Session 3: Way forward – The views of patients
Workshop Patient Engagement Strategy for Innovative Medicines
Yann Le Cam
CEO EURORDIS
EURORDIS IN BRIEF

International non-governmental organisation not-for-profit

Founded in 1997

705 members rare diseases patient organisations

In 63 countries

40 + staff members

320 + volunteers

Paris
Brussels
Barcelona
Zagreb
Belgrade
London
Geneva
EURORDIS & TRAINING

- since 2008
- F2F workshop
- e-learning material

300 alumni from 33 countries

- Full partner
- Content development
- F2F workshops
EURORDIS’ REPRESENTATION IN EXTERNAL NETWORKS, ORGANISATIONS AND INSTITUTIONS IN 2015

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

Learned Societies:
- European Federation of Internal Medicine (EFIM)
- European Hospital & Healthcare Federation (HOPE) - ongoing
- International Federation of Social Workers (IFSW) – ongoing
- European Society of Human Genetics (ESHG) – ongoing
- International Society for Pharmaconomics and Outcomes Research (ISPOR)

**European and International Not-for-Profit Organisations:**
- DIA: Drug Information Association
- EFPIA Think Tank: European Federation of Pharmaceutical Industries and Associations
- EMA-EFPIA Tapestry Group
- EUROPABIO Patients Advisory Group
- EUCOPE
- EPF: European Patients’ Forum
- EFGCP: European Forum for Good Clinical Practice
- 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
- Friends of Europe
- Maladies Rares Info Service (French Helpline for RDs)
- Rare Disease Platform in Paris

**European Commission**
- Commission Expert Group on Rare Diseases
- Commission Expert Group on Cancer Control
- EU Health Policy Forum
- Health Technology Assessment (HTA)
- EUnetHTA Stakeholder Forum
- Scientific Early European Dialogue (SEED)
- MoCCA

**European Medicines Agency (EMA)**
- COMP: Committee for Orphan Medicinal Products
- PDCO: Paediatric Committee
- CAT: Committee for Advanced Therapies
- PCWP: Patients’ & Consumers’ Working Party
- SAWP: Scientific Advice Working Party
- CHMP: Committee for Medicinal Products for Human Use
- EU clinical trials portal and Union database stakeholders group
- EMA Task Force on Registries

**European Network Members**
- E-Rare
- EuroBioBank
- ECRIN
- BBMRI Stakeholders Forum
- Treat NMD
- RD-Connect
- SCOPE Joint Action (Advisory Board)
- OpenMedicine
- IMI EUPATI
- IMI ADAPT-SMART

**Learned Societies**
- European Federation of Internal Medicine (EFIM)
- European Hospital & Healthcare Federation (HOPE) - ongoing
- International Federation of Social Workers (IFSW) – ongoing
- European Society of Human Genetics (ESHG) – ongoing
- International Society for Pharmaconomics and Outcomes Research (ISPOR)
A dialogue with companies involved in the development of orphan medicinal products & rare disease therapies

Since 2004
12 years

over 50 companies

brings together
80-300 representatives from industry, regulatory agencies, patient groups, clinicians, and academics

22nd ERTC workshop
Feb 2015
Rare Diseases: Going Global

23rd ERTC workshop
Sept 2015
Patient-reported Outcomes Measures & Patient-reported Outcomes

Multi-stakeholder Symposium
Feb 2016
Improving Access to Rare Diseases Therapies: Value
Value determination
Pricing
EURORDIS brought the patient perspective during the consultation phase towards the creation of the IMI

EURORDIS is/was involved in several IMI projects

**PARTNER**
![EUPATI](image)
![ADAPTSsMART](image)

**ADVISOR**
![WEB-RADR](image)
![PROTECT](image)

Patient Preference Elicitation in B/R
Objective of the session

To agree on concrete actions towards IMI strategy on patient involvement

All solutions/actions/mechanisms should aim to improve the TRUST between the stakeholders
(neutrality and credibility)
To express a common view regarding the key elements and principles of a meaningful patient throughout the lifecycle of medicines, to enhance patients’ health outcomes

- The continuum from “patient engagement” to “patient centeredness” and “patients health outcomes”
- Along the entire product lifecycle from early dialogue throughout the entire lifecycle of the product
- Cooperation with all stakeholders
- Implementation a structured approach, based on mutual trust
- Operationalisong patient engagement
- International perspective

To devise solutions that are sustainable in the long-term on both levels:
- patient engagement in medicines lifetime
- IMI activities
GOVERNANCE OF THE IMI

“STRUCTURING” PROJECTS

“DISEASE-SPECIFIC” PROJECTS
• Implementation of the IMI-Patient Advisory Council (IMI-PAC)
  o 10 (12) members representing patient organisations and broader
  o A Chair
  o Chair is an Observer to the IMI Governing Board & relevant committee

• Roles
  o To voice the patient perspective within the Governing Board
  o To insure the patient view in the strategy, the process, the call texts

• Procedures
  o Based on Call for Interest coordinated by EPF
  o Selection done by the Governing Board + IMI
  o Same commitment on confidentiality
  o 2 years (+ 2 years) position
  o Supported by the IMI
  o 1 F2F meeting + 2 TC per year
The privileged interlocutors are the umbrella patient organisations to ensure representativeness when relevant and to enable the patient engagement scale-up.

Need to support patient organisations to be even more meaningful partners to build their capacities

- trainings – methodological support

An infrastructure/solution is missing in the European/international landscape to operationalise patient engagement

Triple advantage
- One-stop shop
- Services
- Pre-competitive tool

Training on content (continuation of EUPATI) and processes (new) and of all actors involved

Revenue generation ⇒ better support the patient organisations

i.e fee-based structure for agreed services provision
• The privileged interlocutors are the **disease specific patient organisations**

• **UPSTREAM**: *call life cycle*
  - IMI’s calls should specify, when relevant, that short proposals show patient engagement
  - Patient engagement as review criteria
  - Patient representatives as reviewers of the short proposal
    
    *Processes need to be in place to avoid conflict of interest (= patient as reviewer and actor) (i.e. Patient reviewer could be involved in the advisory board of the project. His/her organisation could not be a full partner of the project)*

• **DOWNSTREAM**: *project life cycle*
  - Regular update to patient organisations (i.e. lay summary in the intermediate reports)
  - Patient participation during annual meetings
  - Patient representatives involves in post project evaluation
A structured approach is needed

When

Where

Who

...to engage?

What for?

Scope and methods of engagement
PATIENT ENGAGEMENT STRATEGY

Where to engage in the research & medicine life cycle?

- **Within the patient group(s)**
  (analysis of needs, registry, natural history study, endpoint studies, patient preferences)

- **With academia**
  (H2020 or IMI projects, in EU infrastructures eg ECRIN, EORTC, in future ERNs, in local research projects)

- **With industry**
  (drug developer or with CROs)

- **With regulators**
  (EMA or National Regulatory Agencies)

- **With HTA**
  (EUnetHTA or national / regional HTA Agencies)

- **With payers**
  (Italy, Netherlands, Germany, Croatia or MOCA)
Unique insights of patients along the whole R&D development life cycle

Setting research priorities
- Study design
- Informed consent
- Setting research priorities

Research priorities and Planning
- Study accessibility
- Patient driven research

Research conduct and operations
- Information / communication / support
  Before, during and after study

Lifecycle management
- Patient Information Leaflet

Level of expertise in R&D required:
- High
- Medium

Source: Geissler, Ryll, EPALCO (2014, unpublished)
• Understand the disease and its impact
• Inform the patient experience of living with the disease and the current state of care
• Determine Patient Preferences of Treatment and the Patient Relevant Outcome Measures
• Contribute to the design of CT choice of statistical methods and eligibility criteria
• Discuss the measurement methods, their relevance and real life practice
• Discuss the selection of sites and patient recruitment plan
• Contribute to Patient Informed Consent and information tools about the study
• Collect Patient reported Outcomes
• Information about results and contribute to interpretation of results
• Communication to the patient community - at large
Who to engage?

*Patients as expert of their individual or collective experience*

- Patient Organisation **representatives** and **Patient Advocates**
- Patients **affiliated** to patient organisations
- Patients **non-affiliated** to patient organisations
Key success factors

- Patient **awareness** & **education**
- Patient trainings on **medicine life cycle** & on patient **engagement**
- **Concrete support** to patient engagement and mediation role
- **Shared structured approach and principles and tools** and training across all stakeholders
- **Trust on independence** and consistency on mutual requirements or expectations
- Management of **Potential Conflict of Interest** transparent realistic proportionate & adaptive
- **Metrics and KPI**
3 KEY WORDS
PUBLIC GOODS - CREDIBILITY - TRUST

3 LEVEL OF ENGAGEMENT
GOVERNANCE
STRUCTURING PROJECTS
DISEASE-SPECIFIC PROJECTS

4 QUESTIONS
HOW – WHEN – WHAT FOR - FOR
THANK YOU FOR YOUR ATTENTION
Session 3: Way forward – The views of patients
Workshop Patient Engagement Strategy for Innovative Medicines

Yann Le Cam
CEO EURORDIS