PUBLIC ENGAGEMENT: PRACTICES AND FOOD FOR THOUGHTS

How Patients and consumers work with the European Medicines Agency

First Meeting of EFSA Stakeholder Forum

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30 & 31 May 2017, Parma

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The European Medicines Agency: 1995-2017

- Evaluates the benefit/risks of medicines for their authorisation to be used in humans / animals
  - From early scientific advice (before clinical trials start) to pharmacovigilance (close watch on medicines after their authorisation)
- Centralised procedure: single evaluation by European experts, evaluation report publicly available
  - Mandatory for:
    - HIV/AIDS, cancer, diabetes, neurodegenerative diseases, immune dysfunctions, viral diseases
    - advanced-therapy medicines (gene-therapy, somatic cell-therapy or tissue-engineered medicines, medicines derived from biotech processes)
    - orphan medicines (for rare diseases)
    - some veterinary products
- As of today: 973 active substances authorised, 177 withdrawn, suspended, expired or not renewed, and 46 refused
- 890 staff, 3,800 experts, 564 meetings in 2015, 36,000 visitors in 2015

EMA changed the regulation of medicines in the EU:
Systematic inclusion of real life experience in the EMA regulatory output

- April 1996: EMA met with 6 patients' representatives
  - 2000: Orphan Medicines Legislation and patients as members of the Committee for Orphan Products
  - 2002: workshop on how to work together
  - 2006: framework of interaction with patients and consumers adopted
  - 2014: dedicated Patients and Healthcare Professionals Department created
  - 2016: 770 occasions where patients and consumers were involved in EMA activities
- Moving away from a situation where 12 MS could take different decisions on marketing authorisations
  - To a EU marketing authorisation valid in 31 EEA, MS serving 508 millions citizens
  - With same product name, same information for patients and healthcare professionals: all documents for the public are now reviewed by patient and consumers

Stakeholder and Patients at EMA: legal background

- EU Treaty (Declaration 17 of the Annex)

  "(…) Transparency of the decision-making process strengthens the democratic nature of the institutions and the public's confidence in the administration."

European Medicines Agency started to operate in 1995

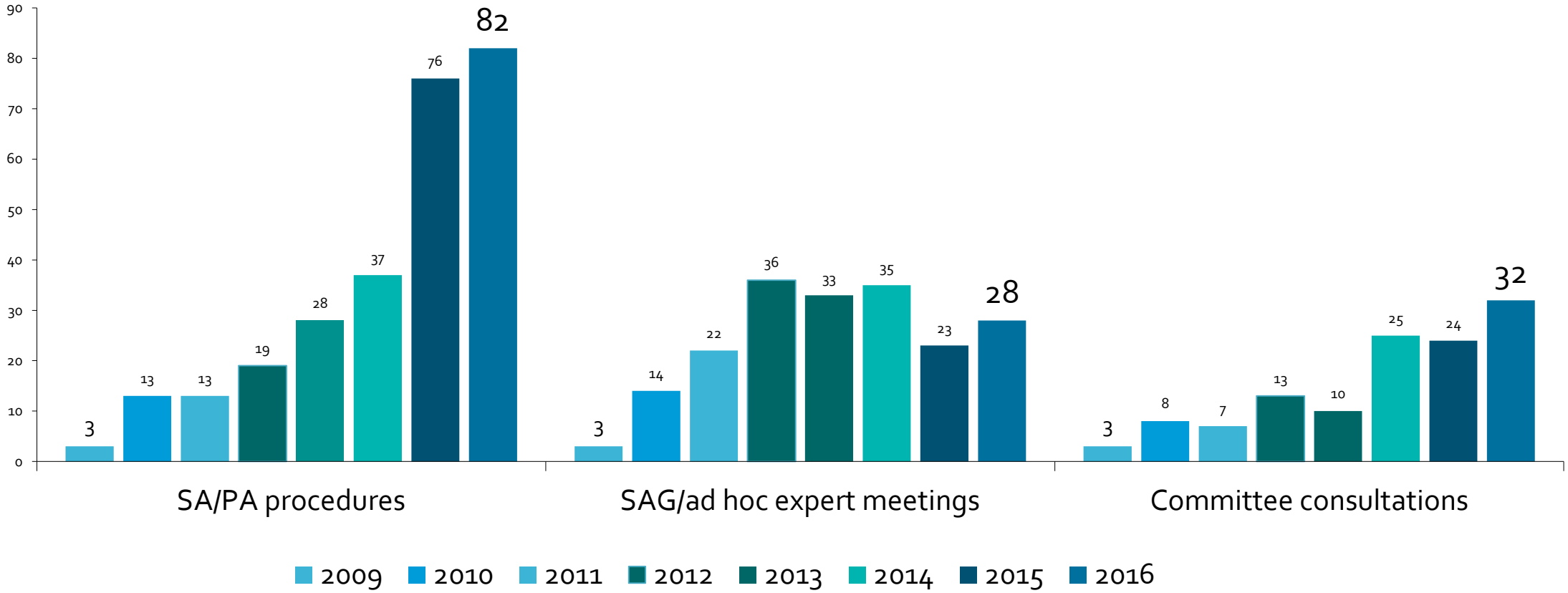
1996

- 6 patients met with the EMA

2016

- Patients and/or consumers involved in EMA on 770 occasions

Involvement of patients/consumers across different activities 2009-2016



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A framework of interaction developed with the Patients and Consumers (see more here):

Guidance and policy adopted by the EMA management board

- The role of patients as members of the EMA human scientific committees
- Pilot phase to involve patients in benefit / risk discussions at CHMP meetings

Reporting

- EMA's interaction with patients, consumers, healthcare professionals and their organisations - 2015

Rules

- Criteria to be fulfilled by patients' and consumers' organisations involved in EMA activities
- Evaluation of financial information from patients' consumers' and healthcare professionals' organisations for assessment of EMA eligibility

Training strategy

- Involvement of patient representatives in scientific advice procedures at the EMA
- Plus Summer School, EUPATI etc..

Disclaimers

- EURORDIS is one of the signatories of the "*Code of Practice between Patients' Organisations and the Healthcare Industry*"
- Read here

CODE OF PRACTICE BETWEEN PATIENTS' ORGANISATIONS[1] AND THE HEALTHCARE INDUSTRY[2]

PREAMBLE

The valuable and serious work of patients' groups and the service they provide needs to be recognised, valued and supported. However, most groups are struggling to find sufficient, diversified resources, to fulfil their mission and objectives and remain independent, whether funding comes from corporate or public sources.

Patients' organisations are keen to work in a constructive manner together with all stakeholders to ensure that the credibility of patients' groups is safeguarded.

For this reason, patient organisations (see list below) have developed the following transparent and robust Code of Good Practice to guide the relations between patient organisations and the industry (including their representatives and consultants). We encourage all patient organisations to adopt this Code when engaging in a dialogue, working partnership, joint initiative, and/or when accepting support from any funding source. We expect all signatories to adhere to this Code which may be revised over time as circumstances demand. This Code does not intend to cover every possible funding opportunity or relationship, but rather to define a set of basic principles and recommendations.

We fully appreciate and support that our European healthcare systems stand for social equity and solidarity. We maintain that access to limited resources is governed by principles of equality. In a democratic society, patients' organisations play an increasingly important role. Their work is extremely varied depending on local need, but generally can be divided into two broad categories:

- Raising awareness and advocacy about diseases and health policy issues and how to best maintain health
- Providing support for patients, their families and carers, building capacity within their membership, setting up self help/support groups and sensitising society to equitable sharing of healthcare.

Our governments in Europe are committed to protecting the health of their citizens based on social solidarity, irrespective of age, race, gender, domicile and socio-economic status. This is intended to ensure equality in healthcare and to support the laudable goal of "health for all".

Increasingly as our populations age in Europe and more and more high-tech treatment becomes available; society will be faced with difficult decisions on how finite resources are fairly allocated within healthcare systems and budgets. Patients' organisations along with other stakeholders need to be involved in those debates to ensure that policy decisions and actions are fully transparent and adopted in a consensual manner.

Many interests and stakeholders interact in our health systems. Patients' organisations have the role to ensure that the patients' voice is heard at all levels of decision making, implementation and monitoring of policies and actions that concern health and healthcare and that the existing system achieves the

[1] Patient organisations are defined as not-for profit organisations which are patient focused, and whereby patients and/or carers represent a majority of members in governing bodies.

2 The healthcare industry is defined as commercial manufacturers of healthcare products, devices and services, including distributors and wholesalers.

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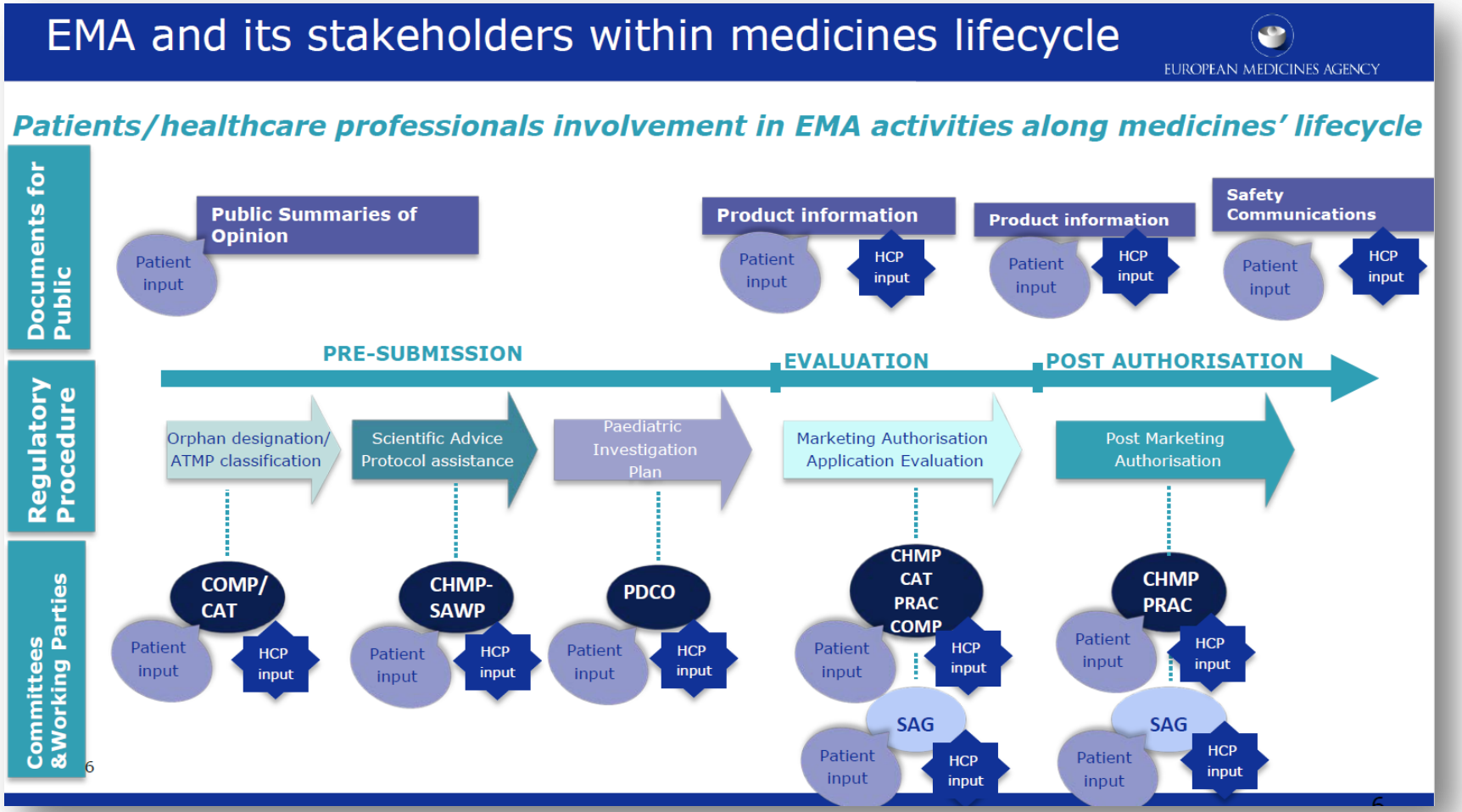
The many roles of patients and consumers at the EMA: scientific activities



PCWP consulted on the EA policy for conflicts of interest
Experts can participate to all discussions with/out restrictions (but don't vote), or as expert witnesses

Impartiality
Independence
Influence
Not just financial interests

And also 2 representatives of patients in the management board

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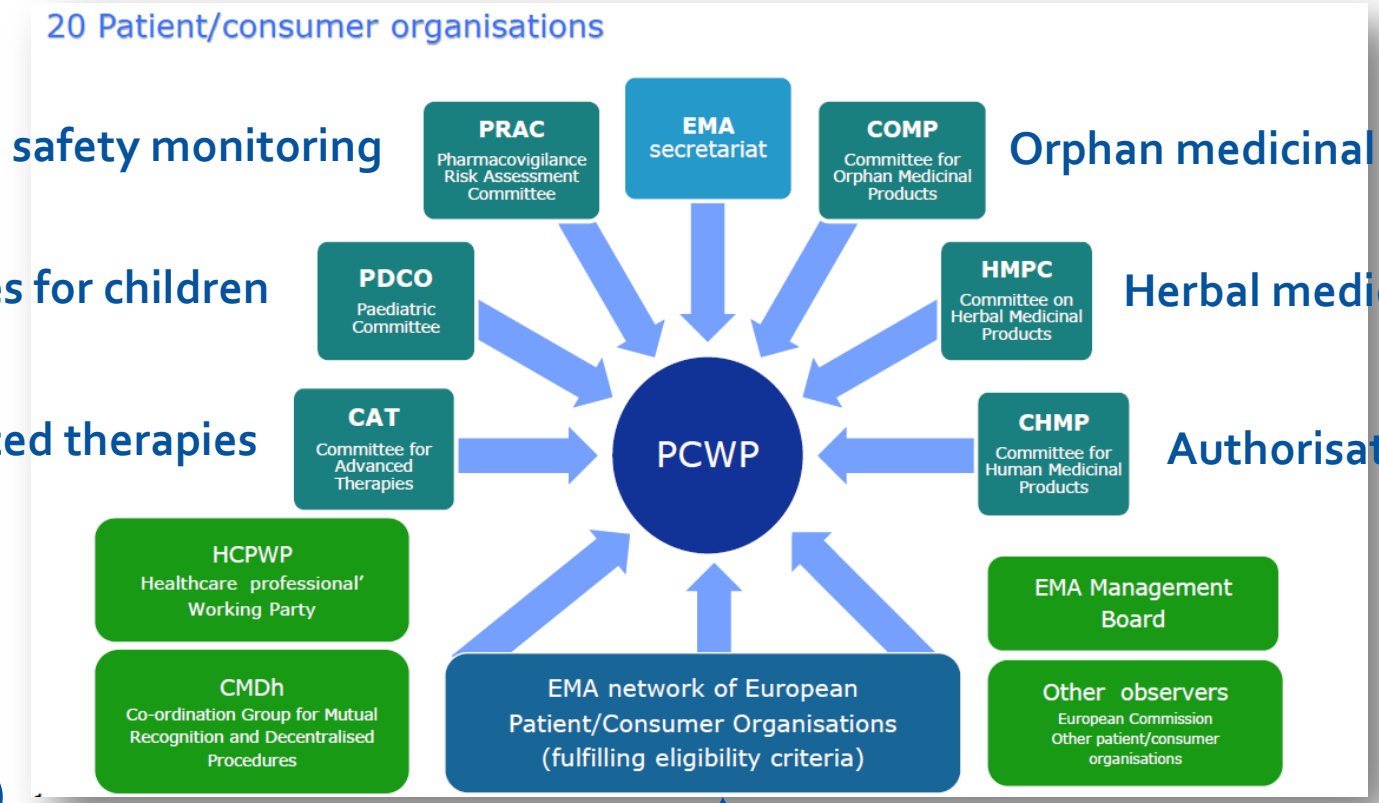
The Patients' and Consumers' Working Party
Meets 4 times a year at the EMA, more if needed

Pharmacovigilance, safety monitoring

Medicines for children

Advanced therapies



Orphan medicinal products (rare diseases)

Herbal medicinal products

Authorisation to be used in humans

Co-chairs
Juan Garcia (EMA)
Kaisa Immonen Charalambous (EPF)

37 eligible organisations

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PCWP 3 main tasks

- Transparency of the regulatory process
  - clinical trial data are now available to third parties to check the accuracy of the regulatory decisions
  - All side effects reported by the public are made public
  - All scientific committees agendas and minutes are public
  - Public hearings
- Information on medicines
  - Public Assessment Reports summaries for the public
- Pharmacovigilance and the role of patients

2016: the PCWP celebrating its 10th anniversary!

Success factors

- High quality dialogue
  - Patients are considered on the same level than other experts
- A dedicated unit at the EMA for stakeholders' involvement
  - Training materials (videos, face to face…)
  - Special needs
- Adequate resources and budget
  - All costs covered
  - Daily allowance, doubled for volunteers
- Rules for involvement defined together with stakeholders
  - For all aspects
  - Revised as often as needed

"With a high quality dialogue, patients and regulators can only agree."

Jean-Michel Alexandre
Former CHMP chair, EMA

Barriers, obstacles

- Patients' advocates are few (availability, language, disease)
- Time commitment (e.g. committee members) and time spent unpaid
- Advising the developers or advising the regulators (CofI policy)?
- Rapid regulatory timelines, and the time it takes to involve patients
- Younger patients gather on social networks, no longer in registered organisations with statutes, general assemblies, board of directors etc.
- No impact assessment when defining a new role for civil society (EU legislation making): training needs, contact database maintenance, support, guidance…

What can be improved? Next steps (among others)

- Public Hearings (September 2017)
- Updated patient information: graphic visualisation, changes in patient leaflet, videos etc.
- PRIME: Priority Medicines – Very early scientific advice, with patients, clinicians, industry, HTA, payers
- Two way communication with patients
  - Social networks
  - Mobile app for patients to report adverse drug reactions (Web-RADR)

Other stakeholders (Stakeholder involvement directly related to EMA deputy executive director / Chief Policy officer)

Borderline medicines / food: how do we do?

Oyster mushroom (Pleurotus ostreatus)

- up to **2.7% lovastatin** by **dry weight**
- 1 serving contains **5.4 mg lovastatin**

Health benefits	Maintain blood sugar, Immunity system, Cardiovascular conditions, Skin problems, Brain health

Usual Adult Dose of Lovastatin (medicine) for Hyperlipidaemia

- Initial dose: 20 mg orally/day
  Maintenance dose: **10 to 80 mg** orally/day

- 5th Congress of Myology 2016 , A case report by Pr Andrew Mammen , Johns Hopkins Hospital, Baltimore
  - A 40 y.o. male patient presents with immune necrotising myopathy
  - Seen in patients using « statin » for lowering cholesterol (1/100,000)*
  - Patient thought that symptoms were caused by a mushroom dietary supplement he took for a few months

*Mammen AL, Chung T, Christopher-Stine L, Rosen P, Rosen A, Doering KR, et al. Autoantibodies against 3-hydroxy-3-methylglutaryl-coenzyme A reductase in patients with statin-associated autoimmune myopathy. Arthritis & Rheumatism. 2011 Feb 25;63(3):713–21

Food, medicine or ?

- The true story of Augusto and Michaela Odone, two parents in a search for a cure for their son Lorenzo's adrenoleukodystrophy (ALD).
- The oil, erucic acid, is supposed to normalise the accumulation of the long chain fatty acids in the brain
- Efficacy never proven
- In some countries the oil is sold as a medicine, in others as food.
- In the US, the FDA regulates it. In the EU?

To conclude

Patients fully integrated in the centralised procedure for marketing authorisations in the EU

With equal credibility as other experts

As experts, members of scientific committees or the management board, or as representatives of their organisation

Impact of patient engagement difficult to assess. Being able to witness the process is a major achievement

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RARE DISEASES EUROPE

Thank you for your attention.

François Houÿez

Director of Treatment Information and Access

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EURORDIS the European Organisation for Rare Diseases

- Founded in 1997
- Rarity defined by prevalence (fewer than 5/10,000 citizens or 230,000 patients in the EU)
- 751 member patient organisations of which 702 in Europe
- 66 countries (28 EU Member States)
- Outreach to over 2,400 patient groups
- Over 4,000 distinct rare diseases represented
- 40+ staff members in Paris (HQ), Brussels, Barcelona, London, Geneva, Belgrade
- 130+ volunteers