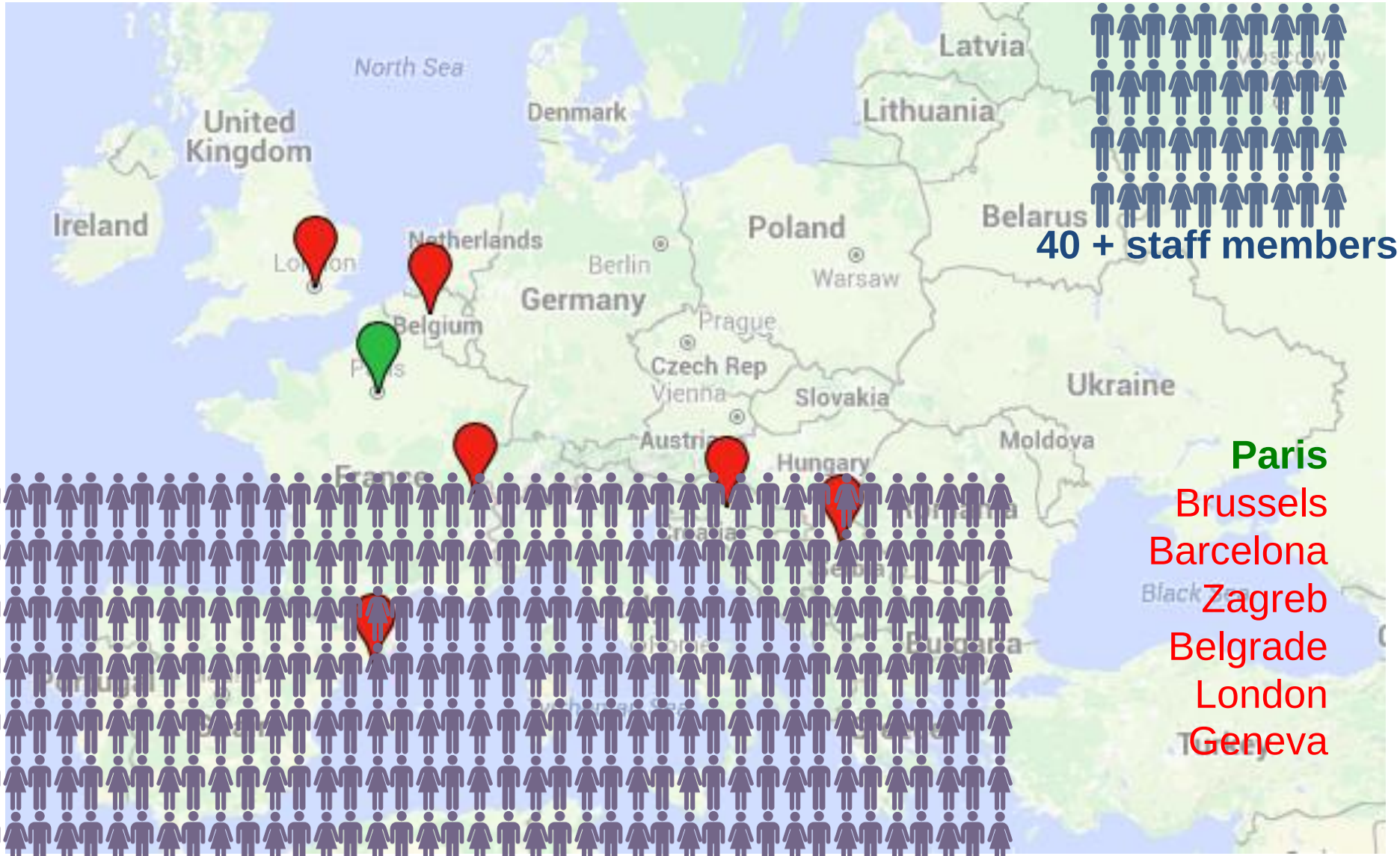
Session 3: Way forward – The views of patients

Workshop Patient Engagement Strategy for Innovative Medicines

Yann Le Cam

CEO EURORDIS

EURORDIS IN BRIEF



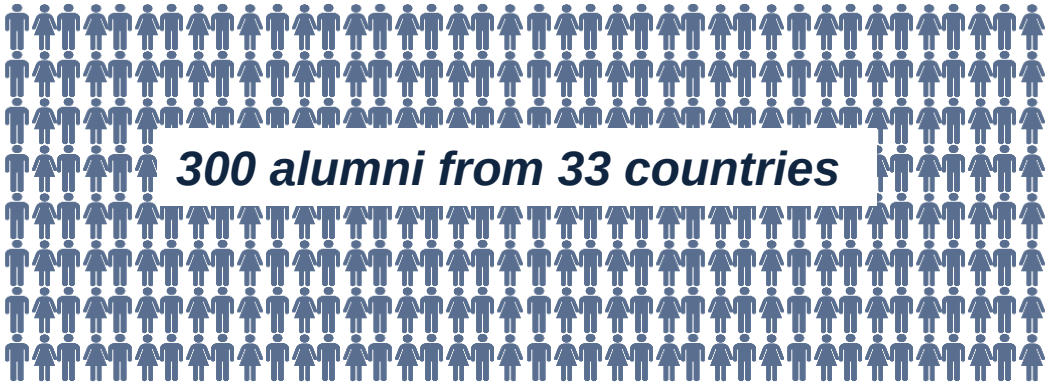
EURORDIS & TRAINING

EURORDIS SUMMER SCHOOL

- since 2008
- F2F workshop
- e-learning material

EUPATI European Patients' Academy on Therapeutic Innovation

- Full partner
- Content development
- F2F workshops



EURORDIS' REPRESENTATION IN EXTERNAL NETWORKS, ORGANISATIONS AND INSTITUTIONS IN 2015

EMA
European Medicines Agency

- 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 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 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

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 Pharmaco-economics and Outcomes Research (ISPOR)

EURORDIS Round Table of Companies

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 rep **industry, regulatory agencies, patient groups, clinicians, 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 & IMI

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
European Patients' Academy on Therapeutic Innovation

ADAPTSMART

WEB-RADR

ADVISOR

PROTECT

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)

BACKGROUND DOCUMENT

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

To devise solutions that are sustainable in the long-term on both levels:

- *patient engagement in medicines lifetime*
- *IMI activities*

✓ 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

✓ Operationalison patient engagement

✓ International perspective

THE 3 DIFFERENT LEVELS OF INVOLVEMENT

GOVERNANCE OF THE IMI

"STRUCTURING" PROJECTS

"DISEASE-SPECIFIC" PROJECTS

- **Implementation of the IMI-Patient Advisory Council (IMI-PAC)**
  - 10 (12) members representing patient organisations and broader
  - A Chair
  - Chair is an Observer to the IMI Governing Board & relevant committee

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

- **Procedures**
  - Based on Call for Interest coordinated by EPF
  - Selection done by the Governing Board + IMI
  - Same commitment on confidentiality
  - 2 years (+ 2 years) position
  - Supported by the IMI
  - 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 shows 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

PATIENT ENGAGEMENT STRATEGY

A structured approach is needed

When

Where ...to engage?

Who

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**

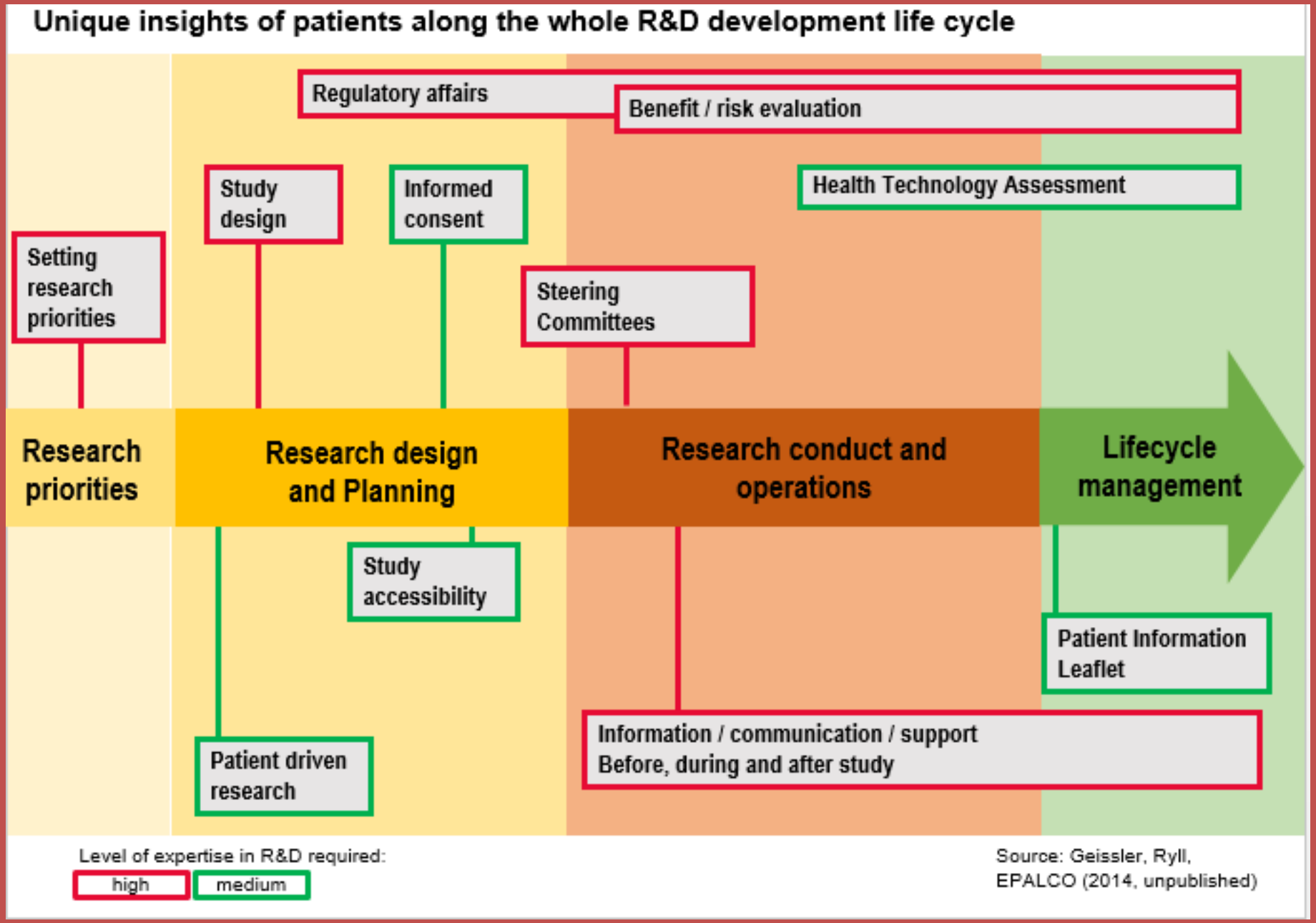
(EMA or National Regulatory Agencies)

- **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
Regulatory affairs
Benefit / risk evaluation
Study design
Informed consent
Health Technology Assessment
Setting research priorities
Steering Committees
Research priorities
Research design and Planning
Research conduct and operations
Lifecycle management
Study accessibility
Patient Information Leaflet
Patient driven research
Information / communication / support
Before, during and after study
Level of expertise in R&D required:
high
medium
Source: Geissler, Ryll, EPALCO (2014, unpublished)

What for? Objective & scope of engagement?

- **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

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