EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Building on 15 years of Orphan Legislation: current and future approaches

EMA- EuropaBio Information Day
Orphan Medicinal Products
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Changes in 15 years of the Orphan Regulation

Stakeholders & Development of Orphan Drugs in the EU

2000

Patients: few drugs for rare conditions

Industry: focus on non-orphans with big pharma & blockbusters

Health care professionals / Academia: not involved

Regulators: at least 28 different procedures for MA

07/2015

Patients: 105 'active' OD, > 1500 products designated

Industry: develops an interest, SMEs and Academia involvement

Health care professionals / Academia: Sponsors of designations / some are MAH

Regulators: 1 procedure – centralised

Orphan Regulation in the EU

- Regulation (EC) No 141/2000 of the European Parliament and of the Council on Orphan Medicinal Products of 16 December 1999
  - Criteria for designation
  - Committee (COMP)
  - Procedure
  - Incentives (market exclusivity)
- Commission Regulation (EC) No 847/2000 of 27 April 2000

  laying down the provisions for implementation of the criteria for designation of a medicinal product as an orphan medicinal product and definitions of the concepts ‘similar medicinal product’ and ‘clinical superiority’
- Commission communication July 2003 (2003/C 178/02)

  Under revision

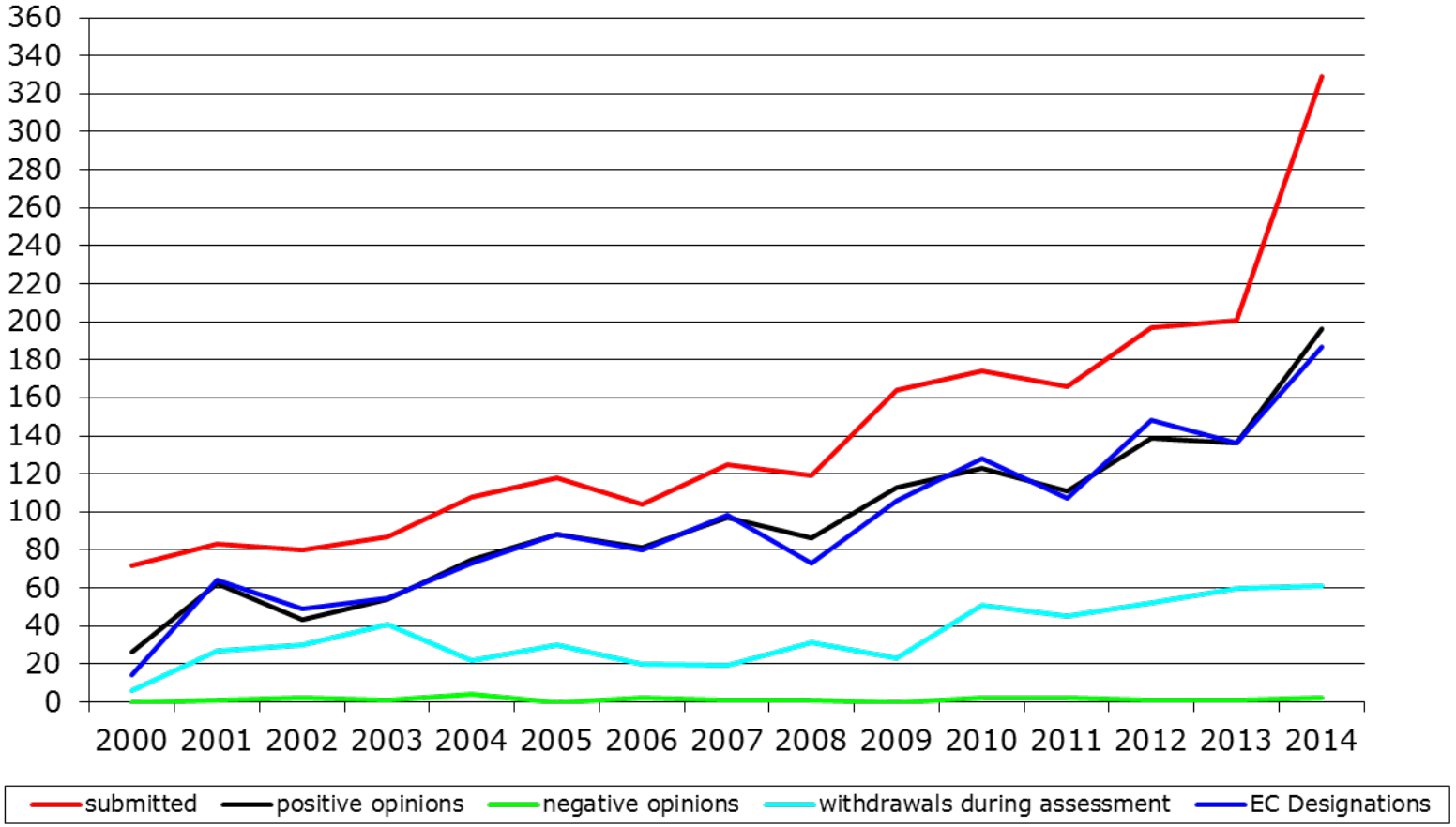
OD Regulation

Incentivises development and authorisation of products for

- diagnosis, prevention or treatment
- up to 5/10,000
- serious condition
- [significant benefit]

By development support (PA) and protection of authorised against direct competition (ME)

OD Applications



COMP review prior to authorisation (2000-2014)

100 COMP Positive opinions on maintenance

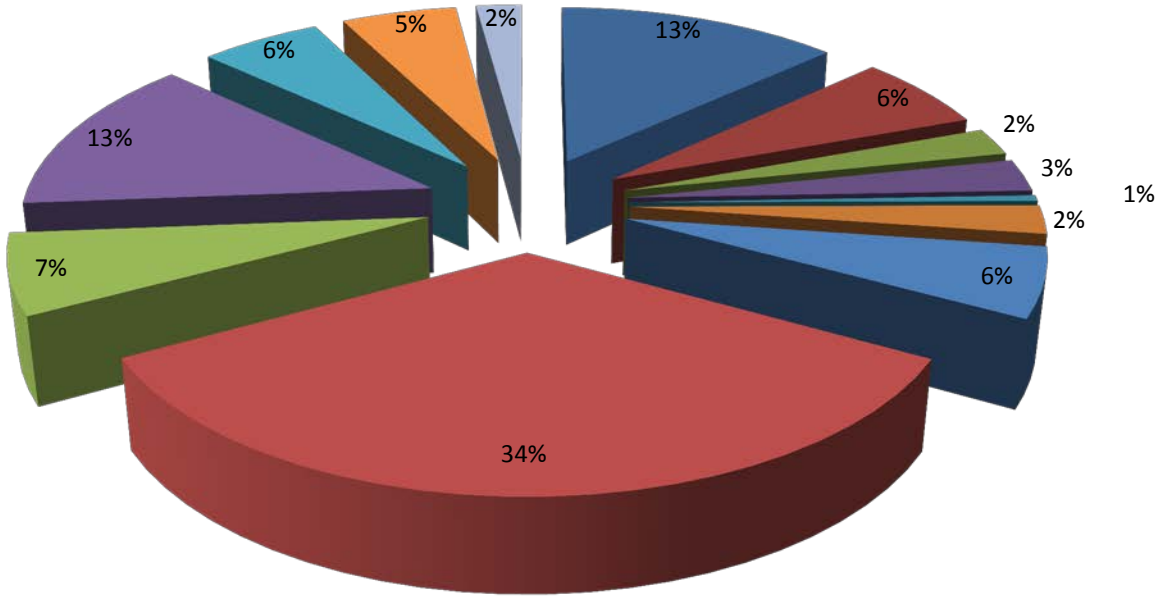
- maintenance of criteria justified

11 COMP Negative outcomes

- mainly issues with significant benefit

Active Designations by therapeutic area

(9/2015)



Double trouble

- Scientific challenges : limited information, no models, no experts, small number of patients
- Regulatory challenges: identify plausible candidates, develop product and authorise

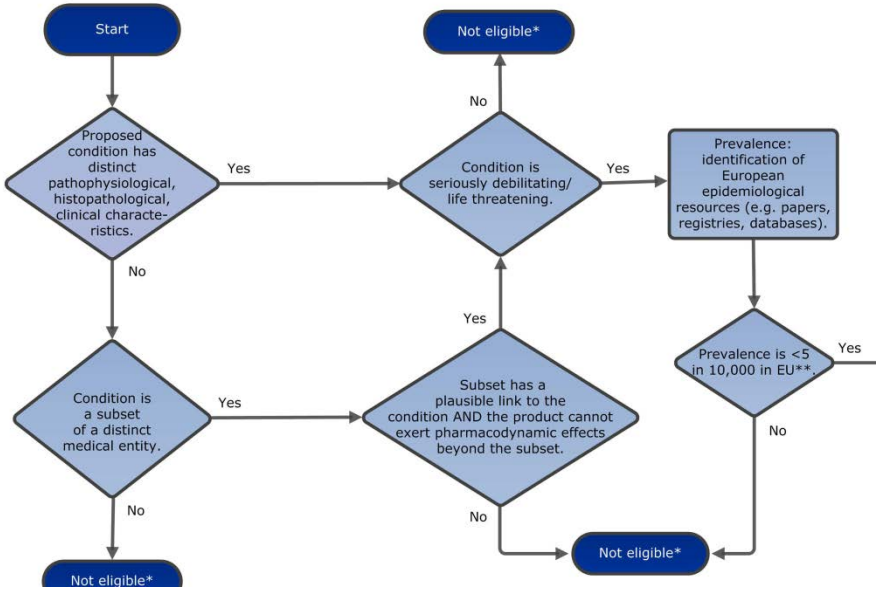
ensure successful, consistent, fair, stable, predictable environment

What can we do?

- Transparency
- Harmonization
- Regulatory docs
- Publications
- Conferences
- Surveys
- Presubmission
- Guidance

Ready to submit? (1)

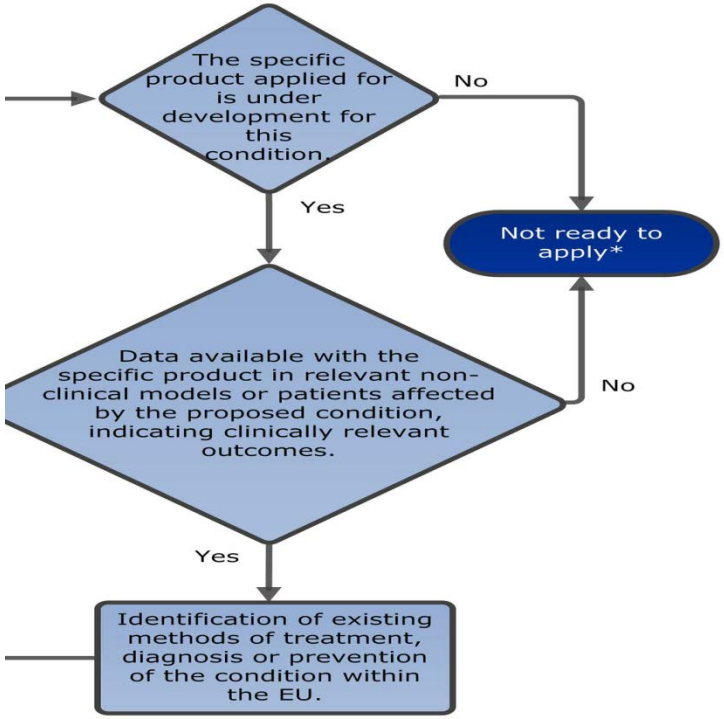
- Find a **DISTINCT entity**
  - etiology, pathophysiology, histopathology, clinical characteristics, classification
  - **rare and serious**
- Not patients with common manifestations of several underlying diseases
- Not subsets of broader conditions



http://www.ema.europa.eu/docs/en_GB/document_library/Other/2015/08/WC500191754.pdf

Ready to submit? (2)

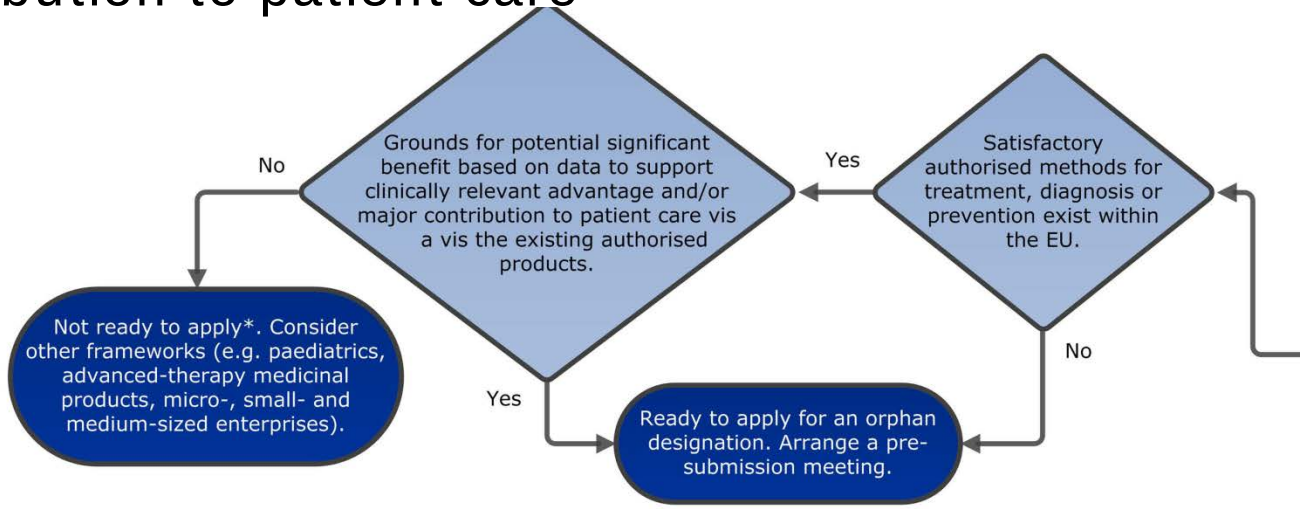
- Have proof of concept **DATA** with the active in context of the specific condition
- Not rationale without data
- Not data on other products
- Not data on other conditions



http://www.ema.europa.eu/docs/en_GB/document_library/Other/2015/08/WC500191754.pdf

Ready to submit? (3)

- **DATA** to support assumption of significant benefit
- Discuss your orphan product **vis a vis** what is already authorised to show improved effects, add-on effects, targeting different aspects or populations, major contribution to patient care



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Telltales of achievement

- Authorised orphans
- Stakeholders
- Frameworks
- Clustering around new orphan indications
- Rare conditions becoming frequent!

Thank you

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