EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

How the EMA/EU support treatment development in rare diseases

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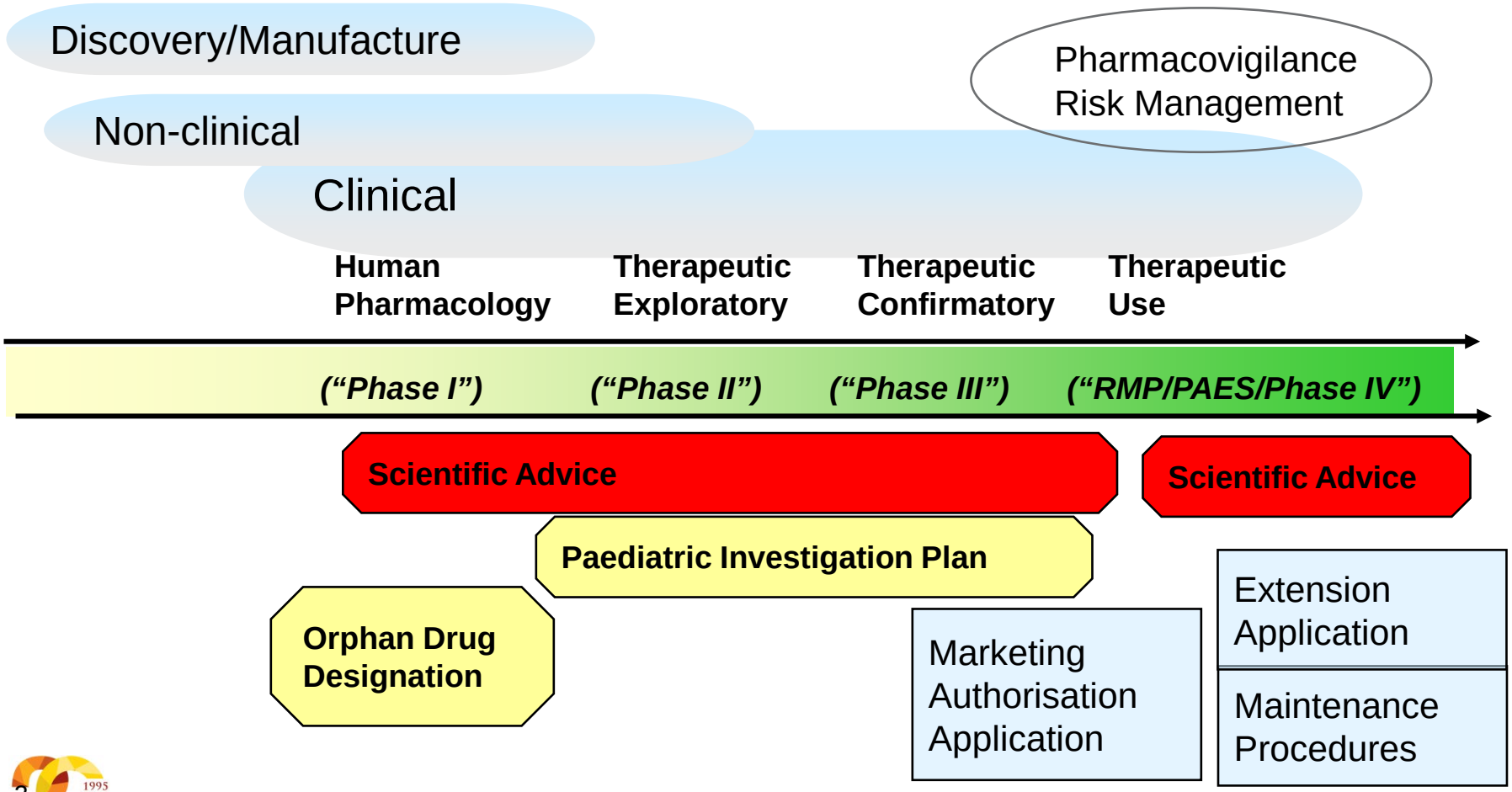
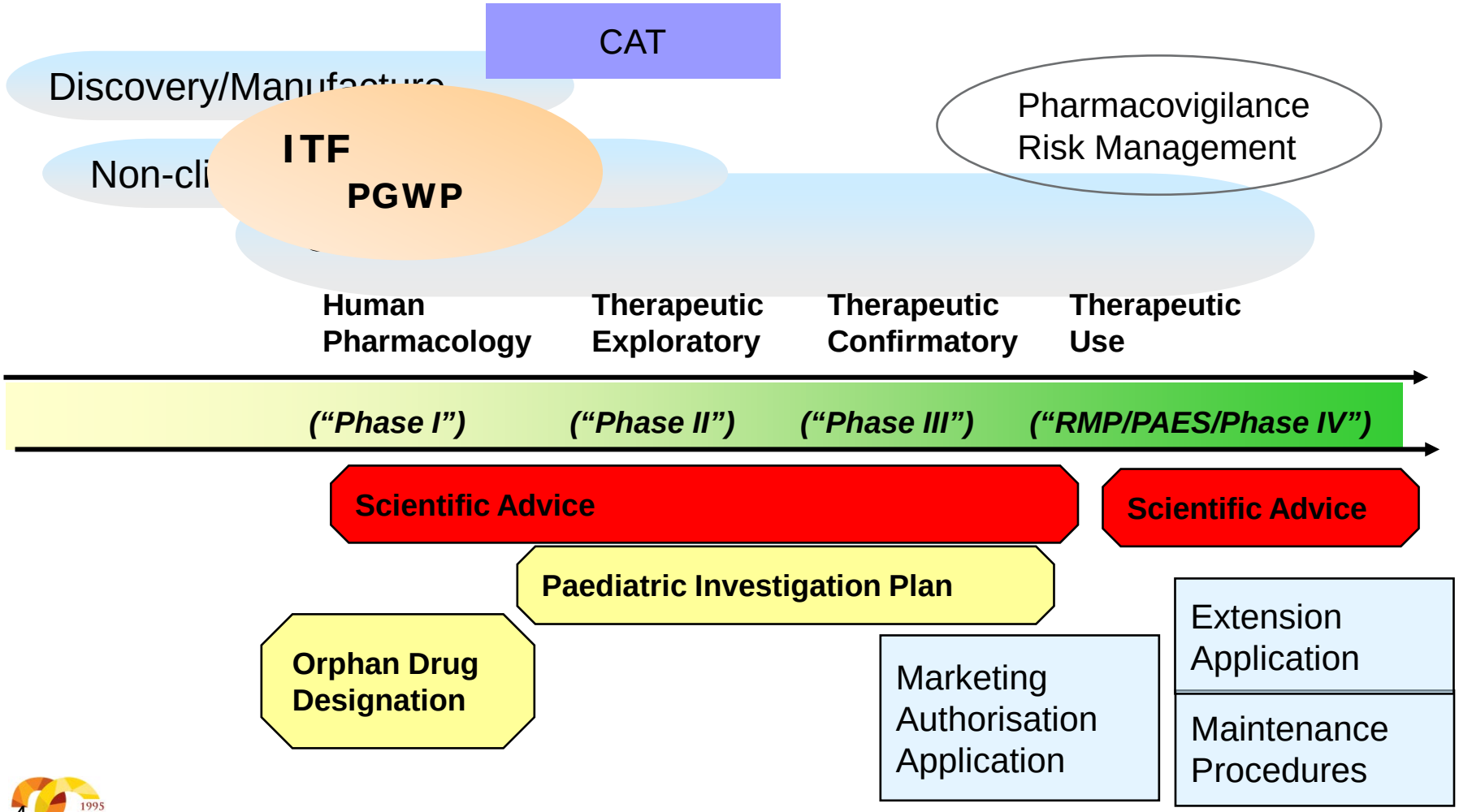
Conflict of interest: none

Drug development in the centralized regulatory system

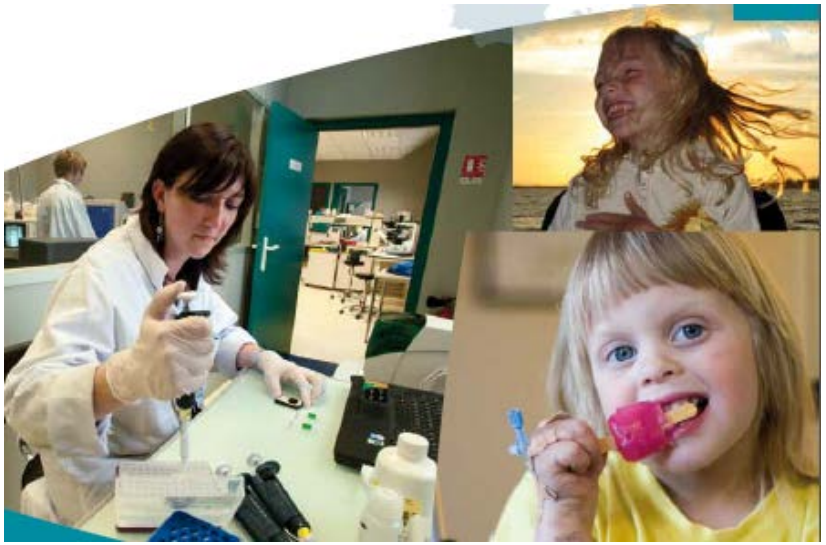


Drug development in the centralized regulatory system



European orphan legislation

- Lack of appropriate treatment – *' Patients suffering from rare conditions should be entitled to the same level of treatments as other patients'*
- Market often economically not interesting
- Lack in return of investment



- 1983 – US Orphan Drugs Act
- 1993 – Japanese legislation for Orphan Drugs
- **1999- EU 'Orphan' Reg. No 141/2000**

Aim of the regulation:

- ***Stimulate R&D of orphan products by providing appropriate incentives***

EMA awareness session April 2015, L. Liebers

Orphan incentives

- Eligible for national incentives (EC inventory)
- Protocol assistance (given by SAWP and COMP and with fee reductions)
- EU Marketing Authorisation (MAA fee reduction)
- 10-year market exclusivity protection against
  - similar products (structure/mech of action)
  - same indication

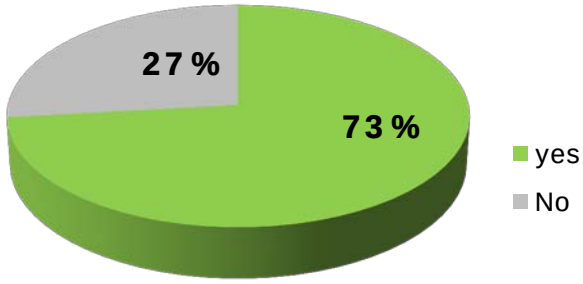
Criteria for designation

Criteria

- **Rare disease** (prevalence in EU <5/10 000)
- **Seriousness -** life threatening and/or chronically debilitating
- **No satisfactory methods** of treatment or if existing **significant benefit** to be demonstrated

Orphan designation

- Assessment by COMP
- EC decision for designation (Orphan register)
- To be granted at any stage before submission
- To be confirmed before the granting of the MAA



Products with significant benefit at MA

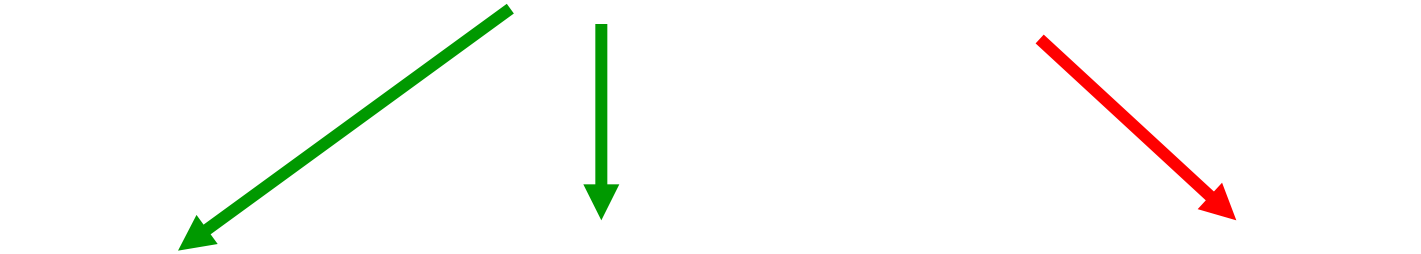
Orphan condition

- Any deviation from the normal structure or function of the body […] characteristic set of signs and symptoms, typically a recognised distinct disease or a syndrome
- Distinct pathophysiology, histology, clinical presentation
- Different severities/stages not acceptable
- Special considerations for sub-setting (distinct entity + exclusive action of the product ONLY in the subset()

- *"orphan condition" versus "therapeutic indication"*
  - *Regardless if there is a well defined mechanism of action (e.g. personalised medicine), designation must be justified for the full condition*

Significant benefit

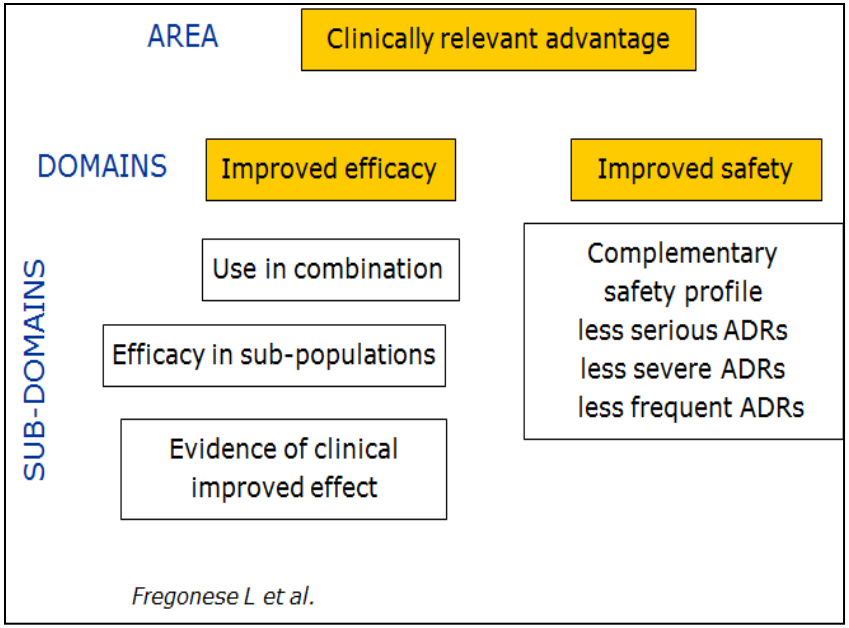
Satisfactory Methods of Treatment, Diagnosis or Prevention

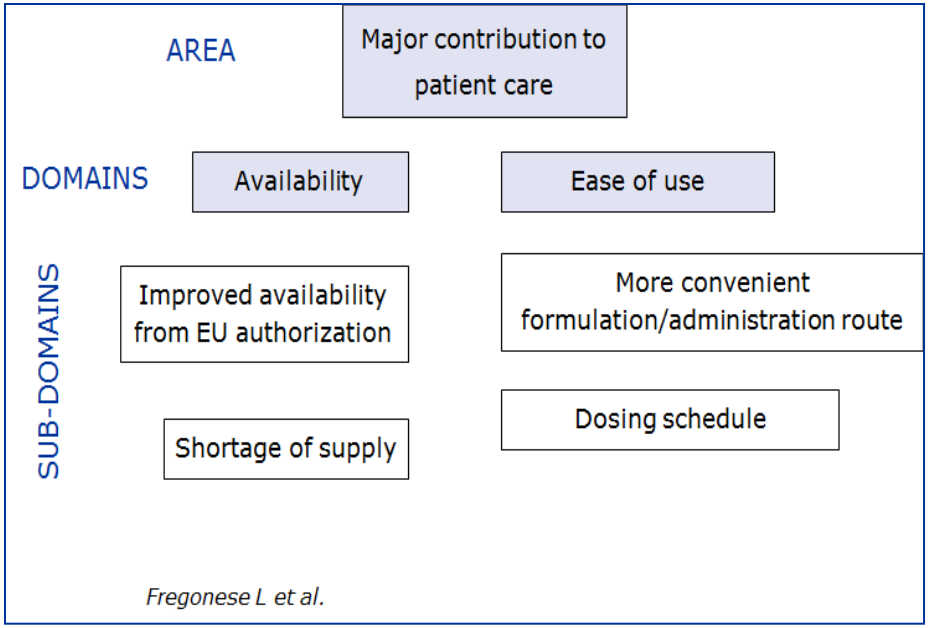


No SB needed

Need to justify SB assumptions

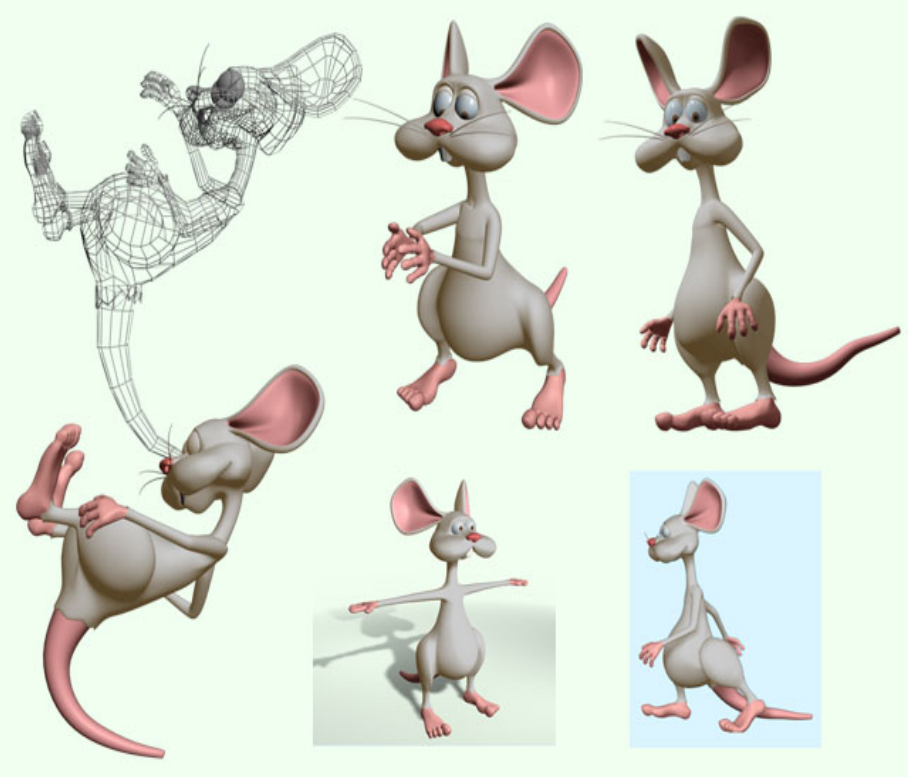
Significant benefit





Significant benefit at the time of Orphan Designation

- Based on scientifically supported assumptions and hypotheses
- Usually evidence from good pharmacodynamic animal models (e.g. transgenic animals, knock-out animals, animals carrying specific mutations, etc.)
- Sometimes based on cell cultures experiments or “proof of principle” clinical data



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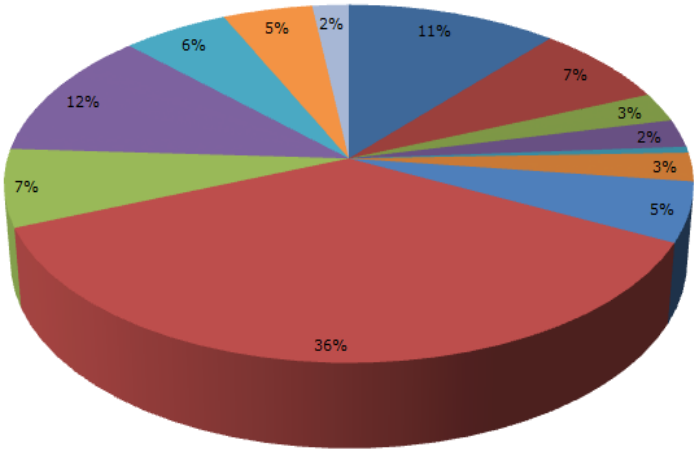
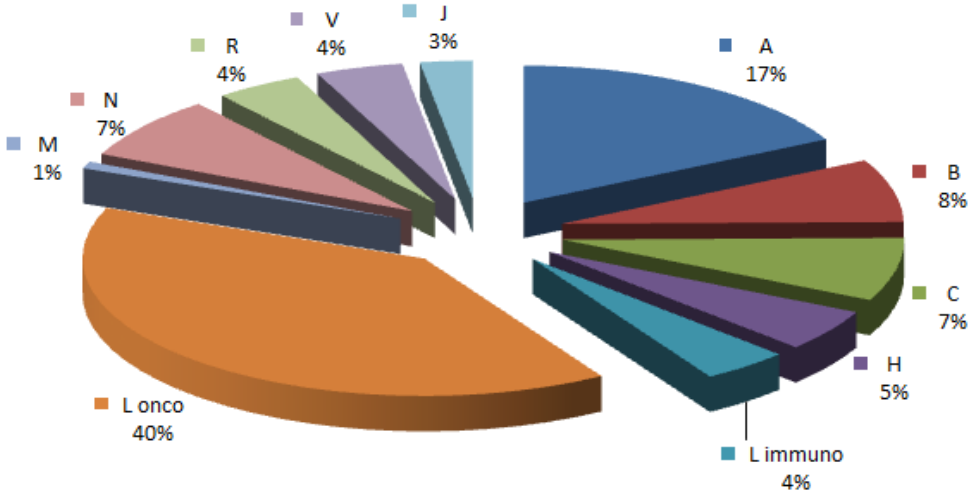
Clinical data always needed at marketing authorization

Orphan Medicinal Products (2000-2014)



Orphan designations (OD)
n=1430



Orphan Medicinal Products at MA
n=105

A Alimentary tract and metabolism; **B** Haematology; **C** Cardiovascular system; **H** Systemic hormonal; **J** Antiinfectives for systemic use ; **L** Immunology; **L** Antineoplastic; **M:** musculoskeletal**; N** Nervous system; **R** Respiratory system; **V** Various

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SCIENCE MEDICINES HEALTH

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The perspective of EMA on Registries

Strategy on Patient Registries

Laura Fregonese, Jacoline Bouvy, Xavier Kurz

European Medicines Agency (London, UK)

An agency of the European Union

Problem Statement

Registries may be requested in the context of risk management plans or other regulatory requirements

Current regulatory approach to registries suboptimal

- lack of common protocols, and data structures
- lack of data sharing and transparency
- lack of sustainability

On-going national and EU initiatives on registries not well coordinated

EMA's strategy on registries

EUROPEAN MEDICINES AGENCY

Cross-Agency Task Force (strategy paper)

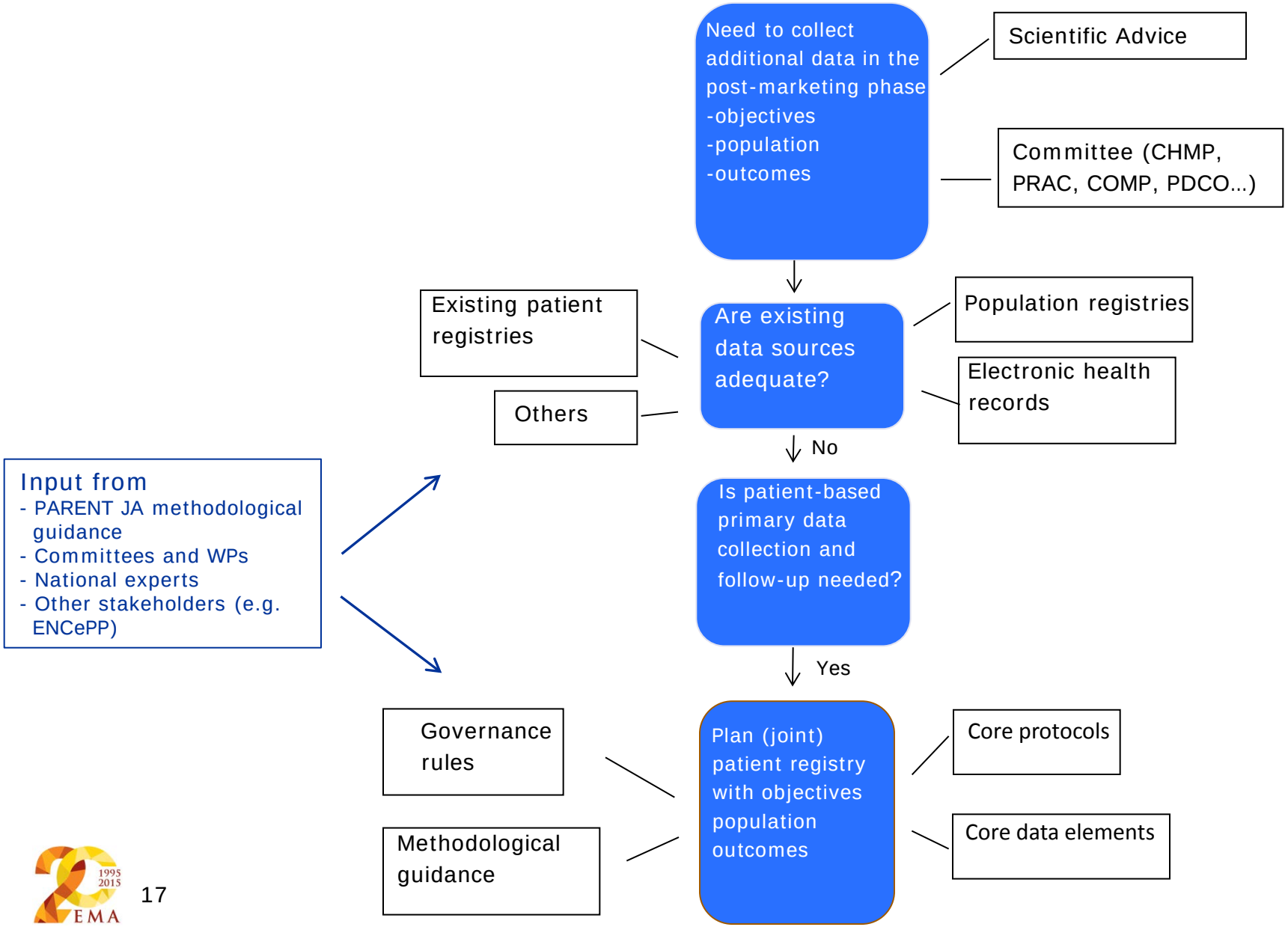
Pilot phase EU Collaborative framework

Objectives

- develop EU collaborative framework for patient registries to facilitate collection and analysis of high quality data to inform regulatory decisions and the benefit-risk profile of medicinal products

- test feasibility of integrating registries in the existing regulatory procedures (e.g. adaptive licensing pilot, joint discussions regulators and HTA bodies/payers)

Main components of strategy



Thank you for your attention

Further information

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