7th Global Patients Congress

9-11 April 2016

International Alliance of Patients' Organizations

A global voice for patients

Selsdon Park Hotel, South London, UK ● www.globalpatientscongress.org #GPC2016

The role of patients at the EMA

Nathalie Bere
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1995 2015 EMA

An agency of the European Union

What is the European Medicines Agency (EMA)

The EMA is the EU regulatory body responsible for the scientific evaluation and supervision of medicines developed by pharmaceutical companies for use in the European Union (Human and Veterinary).

EMA: Centralised procedure

ONE

Marketing Authorisation application

Evaluation

- Authorisation in all EU MS
- Invented name
- Product information
  (Summary of Product Characteristics, Labelling, Package Leaflet)

ALL EU languages

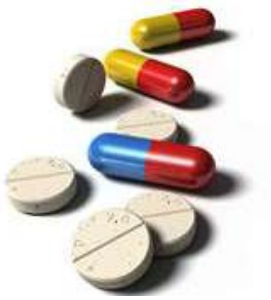
Medicines that are mandatory for evaluation at EMA

- Rare diseases
- HIV, cancer, neurodegenerative disorders, diabetes
- Auto-immune diseases, viral diseases
- All biotech products
- Gene therapy
- Monoclonal antibodies

+ Other innovative products

The EMA is **not** responsible for pricing or reimbursement

The various roles of the EMA



- Evaluation of **marketing authorisation** applications for human and veterinary medicines
- Coordination of European **pharmacovigilance** (supervision of the medicines on the market)
- Provision of **scientific advice** on the development of medicines
- Evaluation of applications for **orphan designation**
- Evaluation of **paediatric investigation plans** (or waivers)
- Evaluation of **arbitration** and **referral** procedures
- Provision of good quality and independent **information on the medicines**
- Coordination of Member States' site **inspections**

EMA-EU Network

28 EEA Member States
+ 4,500 European experts

EU institutions:
Commission - Parliament

Management Board

Committee for Human Medicinal Products (CHMP)

Committee for Veterinary Medicinal Products (CVMP)

EMA
Secretariat

Paediatric Committee (PDCO)

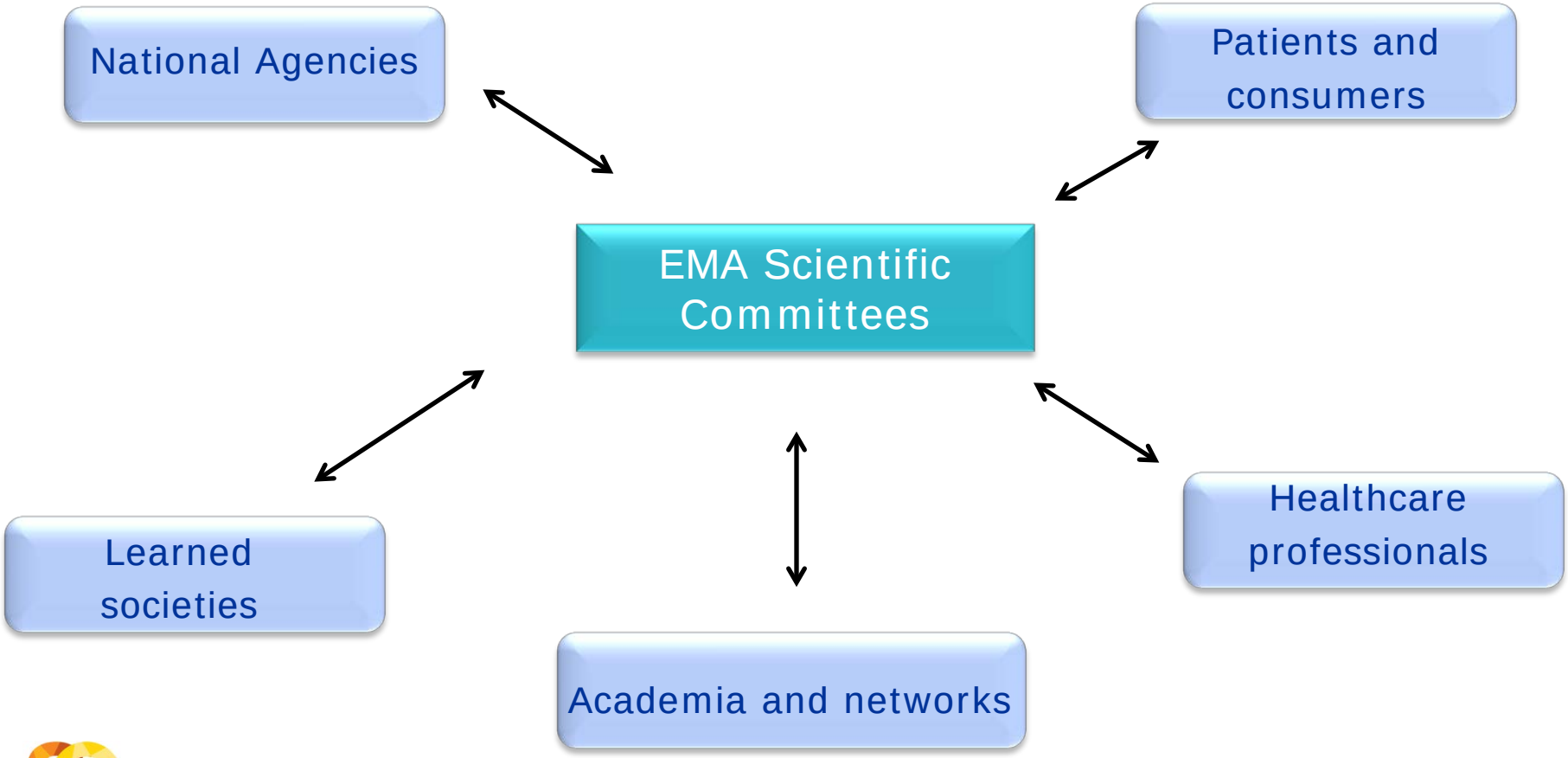
Committee for Orphan Medicinal Products (COMP)

Committee for Herbal Medicinal Products (HMPC)

Pharmacovigilance Risk Assessment Committee (PRAC)

Committee for Advanced Therapies (CAT)

Sources of expertise helping the scientific committees



Patient/consumer involvement in the EMA

Interaction with patients: the EMA journey…

1995
EMA created

1996
Dialogue with HIV patients

2000
Patients join COMP as full members

2003
Working group with patients created

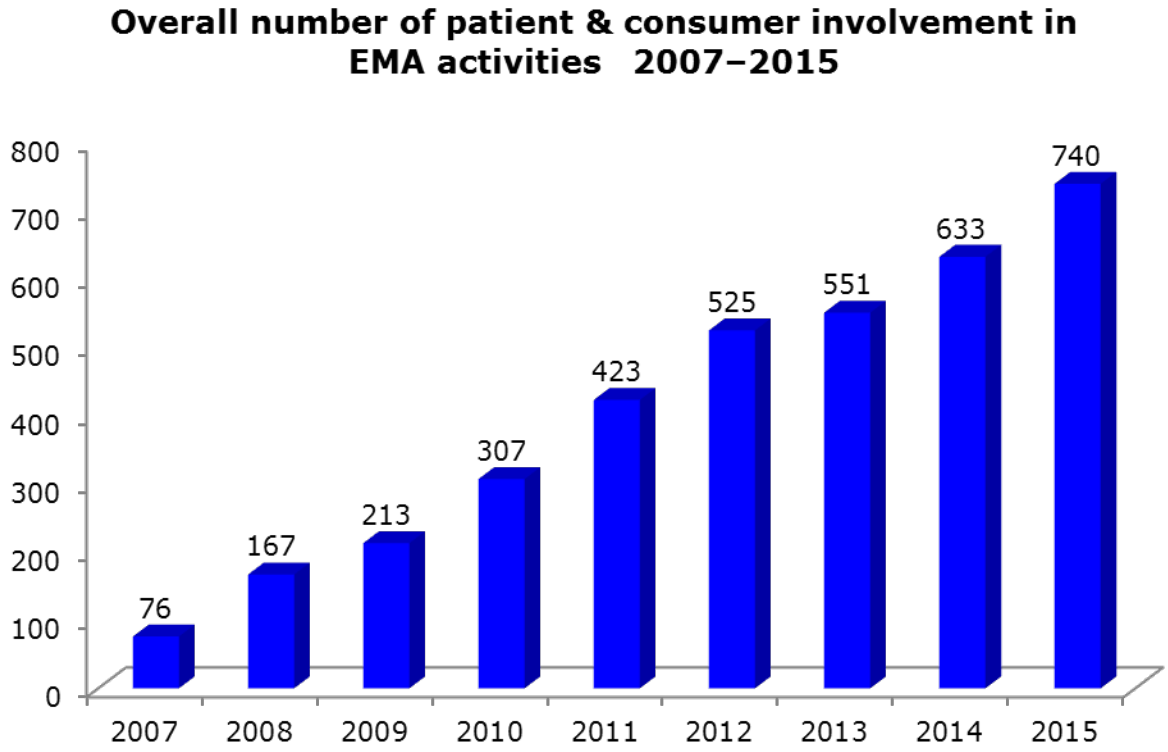
2005
Framework of interaction with patient and consumer organisations

2006
Patients and Consumers Working Party (PCWP)created

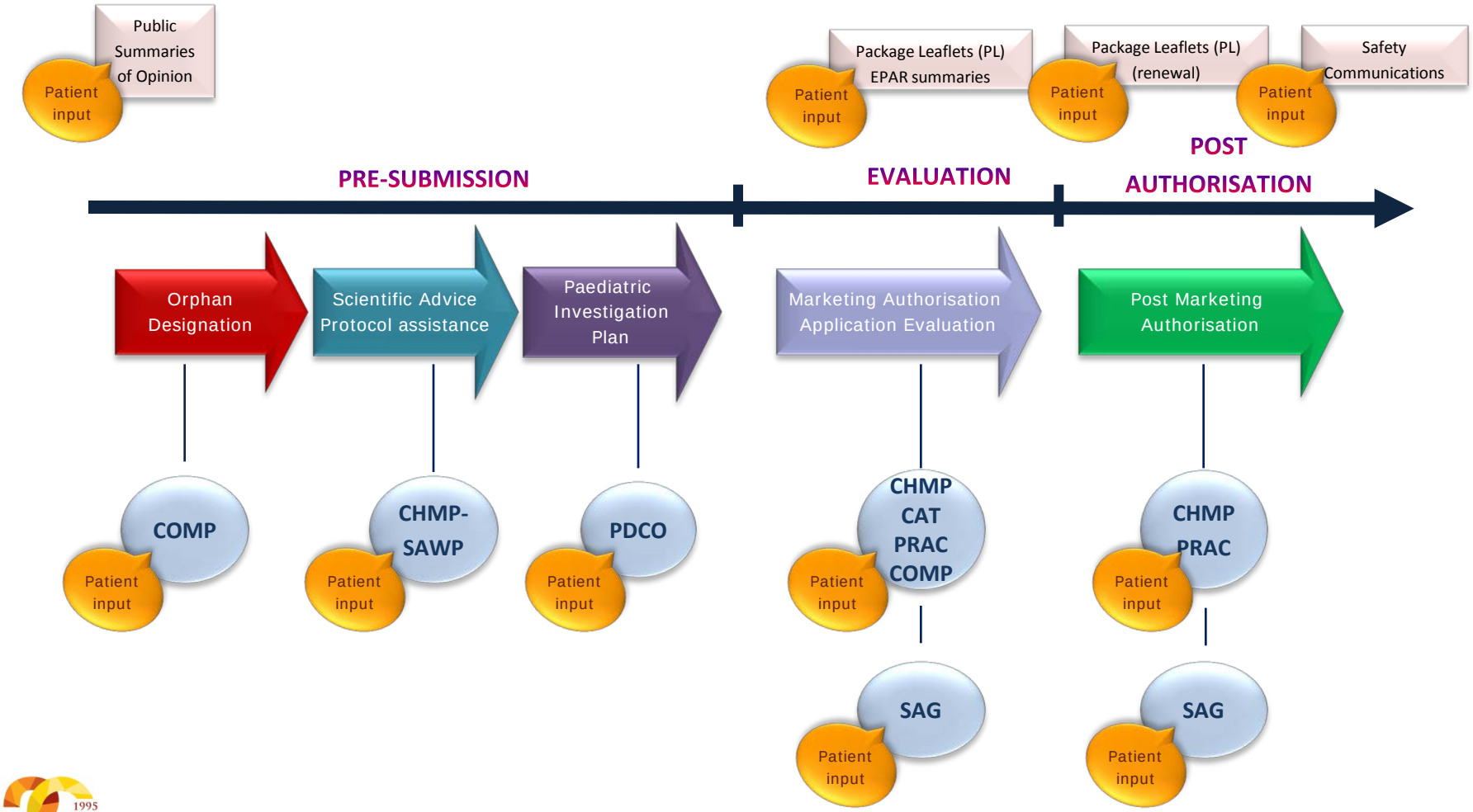
2014
- Dedicated Patients and Healthcare Professionals Department created
- Revised Framework

Ongoing…
Systematic inclusion of real life experience EMA regulatory output

Increasing involvement in EMA activities



Opportunities for Patient involvement along the medicine lifecycle at EMA



Eligible organisations: patients/consumers

EGAN Patients Network for Medical Research and Health
EURORDIS Rare Diseases Europe
European Liver Patients Association
ELPA
ENFA European Network of Fibromyalgia Associations
EHC
AGE Platform Europe
EUROPEAN CANCER PATIENT COALITION
Pain Alliance Europe
ehn
epha
SMA europe
we care, we share ICCCPO
debra Working for a life free of pain
EFA European Federation of Allergy and Airways Diseases Patients Associations
FABRY International Network
EPF European Patients Forum
MPe MYELOMA PATIENTS EUROPE
Alzheimer Europe
EFCNI european foundation for the care of newborn infants
EUROPA UOMO
IBE
International Alliance of Patients' Organizations A global voice for patients Full Member
EPDA EUROPEAN PARKINSON'S DISEASE ASSOCIATION
EGA European Gaucher Alliance Driven by passion for patients
THALASSAEMIA INTERNATIONAL FEDERATION
Insulin Dependent Diabetes Trust
EIWH
International Diabetes Federation
ELF EUROPEAN LUNG FOUNDATION
EFNA European Federation of Neurological Associations
emsp EUROPEAN MULTIPLE SCLEROSIS PLATFORM
GAMIAN EUROPE
IPOPI
EATG European AIDS Treatment Group
hai EUROPE
BEUC The European Consumer Organisation
1995 2015 EMA

Three categories of patient participation:

Patients representing *patients'* organisations

- Management Board
- EMA Scientific Committee(s)

Patients representing *their* organisations

- Patients' and Consumers' Working Party (PCWP)
- EMA consultations
- Workshops

Patients as *individual* experts

- Scientific Advice / Protocol Assistance Procedures
- Scientific expert meetings
- Scientific committee consultations
- Review of documents

EMA Patients' and Consumers' Working Party (PCWP)

- Plays a key role in the interaction between the EMA and patient organisations
- Platform for dialogue and exchange on relevant issues concerning medicines
- Representation from: Patient and Consumer organisations; EMA Scientific Committees & Management Board.
- Four meetings held annually and consulted as and when needed

Patient involvement in EMA activities (individual experts)

Medicines development:

- Participation in scientific advice/protocol assistance procedures for specific medicines

Evaluation of medicines benefit/risk: pre- and post-authorisation

- Participation in expert meetings convened by committees

- Written consultations on specific medicines from scientific committees / working parties

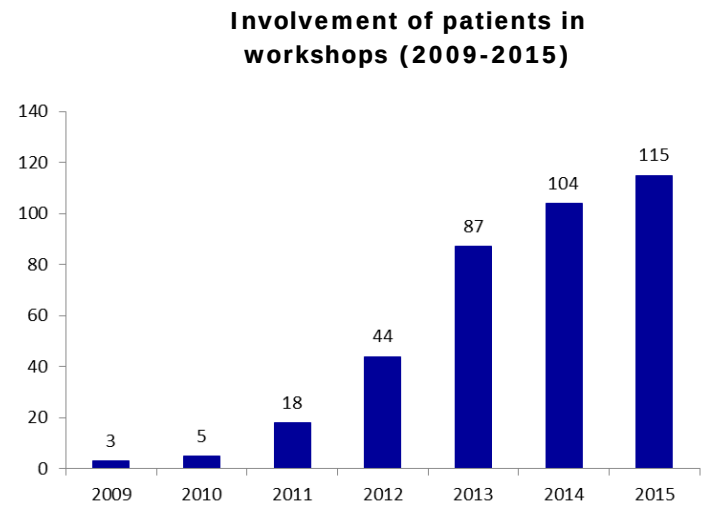
Communication on medicines:

- Review of information on medicines: Package leaflets, EPAR summaries, safety communications (Q&As) and other Agency documents for the public

Patient involvement in EMA activities (organisation reps)

Patients are consistently invited to participate in EMA **conferences and workshops**.

They are also are sent **draft guidelines** for comments

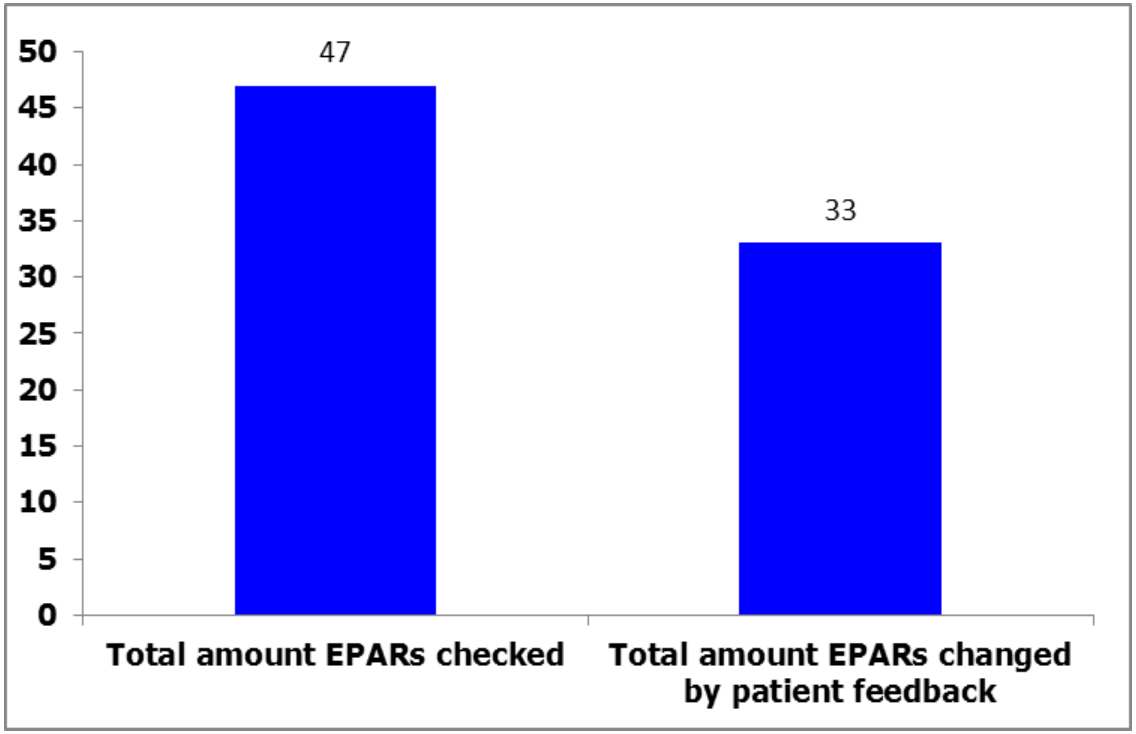


Patients also contribute to **EU-wide initiatives** where EMA is involved such as:

- Enpr-EMA - European Network of Paediatric Research at the European Medicines Agency
- ENCePP - European Network of Centres for Pharmacoepidemiology and Pharmacovigilance
- WEB-RADR - Recognising Adverse Drug Reactions

Impact of input

70% of draft EPARS reviewed were amended following patient input



Monitoring and reporting

- Annual report to EMA Management Board - overview of all activities in which patients and their organisations have been involved – *quantitative* and *qualitative* aspects
- Every 2 years a satisfaction survey is sent to all patients who have participated that year
- Proposals for improvements included in the next PCWP work-plan

Value and impact of involving patients in EMA

- The involvement of patients has;
  - Brought the everyday aspects of living with a disease into the scientific discussions
  - Improved the quality of patient information and communication on medicines
  - Increased the public awareness of EMA outcomes
- Patients are a recognised and integral part of the Agency's work
- Participation has not only increased and diversified but has also been refined to ensure optimal involvement.

This collaborative journey with many stakeholders, cultures and languages has its challenges, however different perspectives are crucial and have ultimately resulted in mutually beneficial exchanges and ultimately more meaningful decisions for all concerned.

Challenges

- Finding suitable patients (e.g. language barrier, availability)
- Ensuring comprehensive, tailored training to facilitate and enhance participation
- Provide a clear definition of patients role in the different activities to manage expectations from all angles
- Managing potential conflicts of interest
- Representation
- Measuring the value / impact of patients

EMA initiatives aimed at supporting development to accelerate patient access to medicines that address unmet medical needs.

Adaptive pathways is a concept for medicine development and data generation which allows for early and progressive access to a medicine.

Based on three principles:

- Iterative development ; approval in stages; beginning with restricted patient population then expanding; or conditional approval based on early data
- Gathering evidence through real-life use to supplement clinical trial data
- Early involvement of patients and HTA bodies in discussions on a medicine's development (brainstorm ways to optimise development pathways and accelerate access to medicines).

Primarily for treatments in areas of high medical need where difficult to collect data

PRIME (PRIority MEdicines) scheme (launched March 2016) aimed to strengthen support for medicines that target unmet medical needs and help patients benefit as early as possible from therapies that may significantly improve their quality of life.

- Offers early, proactive and enhanced support to medicine developers to optimise generation of robust data on a medicine's benefits and risks and enable accelerated assessment of medicine applications.

- Focuses on medicines that may offer major therapeutic advantage over existing treatments, or benefit patients with no treatment options.

- To be accepted for PRIME, a medicine has to show its potential to benefit patients with unmet medical needs based on early clinical data

Input from patients, completes the picture…..

Contact

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Thank you