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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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

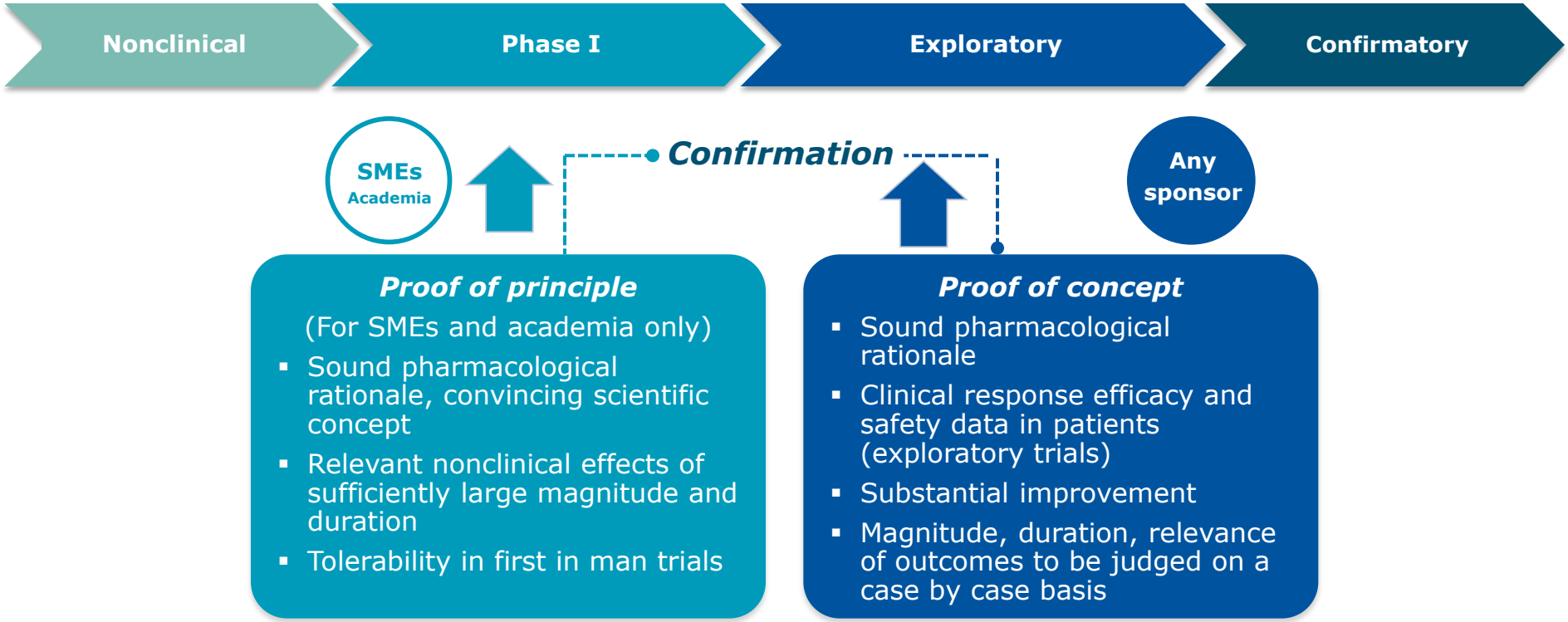
- Epidemiological data about the disease
- Description of available diagnostic, prevention and treatment options/standard of care (SOC), their effect and how medical need is not fulfilled

Potential to significantly address the unmet medical need

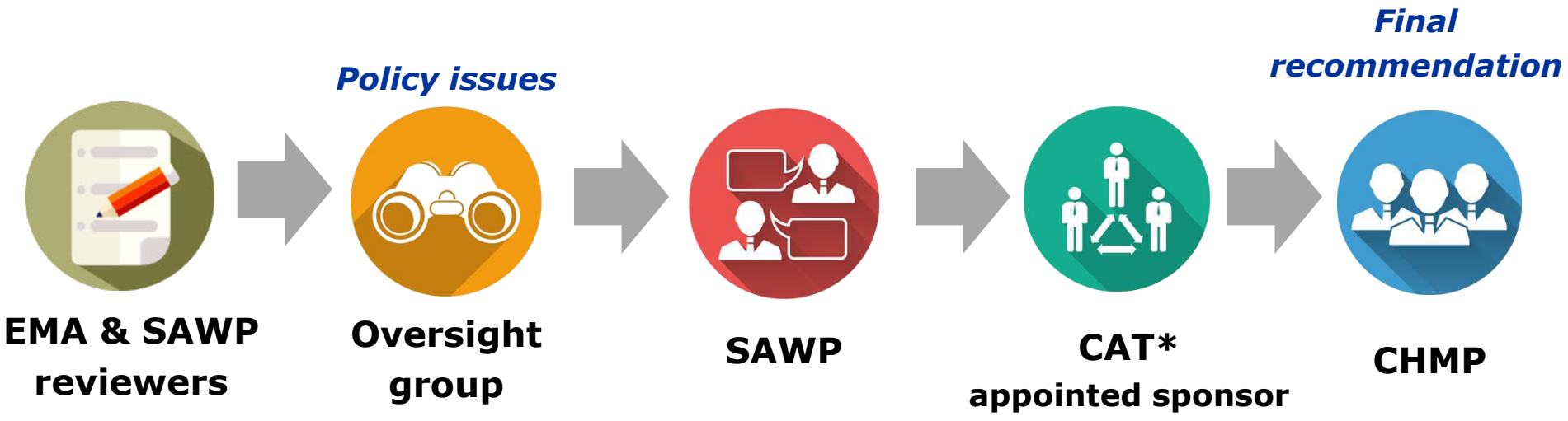
- Description of observed and predicted effects, clinical relevance, added value and impact
- If applicable, expected improvement over existing treatments

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

*For advanced therapies

What do we expect to grant eligibility?

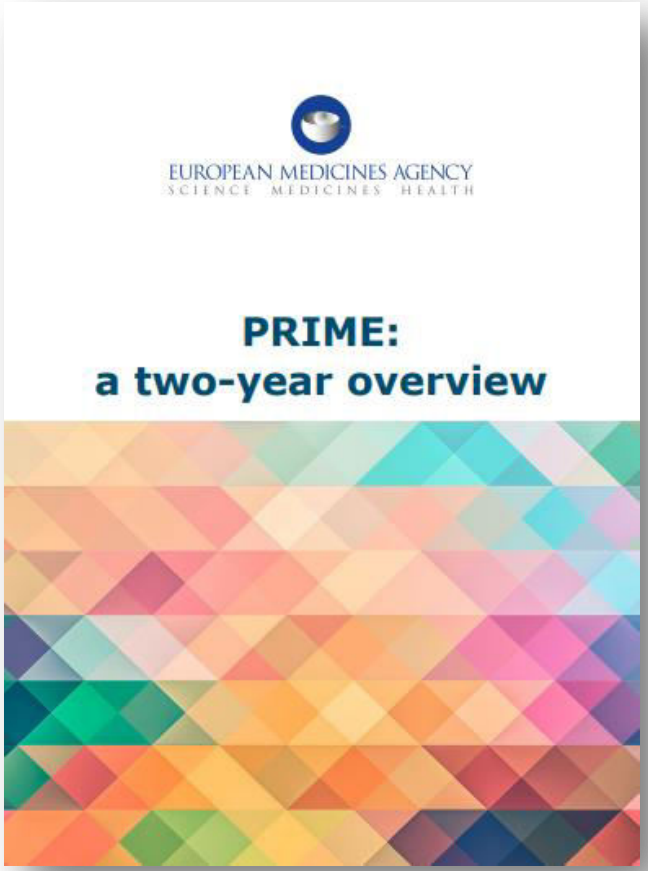
Unmet medical need
No treatment or clear limitations of existing therapies

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

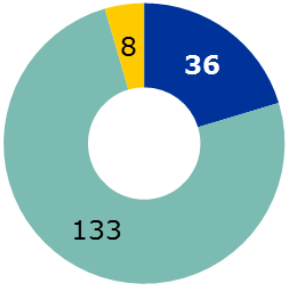


Report published on
[EMA website]

How criteria for eligibility have been applied

What type of support applicants have received so far

Two years of PRIME

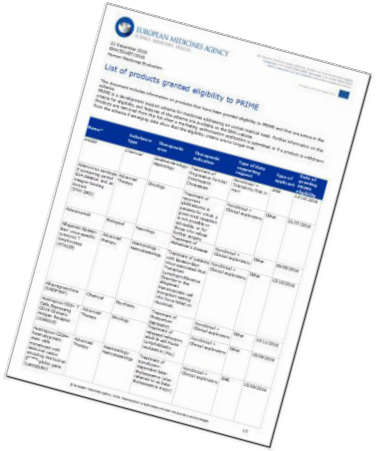


■ Granted ■ Denied ■ Out of scope*

21% success rate

177 requests received
> 50% from SMEs
36 granted*

+
Publication of report and list of products on EMA website



Overview of therapeutic areas

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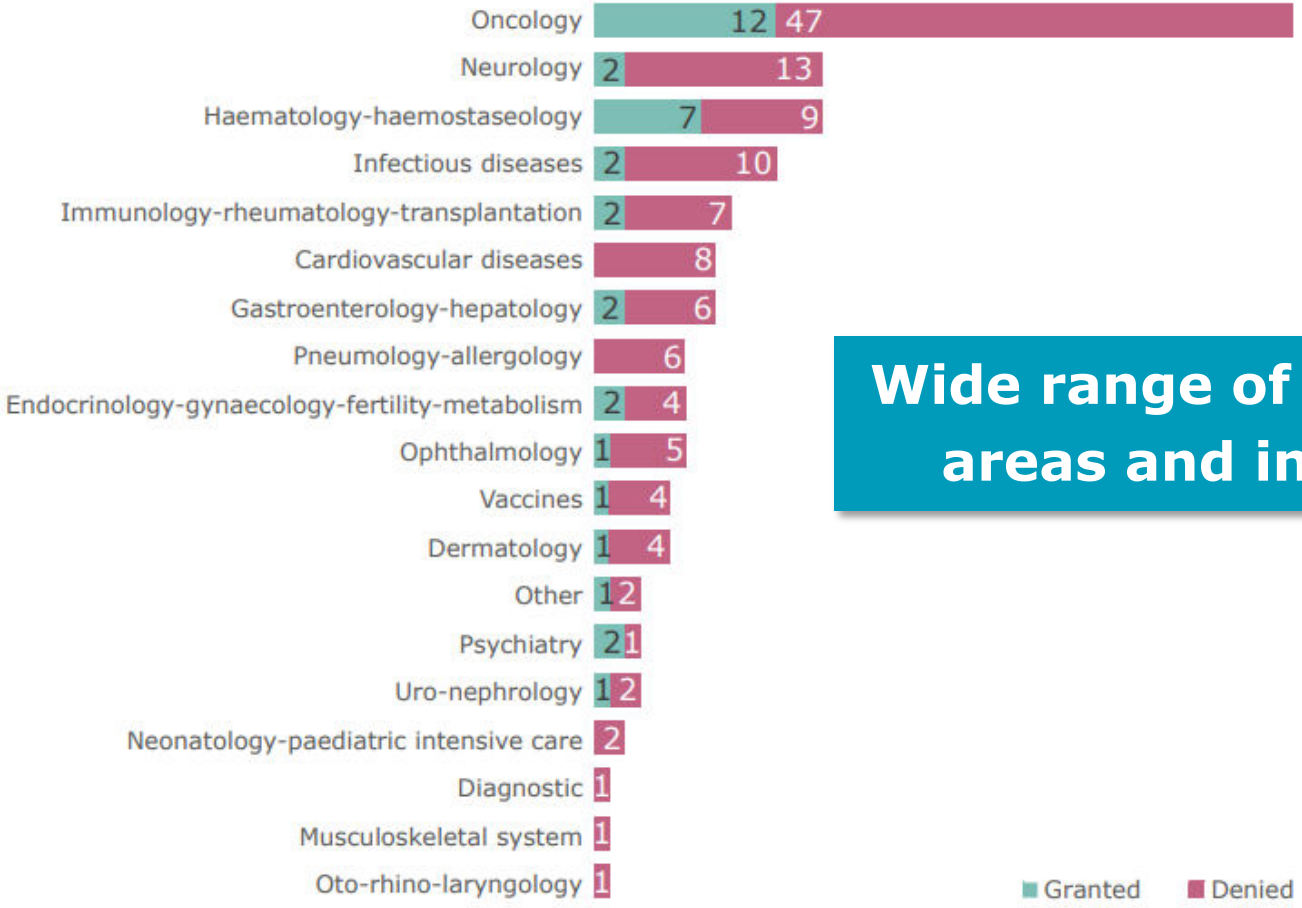
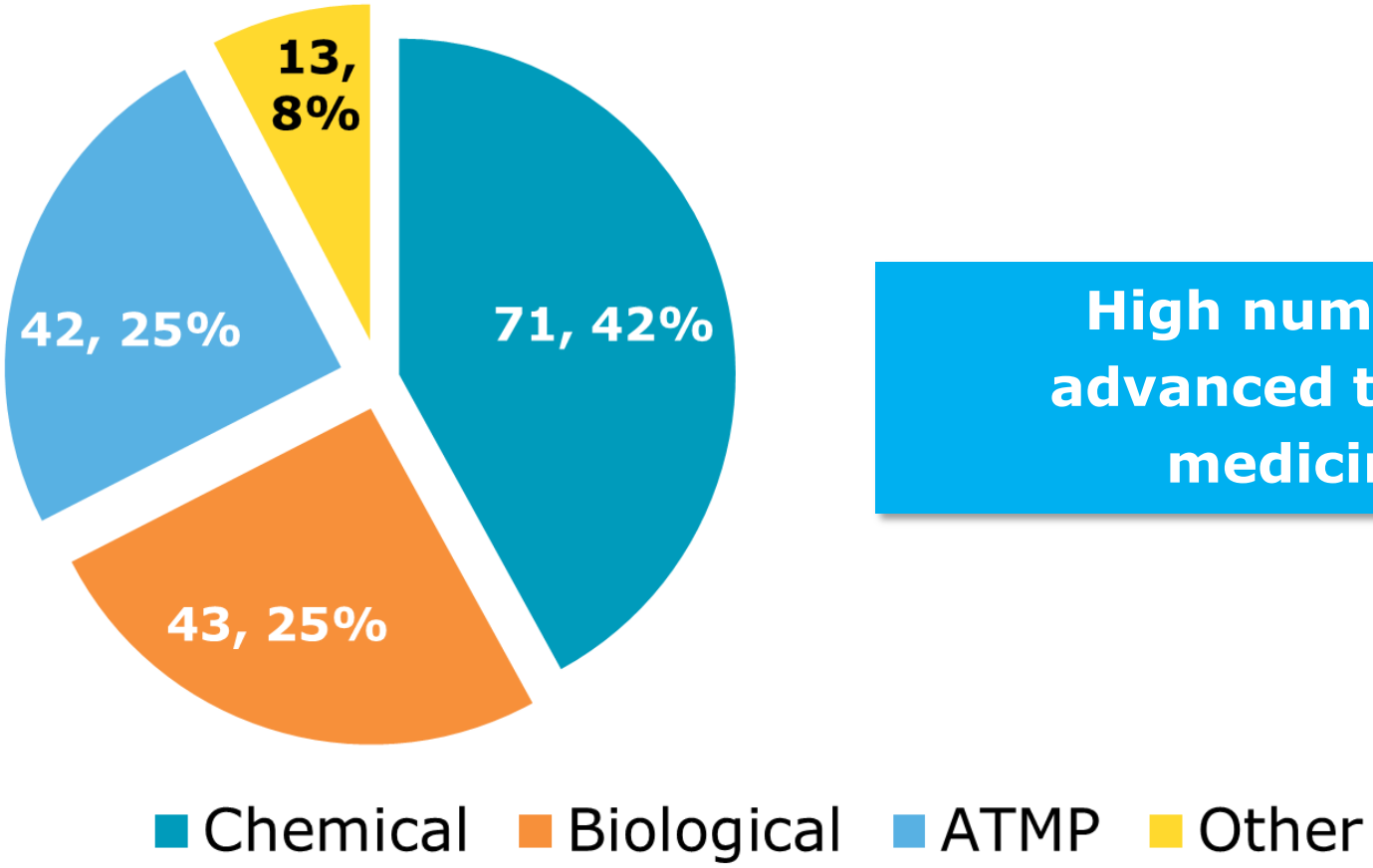


Wide range of therapeutic areas and indications

Overview of type of products



High number of advanced therapy medicines

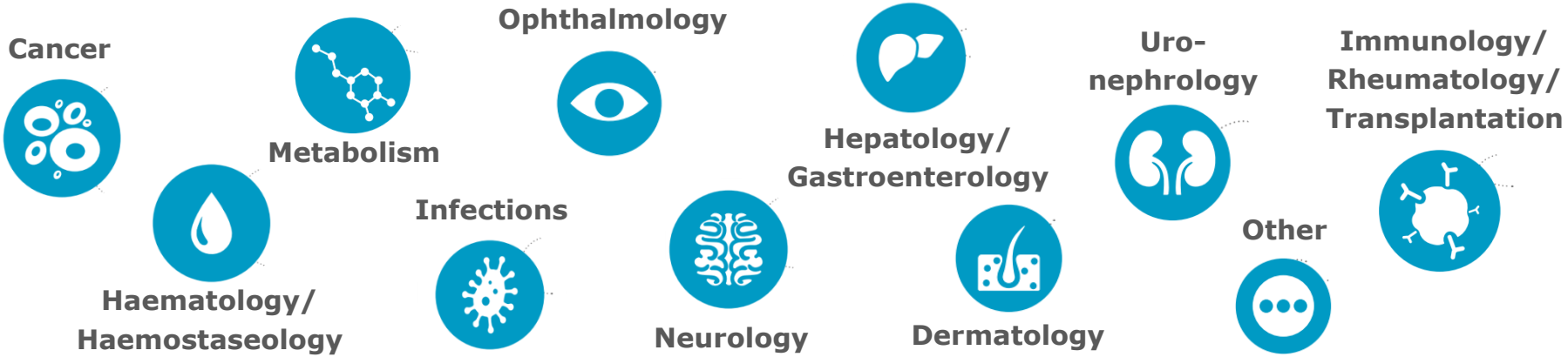
36 products eligible to PRIME since launch

30 in rare diseases

16 for paediatric patients

15 advanced therapy

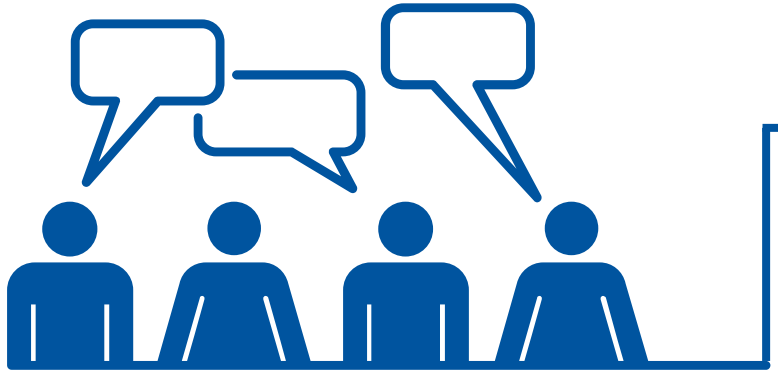
Areas of unmet medical need and therapeutic indications covered



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

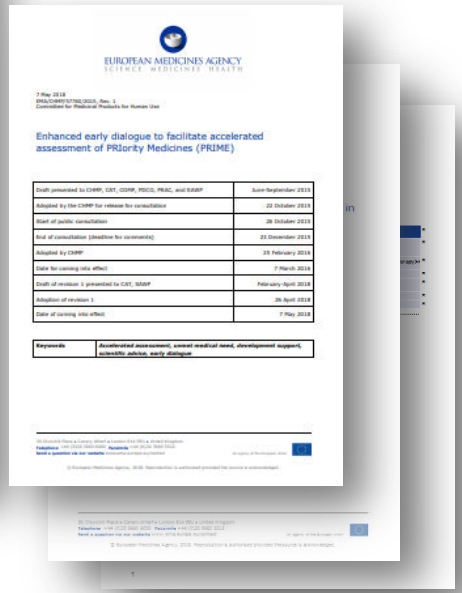
Rapporteur involvement
through one of SAWP coordinator

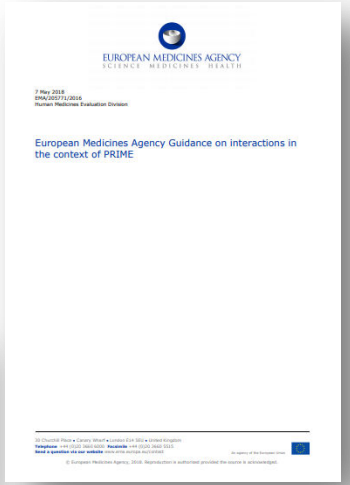
All aspects covered
Quality,
nonclinical, clinical,
including post-authorisation

Flexibility
Shorter pre-submission
12 with shorter timelines

New and updated guidance to reflect 2 years of experience

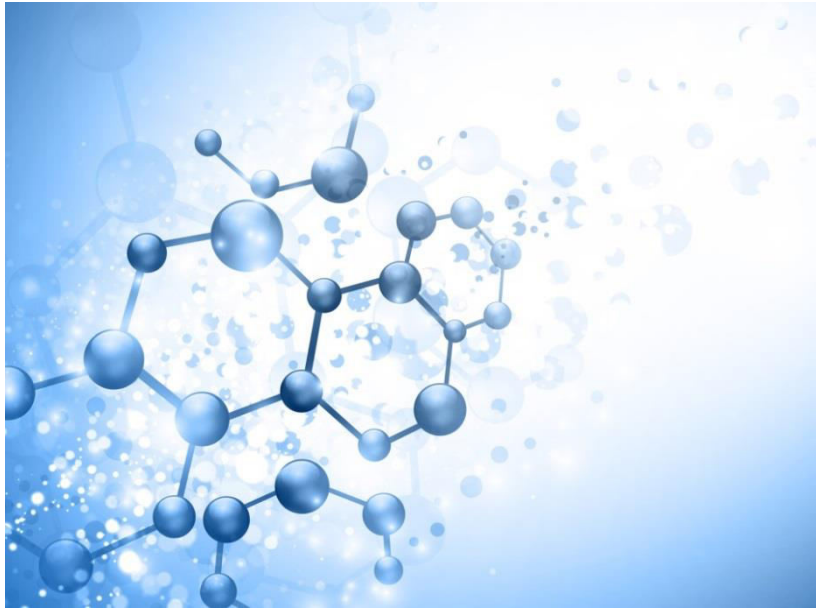
Updated
Reflection paper, Q&A, template





***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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