



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

PRIME: where are we in May 2018

Products, diseases, interactions with Health and Technology Assessment bodies
and submission of MA for products benefiting from PRIME

ECRD meeting, May 2018

Presented by Zahra Hanaizi
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An agency of the European Union





PRIME scheme - Goal & Scope

To foster the development of ***medicines with major public health interest.*** •



Reinforce scientific and regulatory advice

- Foster and facilitate early interaction
- Raise awareness of requirements earlier in development



Optimise development for robust data generation

- Focus efficient development
- Promote generation of robust and high quality data



Enable accelerated assessment

- Promote generation of high quality data
- Facilitated by knowledge gained throughout development

Building on
existing
framework;

Eligibility
according to
existing
'Accelerated
Assessment
criteria'

Eligibility to PRIME scheme

Based on Accelerated Assessment criteria



Medicinal products of major public health interest and in particular from the viewpoint of therapeutic innovation.

- Potential to address to a significant extent **an unmet medical need**
- Scientific justification, based on data and evidence available from nonclinical and clinical development

No satisfactory method or if method exists, bring a major therapeutic advantage

Introducing new methods or improving existing ones

Meaningful improvement of efficacy (impact on onset, duration, improving morbidity, mortality)

Justification for eligibility to PRIME

For products under development yet to be placed on the EU market



Unmet medical need

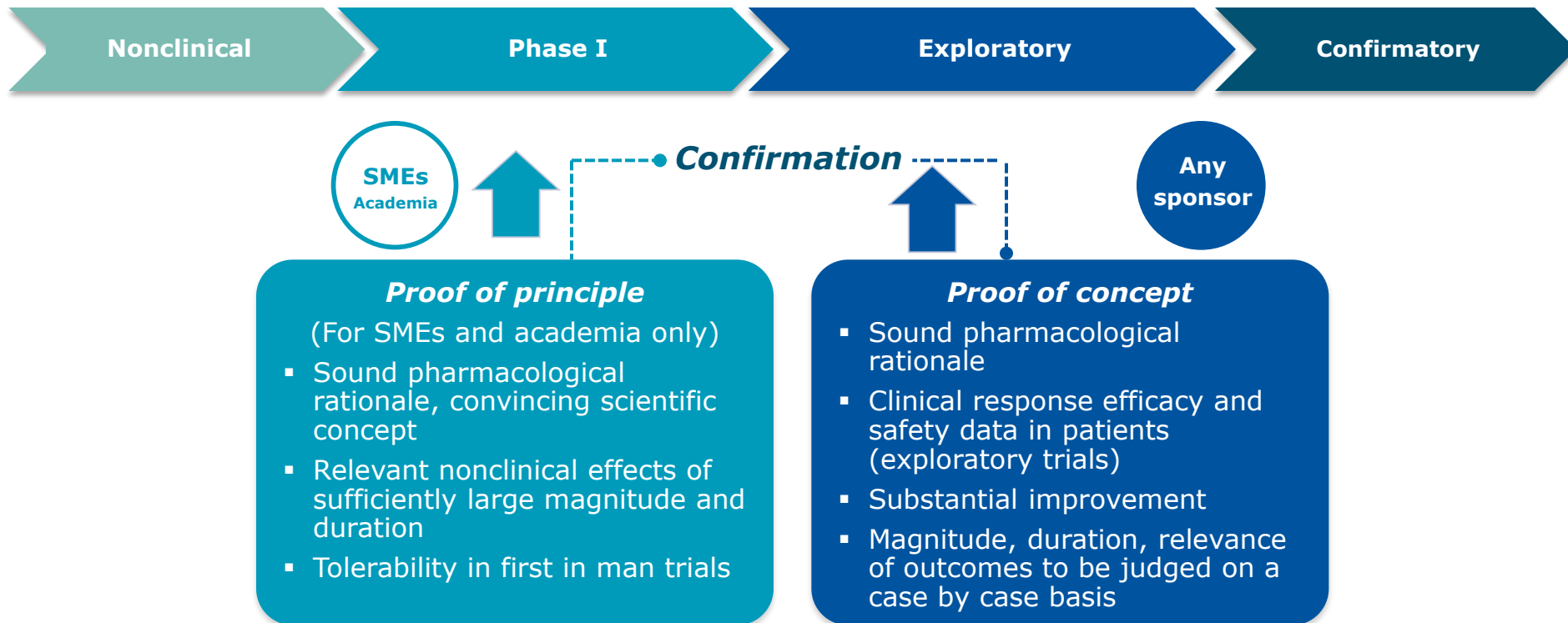
- Epidemiological data about the disease
- Description of available diagnostic, prevention and treatment options/standard of care (SOC), their effect and how medical need is not fulfilled

Potential to significantly address the unmet medical need

- Description of observed and predicted effects, clinical relevance, added value and impact
- If applicable, expected improvement over existing treatments

Data required at different stages of development

Entry points PRIME eligibility and required evidence



Assessment of eligibility requests: 40-day procedure



**Short, lean process, involving multiple committees
for robust assessment**

What do we expect to grant eligibility?



Unmet medical need

No treatment or clear limitations of existing therapies

Nonclinical data supporting pharmacological rationale (e.g. gene therapy)

Promising clinical exploratory data on **relevant endpoint**

If uncontrolled, use **comparable historical control** i.e. need sufficient information on baseline characteristics

Magnitude of the effect supporting major therapeutic advantage



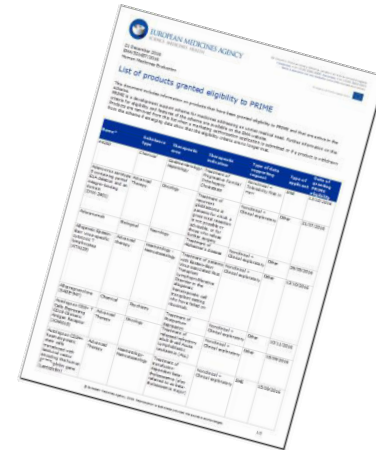
Report published on
[EMA website](#)

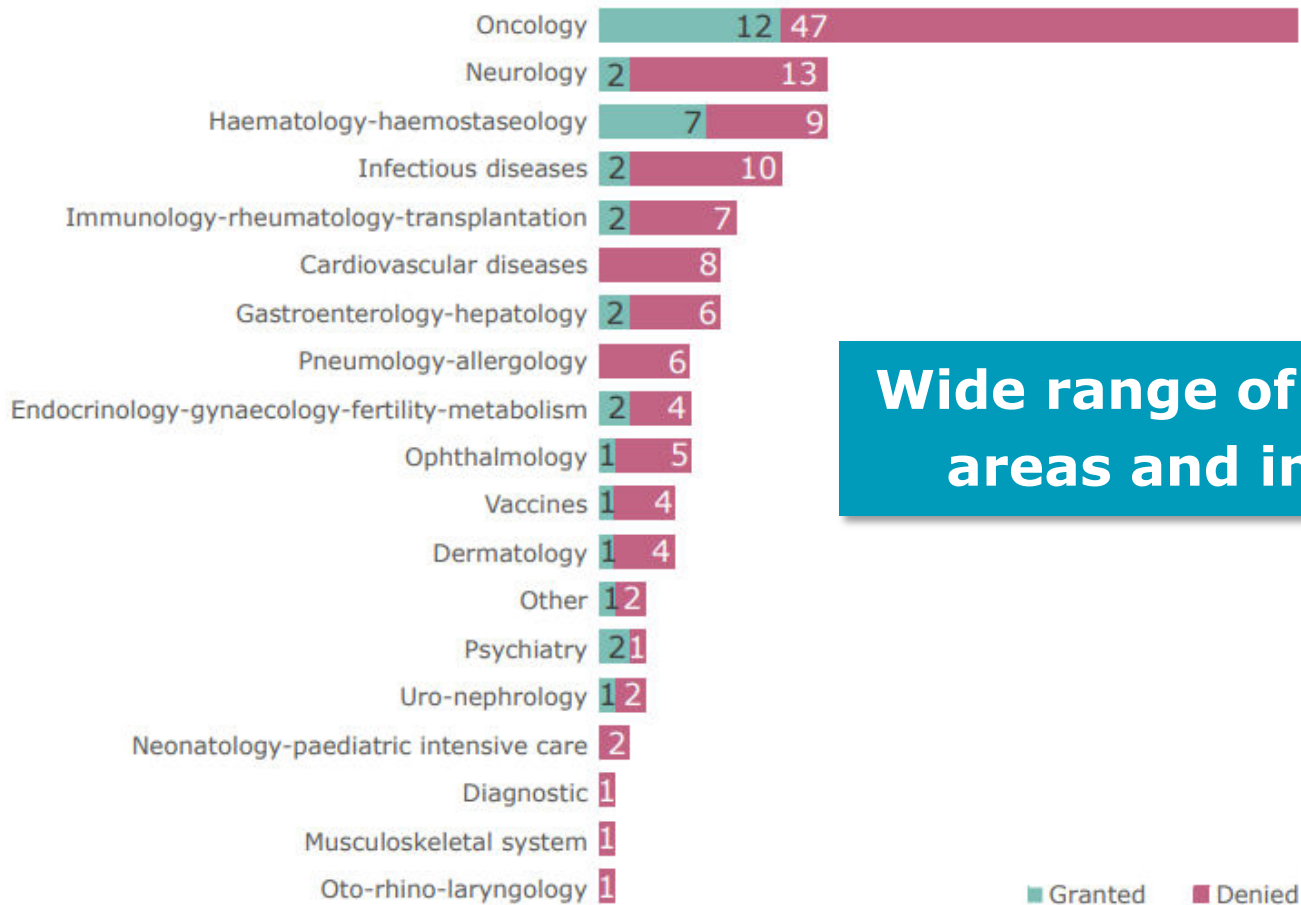
How criteria for eligibility have
been applied

What type of support
applicants have received so far

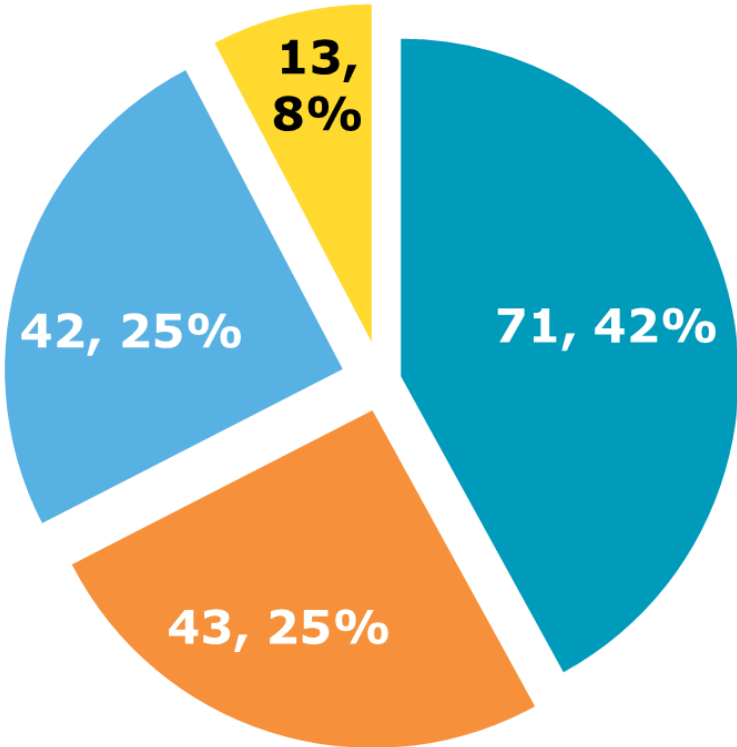


+ Publication of report and list of products on EMA website





Wide range of therapeutic areas and indications



**High number of
advanced therapy
medicines**

■ Chemical ■ Biological ■ ATMP ■ Other



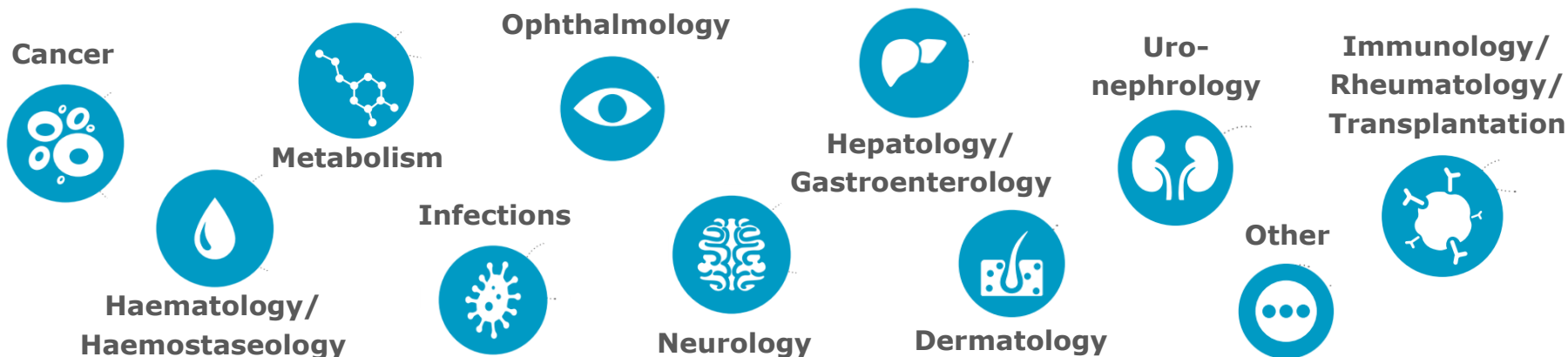
36 products eligible to PRIME since launch

30 in rare diseases

16 for paediatric patients

15 advanced therapy

Areas of unmet medical need and therapeutic indications covered



Features of the PRIME scheme

Early access tool, supporting patient access to innovative medicines.



- **Written confirmation of PRIME eligibility** and potential for accelerated assessment;
- **Early CHMP Rapporteur appointment** during development;
- **Kick off meeting** with multidisciplinary expertise from EU network;
- **Enhanced scientific advice** at key development milestones/decision points;
- **EMA dedicated contact point**;
- **Fee incentives** for SMEs and academics on Scientific Advice requests.



Kick-off meeting

- Multi disciplinary meeting with relevant experts from SAWP and CHMP and other committees;
- Introduction of product and development status by applicant;
- To take place shortly after eligibility confirmation, at EMA
- Facilitate initial interaction between applicant and EU regulatory network;
- Discuss the overall development plan and regulatory strategy;
- Provide recommendation on milestones and topics for scientific advice.

Our experience so far: 31 meetings

- Excellent tool to initiate dialogue and provide recommendation on the next steps
- Tailored to individual product
- Opportunity to raise awareness on importance of early dialogue with HTAs

Recent industry survey (15 responders)

93% agree the meeting is useful

Areas for improvement:

Support understanding of how to interact with different committees
Post-meeting follow-up

Enhanced scientific advice

22 products
37 SA requests
following kick-off meetings

Multi-stakeholder

7 EMA/HTA parallel advice
6 with patients involved

Rapporteur involvement

through one of SAWP
coordinator



All aspects covered

Quality,
nonclinical, clinical,
including post-authorisation

Flexibility

Shorter pre-submission
12 with shorter timelines

New and updated guidance to reflect 2 years of experience



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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.

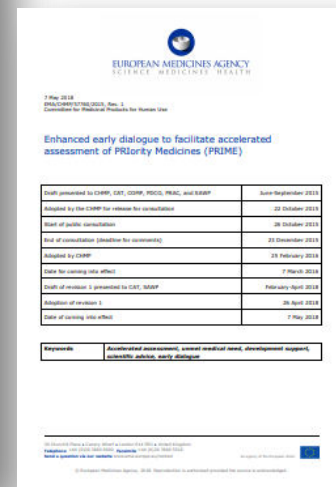
Related content

- ▶ Support for early access
- ▶ Two years of PRIME (7/5/2018)
- ▶ First anniversary of PRIME: experience so far (19/5/2017)
- ▶ Launch of PRIME – Paving the way for promising medicines for patients (07/03/2016)

Report: PRIME: a two-year overview



Updated Reflection paper, Q&A, template



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7 May 2018
EMA/000711/2018
Communication for Medicines Professionals for Human Use

Enhanced early dialogue to facilitate accelerated assessment of Priority Medicines (PRIME)

Event	Date
Scout presented to CHMP, EPAR, CHMP, PRIME, PRAC, and SBMP	June September 2016
Adopted by the CHMP for release for consultation	22 October 2016
Start of public consultation	26 October 2016
End of consultation (deadline for comments)	23 December 2016
Adopted by CHMP	23 February 2017
Date for entering into effect	17 March 2017
Draft of guideline 2 presented to CHMP, SBMP	February-April 2017
Adoption of guideline 2	26 April 2017
Date of entering into effect	7 May 2018

Keywords: Accelerated assessment, unmet medical need, development support, scientific advice, early dialogue

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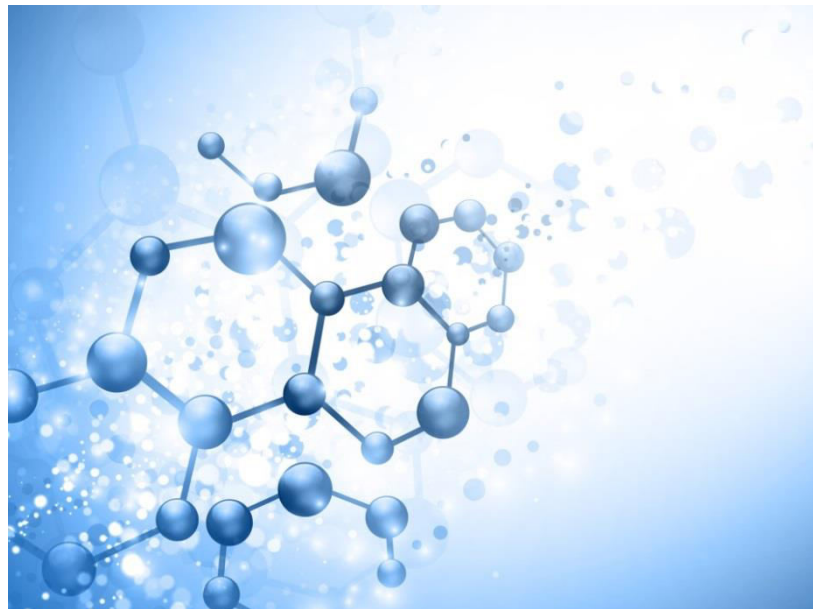
7 May 2018
EMA/000711/2018
Human Medicines Evaluation Division

European Medicines Agency Guidance on interactions in the context of PRIME

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New guidance on kick-off meetings and other interactions

In summary,



PRIME continues to meet expectations after successful implementation in 2016

Platform promoting development of promising medicines that focus on unmet medical needs

Majority of PRIME products in rare diseases

Scientific advices at key milestones with opportunity for multi-stakeholders involvement, including HTAs and patients

3 marketing authorisations under evaluation



Thank you for your attention

Further information

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