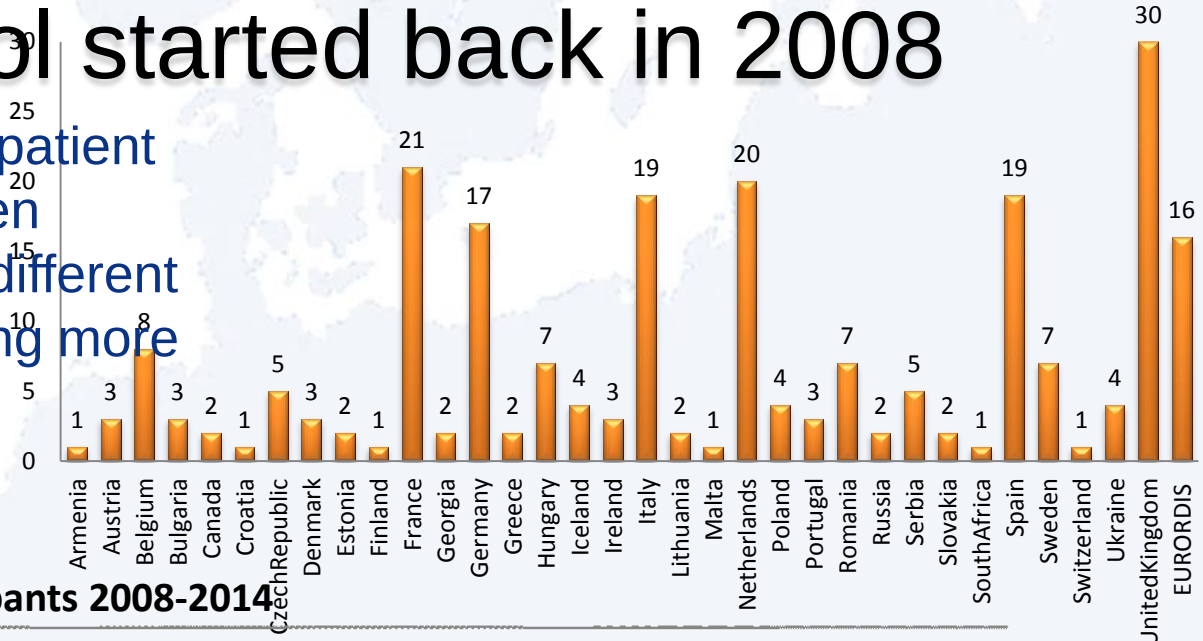
- 4-days F2F in Barcelona (formal lectures, breakout sessions, mini COMP, mini PDCO, etc.) coupled with online training (quizzes, video recordings, webinars)
- Content not Rare Disease specific, collaboration with ECRIN (participants from other medical fields, medical devices, nutrition, etc)
- Evidence-based medicine, clinical research, clinical trials methodology, ethics, statistics, regulatory principles & processes in the EU, EMA organisation and opportunities for patients, HTA appraisal, pharmacovigilance, etc.

Organisation of four-day programme

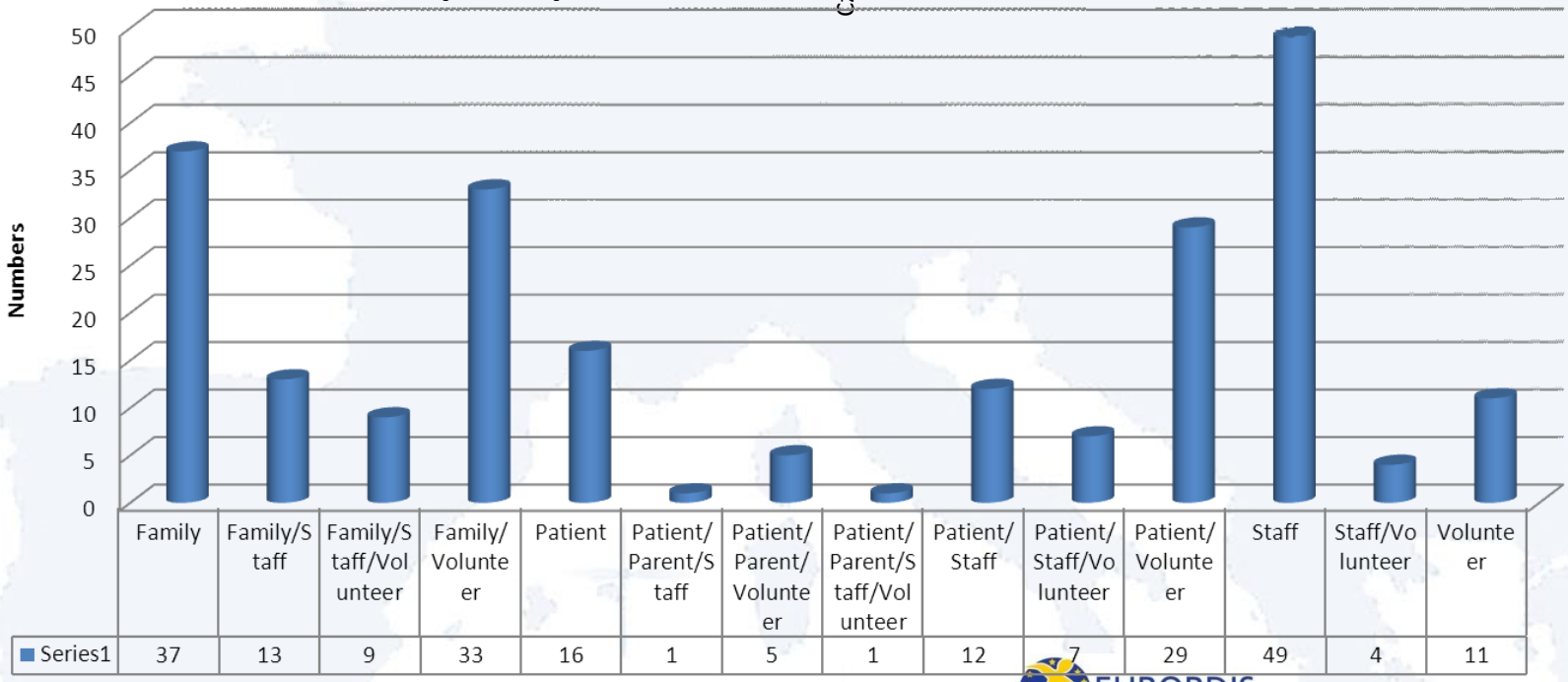
- Pre-Summer School preparation
- Four-day programme
  - General organisation
    - Days 1 and 2 – Clinical trials and Drug development
    - Days 3 and 4 – EMA overview and committees, HTA, etc.
  - Specific organisation
    - Mix of small group sessions and large group formal presentations
    - Problem-based learning model
    - Small group sessions of 10 -12 individuals (maximum) to encourage interactions and exchanges
    - Case-based tutorials

Summer School started back in 2008

- From 2008 to 2014, 227 patient representatives have been trained, coming from 33 different countries and representing more than 75 diseases



Distribution / Countries of the participants 2008-2014



“ExPRESS Yourself!”

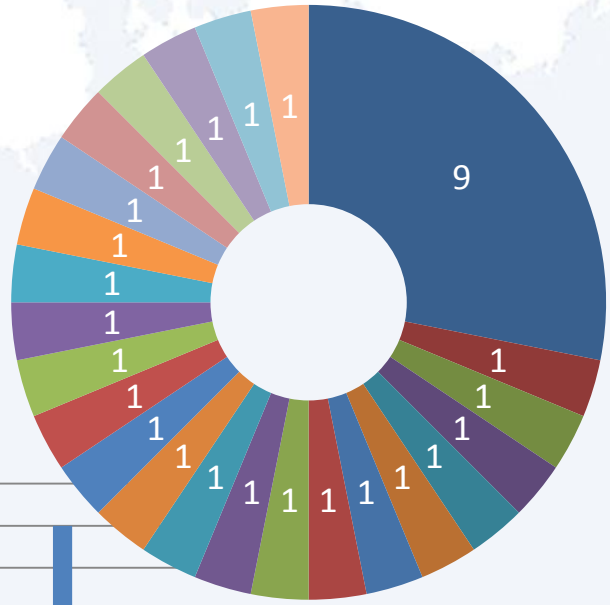
Expert Patients and Researchers
EURORDIS Summer School

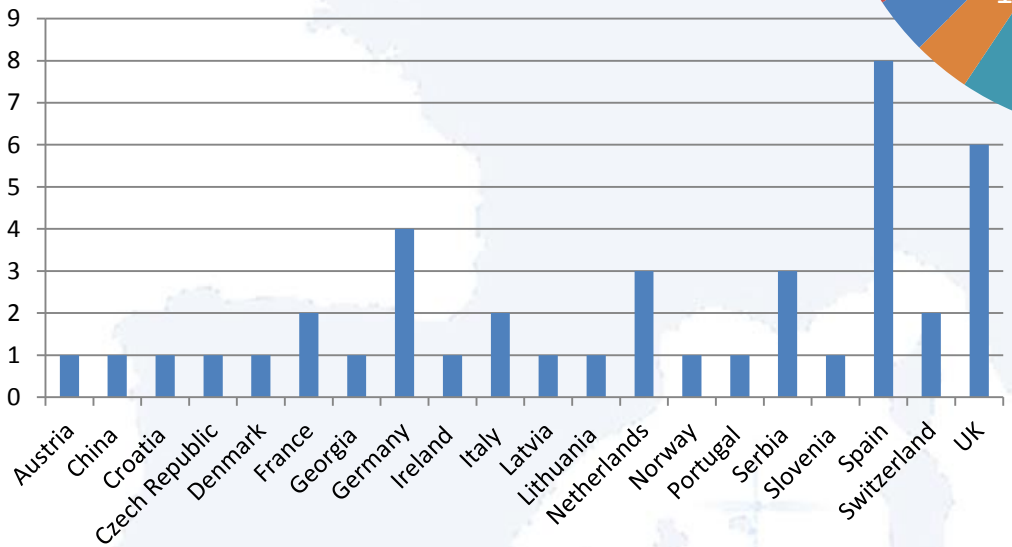
UAB Campus Barcelona, Spain

June 1 - 5, 2015

ExPRESS 2015!

- 30 patient representatives
- 12 academic researchers
- 20 different countries





All rare
Child with Cystic Fibrosis
Esophageal Atresia and Tracheo Esophogeal Fistula
Fabry
Gaucher
Juvenile Idiopathic arthritis
Malattie metaboliche ereditarie
Melenomas Uveal
Morbus Osler
Mucopolysaccaridosis
Mucopolysaccharidosis type IV
Myeloma
Neurofibromatosis
Neuromuscular and Mitochondrial Pathology
Niemann Pick Type A B & C
Ocular Melanoma
Osteogenesis imperfecta
Pulmonary arterial hypertension
Pulmonary hypertension
Rare and Inherited retina degenerations

ExPRESS 2015!

- This Summer School was co-organised by:
  - COST Action BM2107
  - European Medicines Agency
  - EURORDIS
  - Platforma Malaties Minoritàries

- Programme Committee:

Annemieke Aartsma-Rus
Monica Ensini
Nathalie Goemans
Elizabeth Vroom

Nathalie Bere
Maria Mavris

Nancy Hamilton
Virginie Hivert
François Houÿez
Yann Le Cam

Josep Torrent-Farnell

Also thanks to:

- Funding from:

Co-funded by
the Health Programme
of the European Union

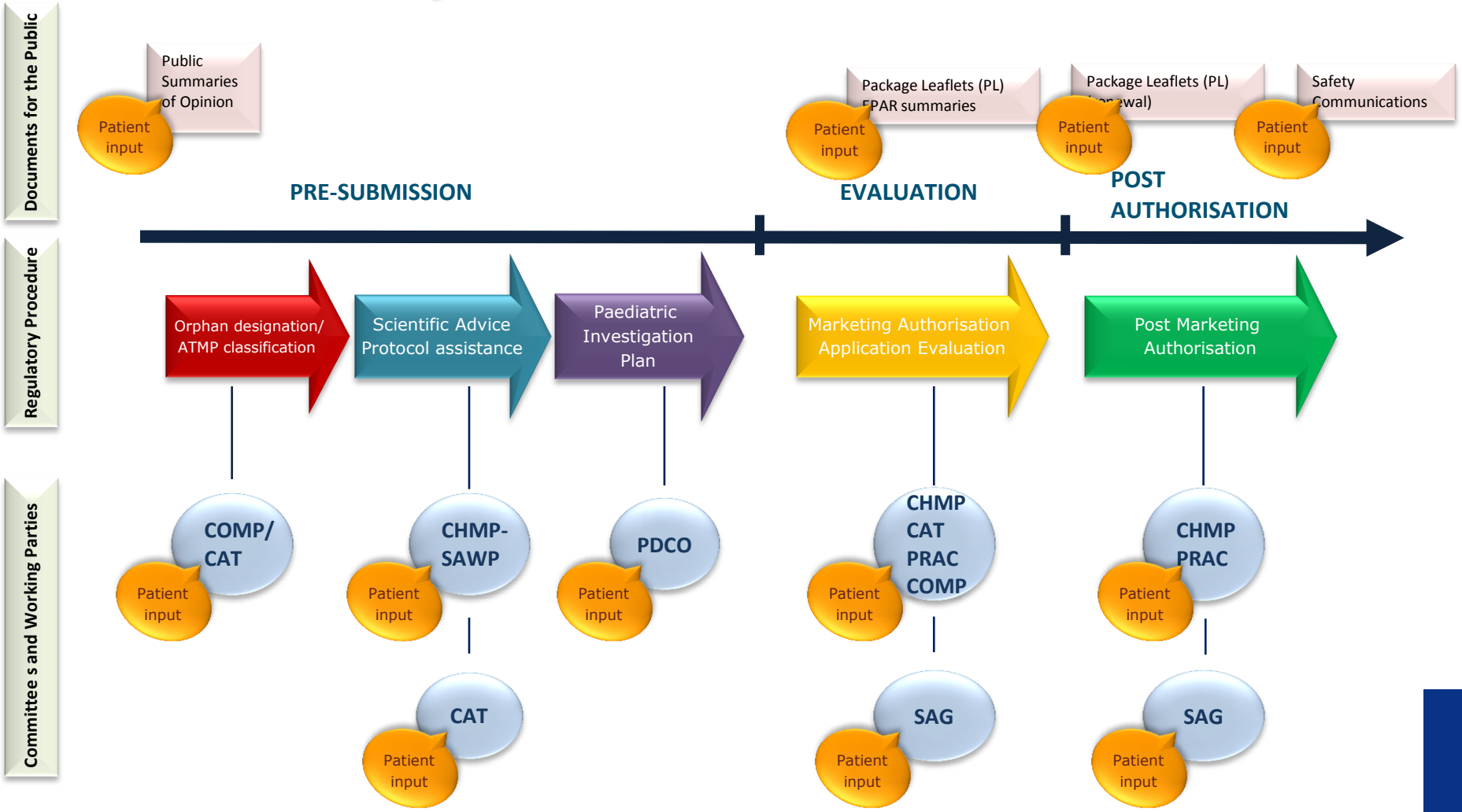
- Coordination and local organisation:
  - Nancy Hamilton (EURORDIS)
  - Iolanda Arbiol (Platforma Malaties Minoritàries)

- Our Faculty: Great thanks!! Highly committed people, academics, regulators, industry and expert patients – some of them since the beginning

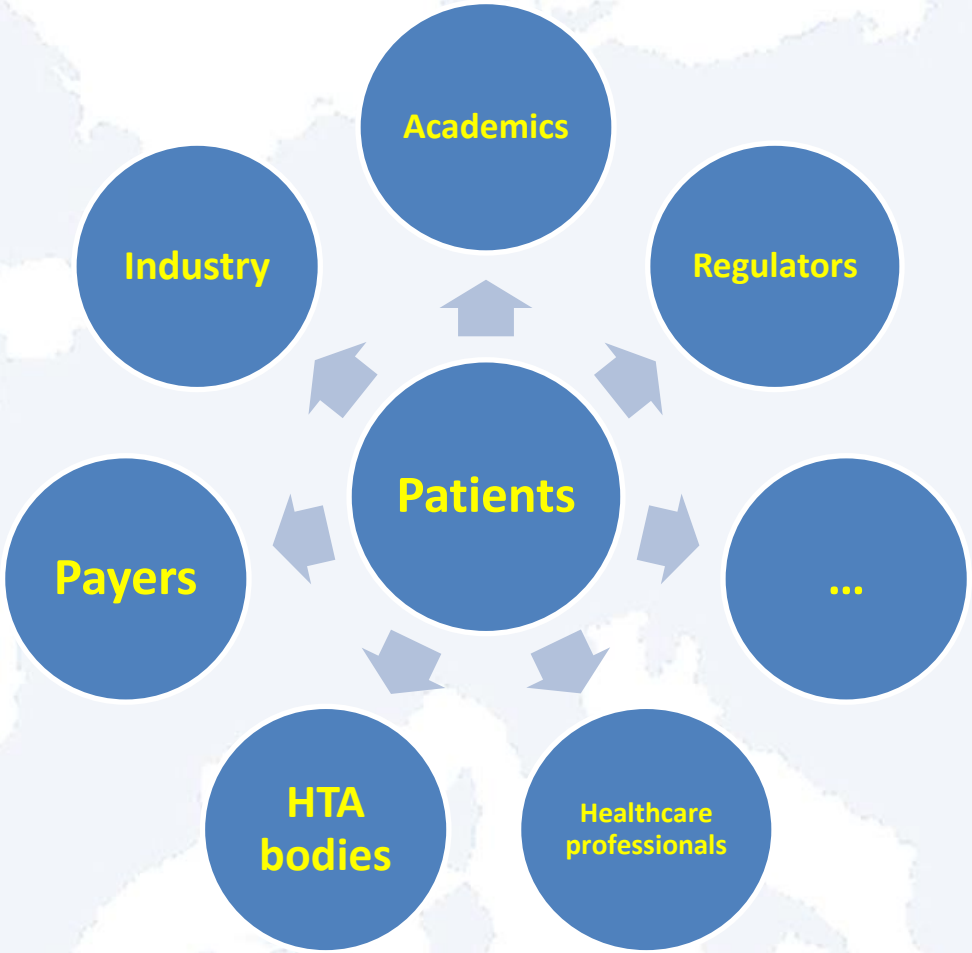
What is it about? & What is new?

- First Training Programme with Patients and Researchers being trained together
- Blended approach with plenary/formal lectures, small groups sessions, round table discussion and practical exercises ('mock' COMP & SAWP, review of product information, etc.)
- Capacity building Programme → increasing involvement in the R&D processes, Health Technology Assessment, information and access to medicines

Opportunities for Patient involvement along the medicine lifecycle at EMA



Patient involvement all along the product life cycle: interaction with all the stakeholders



Feedback from patients

- **Question - what did you think about having the researchers with us in the training:**

- In discussions with the researchers, I expected more disagreement.
- We never get the chance to exchange with researchers, so it was very special to be able to approach them and realize that they are also normal people!
- In the sessions, it was nice to listen to someone looking at diseases at a totally different perspective.
- It was nice to make personal connections with them as people and see how they relate to our diseases in a different way.
- It was interesting to learn about what they are doing and about what other diseases are doing. The networking opportunity was appreciated.

Feedback from academics

- **The best thing/what they liked most**

- Learned a LOT
- Interaction with patients
- Small group sessions (some said this was where they learned most)
- This is a very unique opportunity to get this type of training
- Participants noted that they have a changed view on the regulatory process and regulators; they understand more why regulation is needed and were pleasantly surprised about how nice and approachable the regulators are
- Patient engagement was made much more tangible, researchers more convinced now that patients can be involved in research every step of the way
- How cultural differences became apparent … and can be better understood

We are now working on ExPRESS 2016

- Online content accumulated from previous editions of the EURORDIS Summer School such as webcasts and slide presentations as well as webinars, and interactive training modules.
- Even more hands-on/practical approach related to opportunities offered to be engaged into therapeutic development and regulatory processes (Scientific Advice/Protocol Assistance, Early dialogue with HTA bodies, Benefit/Risk assessment at CHMP, Adaptive Pathways, etc.)
- Reinforcement of networking and interactions between patients and researchers - sharing experiences & peer-to-peer training

Summer School → Emergence of leadership + Network of alumnis






From 2008 to 2013

THANK YOU!

2014

2015