



EUROPEAN MEDICINES AGENCY
SCIENCE · MEDICINES · HEALTH

Regulatory challenges in rare cancers drugs development

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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**
 - criteria for designation of a medicinal product as an orphan medicinal product
 - definitions of the concepts 'similar medicinal product' and 'clinical superiority'
- ❑ **Commission notice on the application of Articles 3, 5 and 7 of Regulation (EC) No 141/2000 on orphan medicinal products**



Designation criteria

- **Rarity (prevalence)**

Medical condition affecting not more than 5 in 10,000 in the Community (around 250,000 people)

- **Seriousness**

Life –threatening or chronically debilitating

- **Alternative methods authorised**

No satisfactory methods of treatment or if existing significant benefit to be demonstrated



Committee for Orphan Medicinal Products (COMP)

1 Elected Chair

1 Vice chair (Patient Rep)

1 Representative per Member State (28)

3 Patients' Representatives appointed by European Commission

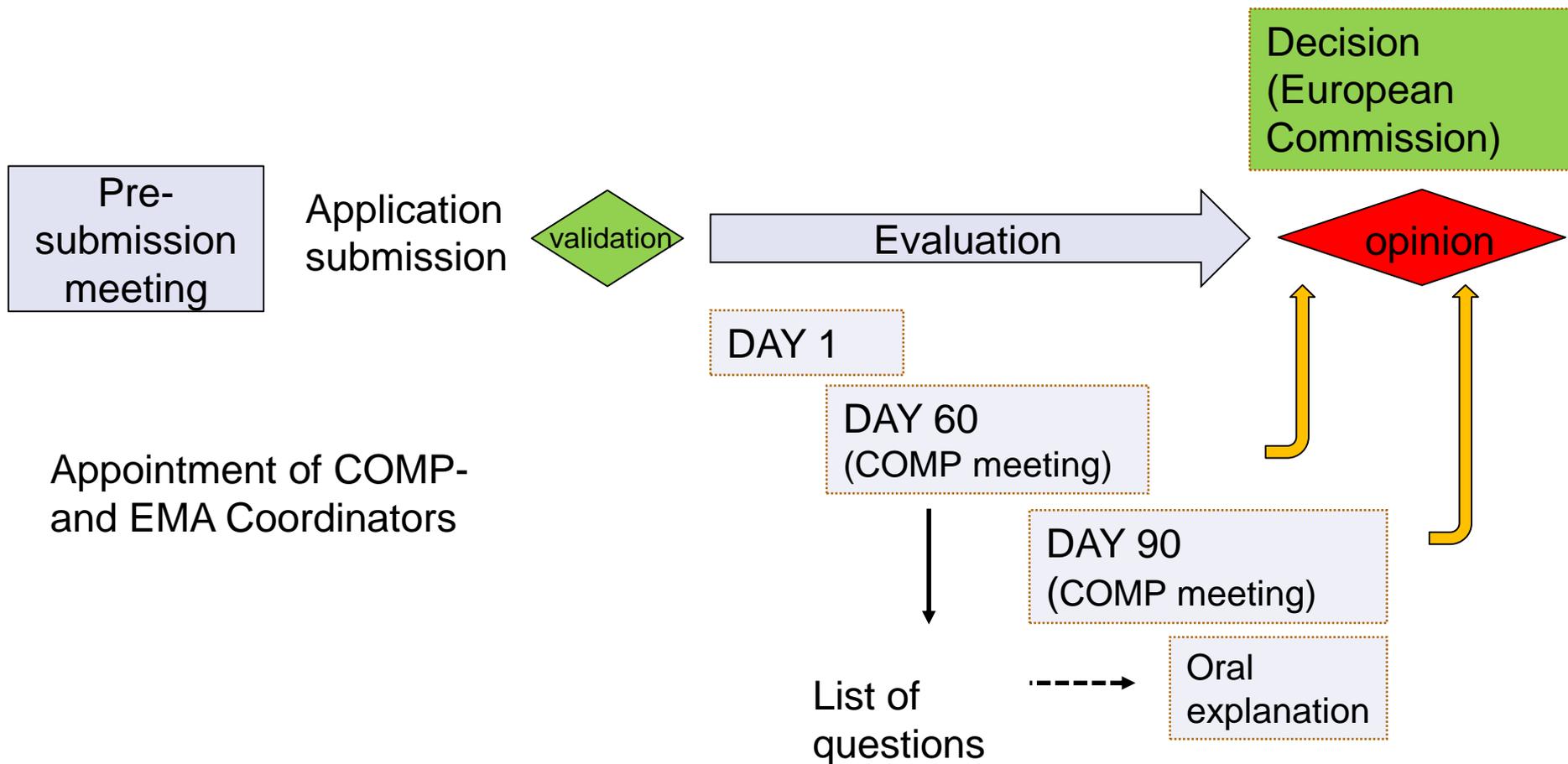
3 Members appointed by European Commission on proposal from Agency

1 Member for Norway, and 1 for Iceland





The designation process in the EU





Incentives

Protocol
assistance/Scientific
Advice

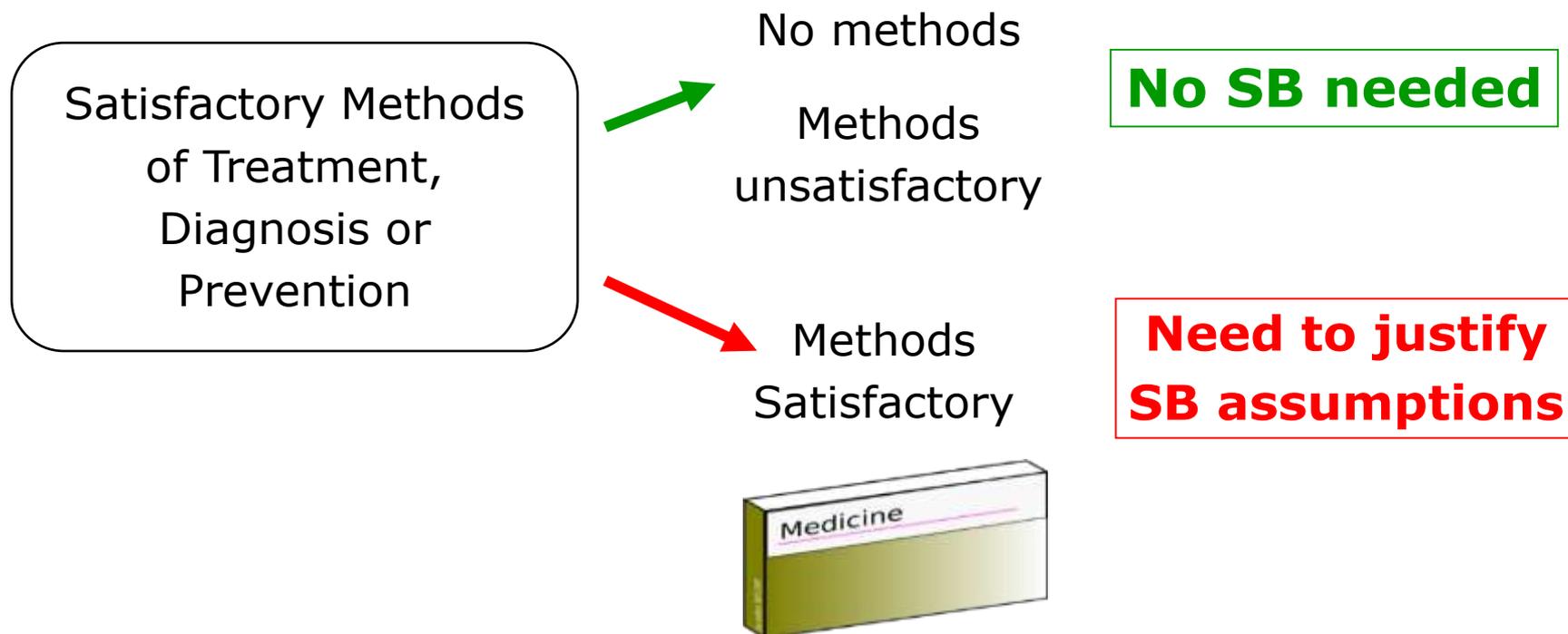
Market exclusivity

Fee reductions

Extended incentives
for SMEs

Access to the
centralised
authorisation
procedure

Significant benefit





Significant benefit

- Unique to the European Orphan Regulation
- Defined as:
 - **a clinically relevant advantage**
 - **a major contribution to patient care**



Clinically relevant advantage

- improved efficacy for the entire population suffering from the condition or a particular population subset or a subset that is resistant to the existing treatments

or

- a better safety profile or a better tolerability for the entire population suffering from the condition or for a particular subset



Major contribution to patient care

- ease of self-administration
- significantly improved adherence to treatment due to a change in pharmaceutical form

Theoretical examples

Pills vs. injection (but not 3 pills a day vs 1 injection per month)

Ready to inject vs need to reconstitute (sterile)

Easy to carry (e.g. not requiring storage in the fridge)



Significant benefit

Should not be based on:

- possible increased supply/availability due to shortages of existing authorised products or to existing products being authorised in only one or a limited number of Member States (except evidence of patient harm)
- a new pharmaceutical form, a new strength or a new route of administration unless it brings major contribution to patient care
- an alternative mechanism of action *per se*



Orphan Similarity

- Paragraph 3 of Article 8 of EC Regulation 141/2000 establishes the basis for Orphan Medicinal Similarity.
- Orphan Similarity occurs when a product is submitting for an indication where there is already one or more Orphan Designated products authorised which have an active 10yr Market Exclusivity (not expired).
- Orphan Similarity involves three similarity tests:
Structure, mode of action and therapeutic indication.



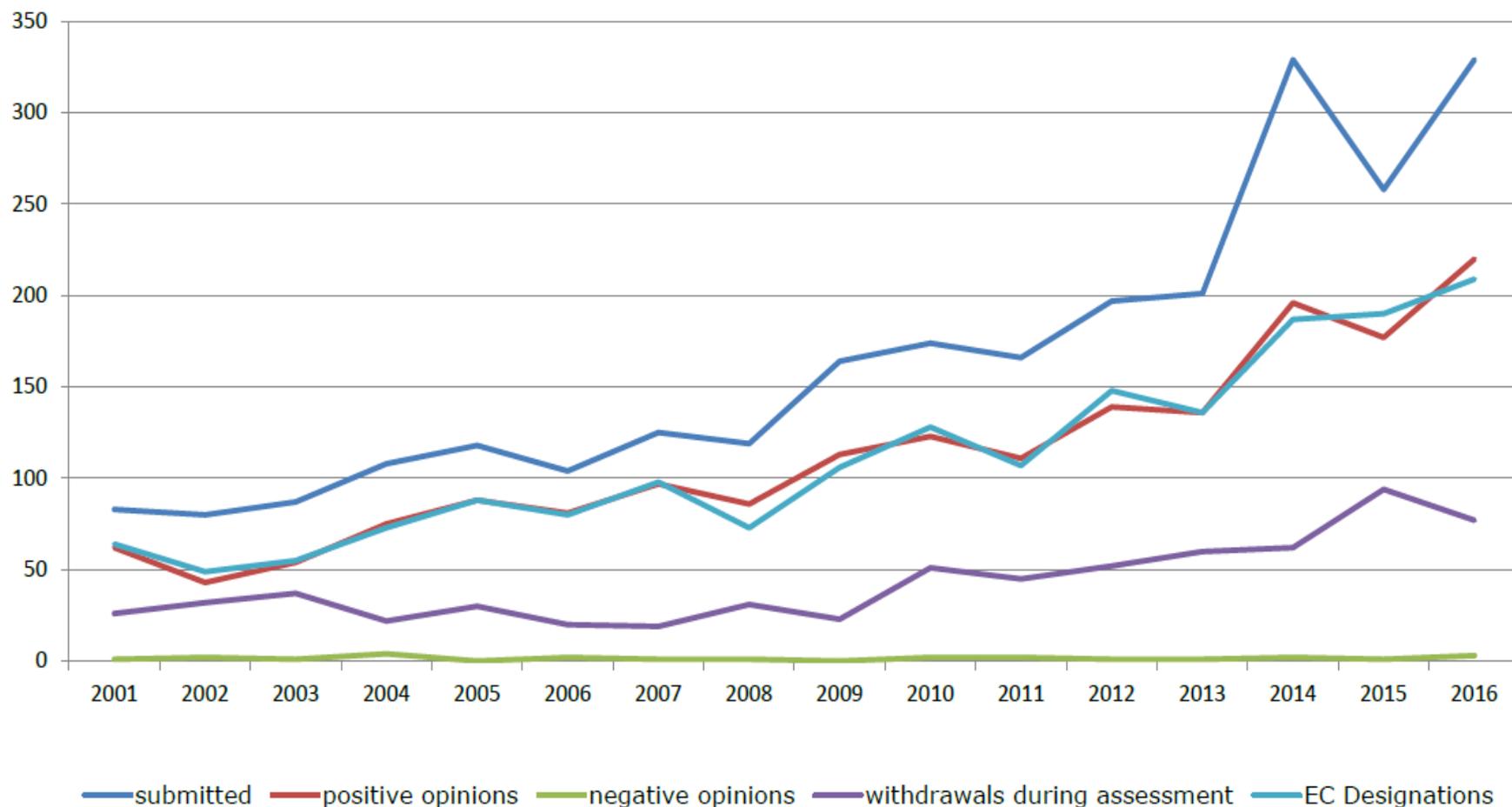
Derogations to market exclusivity if Orphan Similarity applies

Three derogations (Art 8(2))

- First MAH's consent (agreement market sharing)
- Insufficient supply: long term and clinical consequences (presumably)
- Clinical superiority: better efficacy, better safety or exceptionally major contribution to patient care



Status of orphan applications





PRIME was launched in March 2016

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PRIME: priority medicines

PRIME - PRIORITY MEDICINES

PRIME is a scheme launched by the European Medicines Agency (EMA) to enhance support for the development of medicines that target an unmet medical need. This voluntary scheme is based on enhanced interaction and early dialogue with developers of promising medicines, to optimise development plans and speed up evaluation so these medicines can reach patients earlier.

Through PRIME, the Agency offers early and proactive support to medicine developers to optimise the generation of robust data on a medicine's benefits and risks and enable accelerated assessment of medicines applications.

This will help patients to benefit as early as possible from therapies that may significantly improve their quality of life.

Accelerated assessment

PRIME builds on the existing regulatory framework and tools already available such as scientific advice and accelerated assessment. This means that developers of a medicine that benefitted from PRIME can expect to be eligible for accelerated assessment at the time of application for a marketing authorisation.

Fostering early dialogue

Related documents

- Enhanced early dialogue to facilitate accelerated assessment of Priority Medicines (PRIME) (07/03/2016)

PRIME - PRIORITY MEDICINES

Paving the way for promising medicines for patients

Why PRIME is needed

Many patients will continue to live with or die of debilitating chronic diseases and will be unable to benefit from scientific advancement and existing drugs unless new medicines are available.

The European Medicines Agency (EMA) develops PRIME in line with the European Commission's priorities and the overall strategy to 2020 for the European Union's regulatory network. The goal is to foster research and development of medicines for patients whose disease cannot be treated or who have better treatment options to help them live better lives.

Benefits of PRIME

FOR PATIENTS

- PRIME offers medicines that address an unmet medical need. As the major therapeutic challenge was solving medicines in small patient sets to current treatment options for their disease.
- Helps to translate research in the development of medicines into meeting regulatory requirements.
- Aims to bring promising treatments to patients earlier, without compromising high production standards and safety.

FOR MEDICINE DEVELOPERS

- PRIME helps developers of promising new medicines to optimise development plans.
- Offers early dialogue with EMA to facilitate robust data collection and high quality marketing authorisation applications.
- Needs to evaluate whether medicines can meet patients' needs, reduce or avoid new risks to make a difference to patients' lives.
- Encourages developers to focus resources on real patients' needs.

PRIME in brief

Medicine developers for PRIME medicines are given a special medical need.

Medicine developers can be eligible through the scheme and given guidance to capabilities in preparing their requests.

PRIME will provide early and enhanced support to enhance the development of digital medicines, speed up their evaluation and facilitate access to patients.

Factsheet in lay language

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

7 August 2016
EMA/135/2016/EN
Human Medicines Research and Development Support Division

European Medicines Agency Guidance for applicants seeking access to PRIME scheme

This guidance document addresses questions that applicants seeking support through the PRIME scheme may have.

This guidance also explains the design and features of PRIME. It provides an overview of the procedure to obtain support through the scheme and gives guidance to capabilities in preparing their requests. This guidance will be updated regularly to reflect new developments as a process is gained with the scheme.

To obtain more detailed information on:

- [PRIME scheme](#)
- [Eligibility criteria for the scheme](#)
- [Application process](#)
- [Support available to applicants](#)
- [Access to the scheme](#)
- [Data protection and confidentiality](#)
- [Contact information](#)

If you require further information on any of the related topics, do not hesitate to send your request to prime@ema.europa.eu, and we will deal with your query in a timely manner.

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Q&A, templates, application form for applicants



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Thank you for your attention!

Further information

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