EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Priority Medicines (PRIME) scheme

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An agency of the European Union

Outline

- Why Prime?
- What is PRIME?
- PRIME Eligibility
- PRIME Support
- PRIME Live

Why PRIME?

Drivers for change

Patients

- Areas of unmet need
- Focus on accelerating regulatory approval of new medicines

Research & Development

- Scientific and regulatory challenges
- Importance of early dialogue with regulators and scientific advice
- Difficulty in access to capital investment for academia & SMEs

EU Network Strategy to 2020

- Ensure timely access to new beneficial and safe medicines for patients
- Support for patient focused innovation and contribute to a vibrant life science sector in Europe

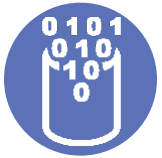
What is PRIME?

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

- Facilitated by knowledge gained throughout development
- Feedback of relevant SA aspects to CHMP

Building on existing framework;

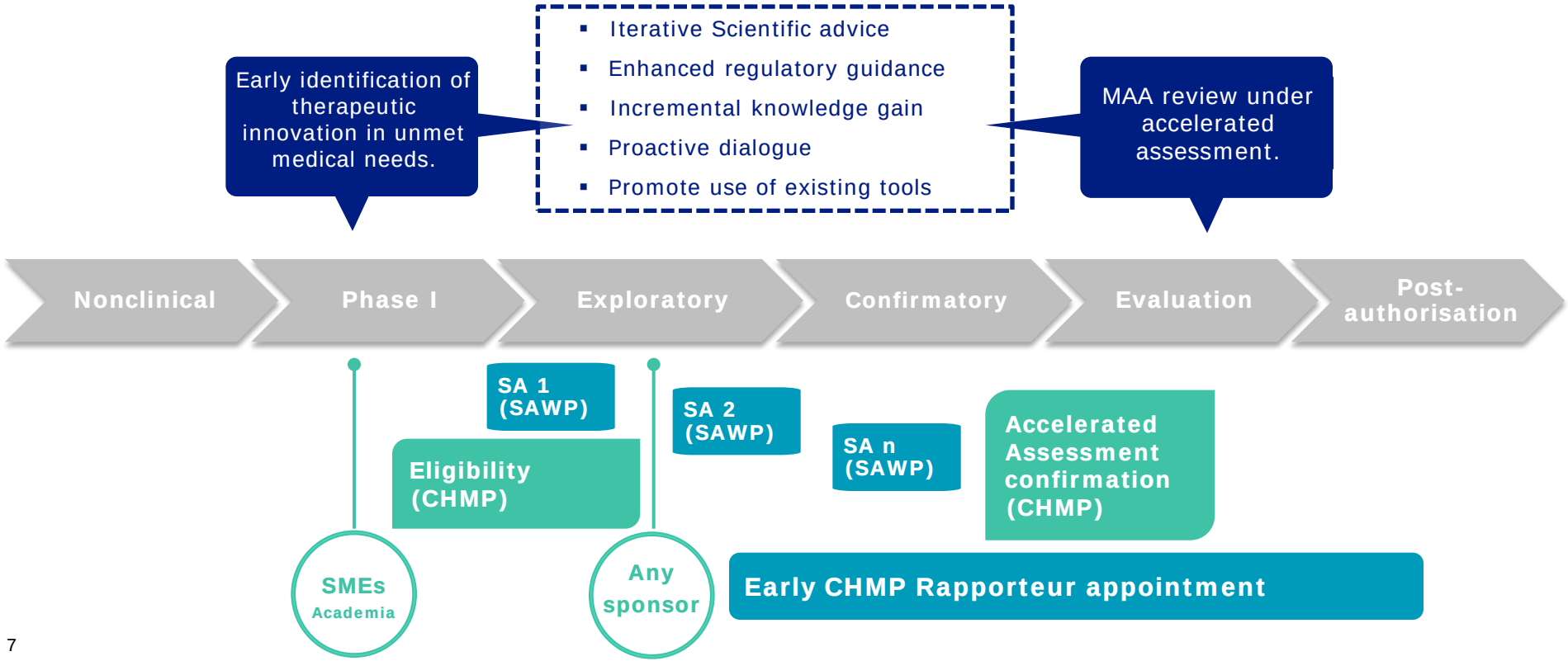
Eligibility according to existing ‘Accelerated Assessment criteria’

Features of the PRIME scheme

A tailored and enriched scientific and regulatory development support

- **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.

Overview of PRIME scheme



Timing of PRIME eligibility

For products under development (i.e. not yet authorised)

- Entry to scheme at two different stages in development:
  - at the earlier stage of **proof of principle** (prior to phase II/exploratory clinical studies) focusing on SMEs and Academia.
  - at **proof of concept** (prior to phase III/confirmatory clinical studies).
- Must be based on adequate data to justify a potential major public health interest.

If PRIME is not the right tool

- Very early stage of development
- Products already authorised
- Products that are already in pre-submission stage (i.e. letter of intent received)

EMA still can provide support through…

PRIME Eligibility

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

Unmet medical need

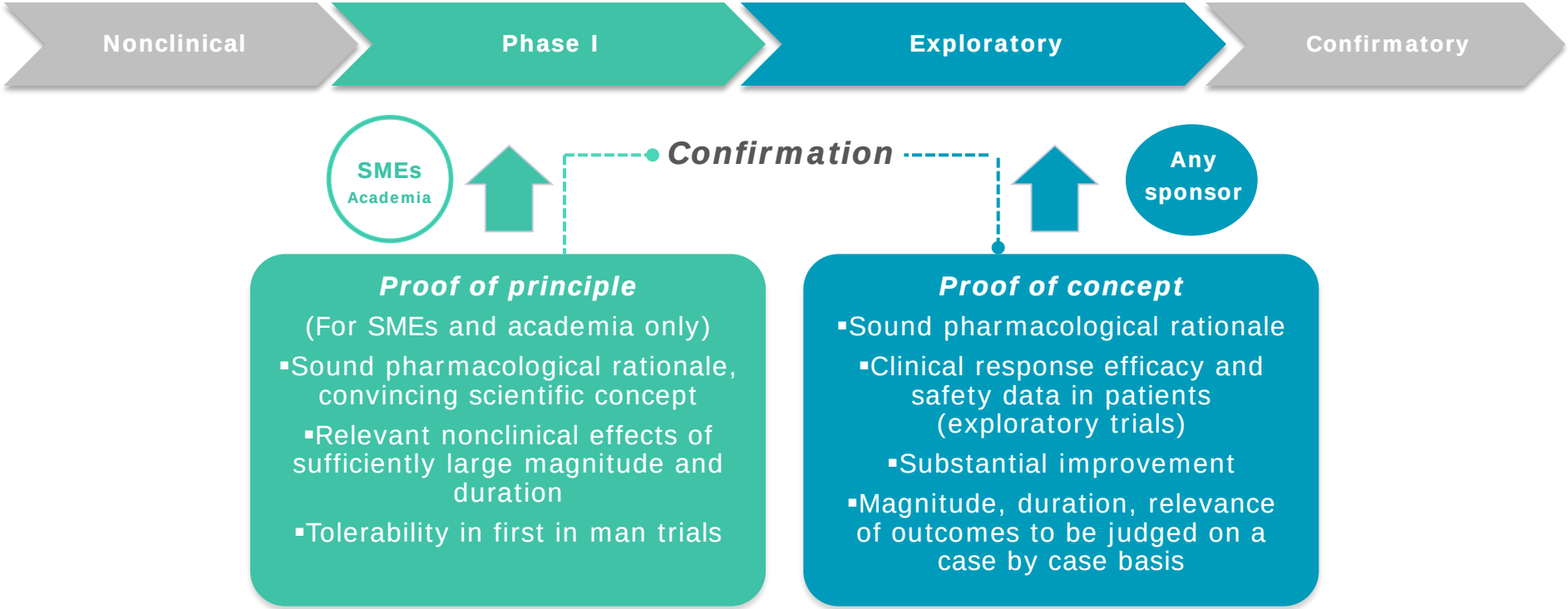
- Epidemiological data about the disease
- Description of available diagnostic, prevention and treatment options/standard of care, their effect and how medical need is not fulfilled

Potential to significantly address the unmet medical need

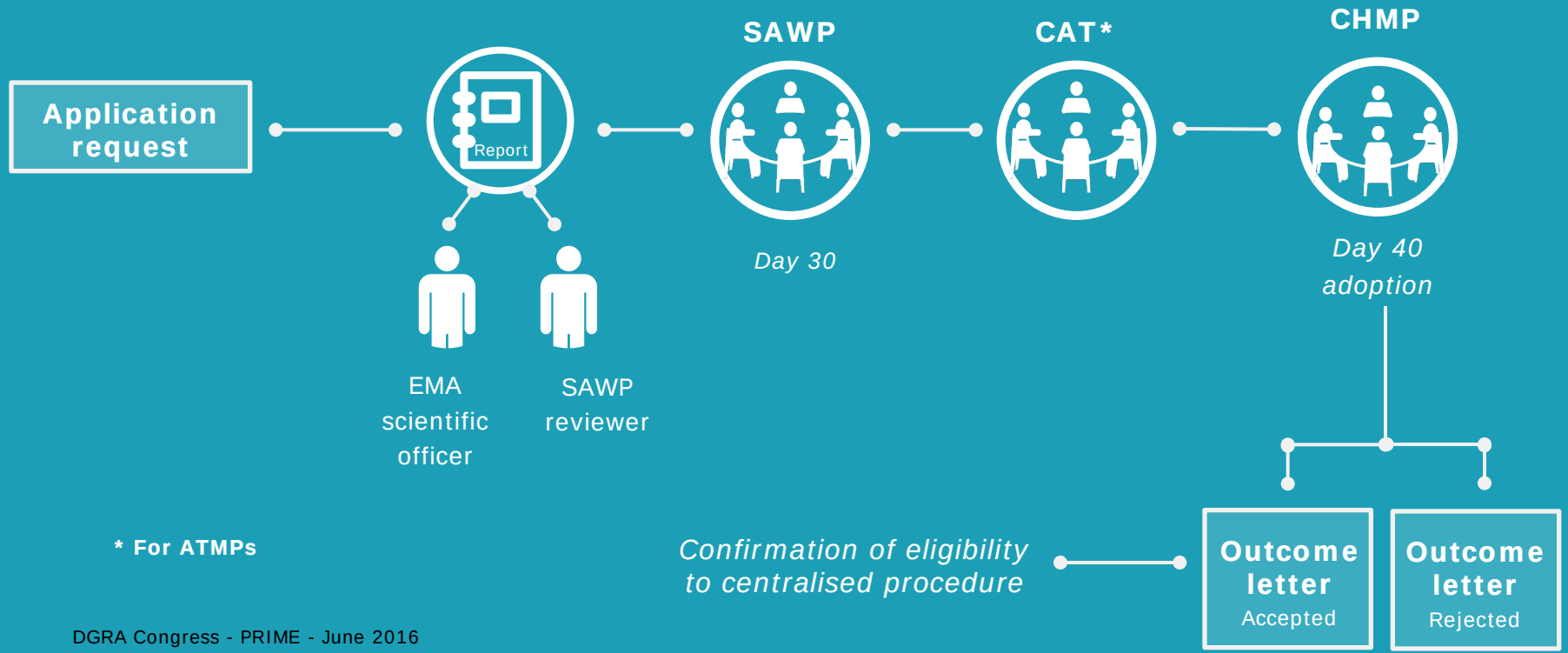
- Description of observed and predicted effects, clinical relevance, added value and impact
- If applicable, expected improvement over existing treatments

Data required at different stages of development

Entry points PRIME eligibility and required evidence

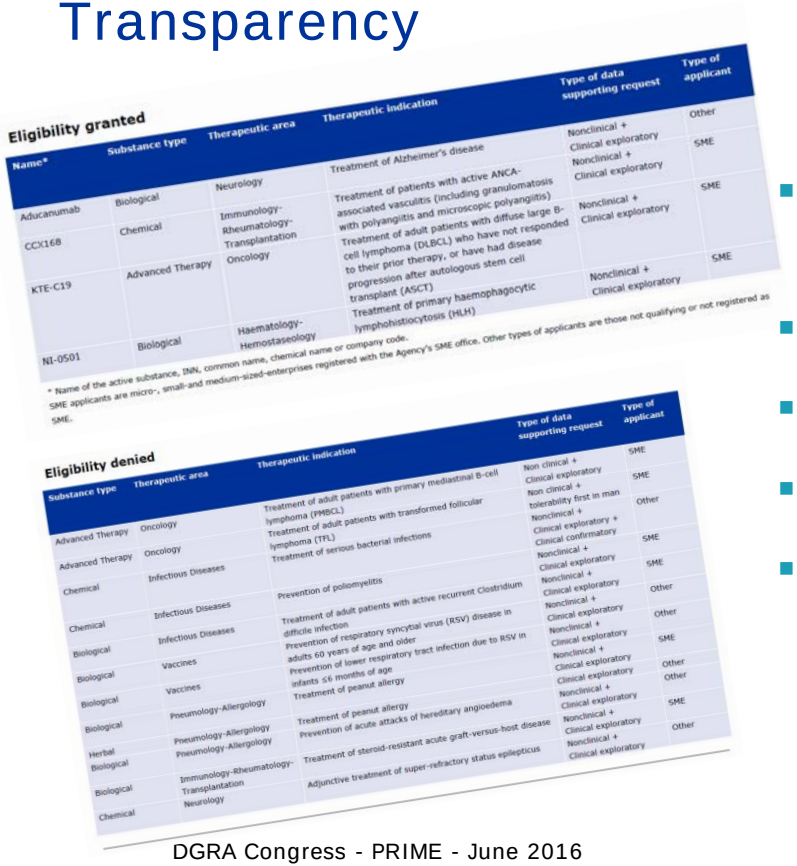


Assessment of Eligibility: 40-day procedure



*** For ATMPs**

Transparency



Eligibility granted

Name*	Substance type	Therapeutic area	Therapeutic indication	Type of data supporting request	Type of applicant
Aducanumab	Biological	Neurology	Treatment of Alzheimer's disease	Nonclinical + Clinical exploratory	Other
CCX168	Chemical	Immunology-Rheumatology-Transplantation	Treatment of patients with active ANCA-associated vasculitis (including granulomatosis with polyangiitis and microscopic polyangiitis)	Nonclinical + Clinical exploratory	SME
KTE-C19	Advanced Therapy	Oncology	Treatment of adult patients with diffuse large B-cell lymphoma (DLBCL) who have not responded to their prior therapy, or have had disease progression after autologous stem cell transplant (ASCT)	Nonclinical + Clinical exploratory	SME
NI-0501	Biological	Haematology-Hemostaseology	Treatment of primary haemophagocytic lymphohistiocytosis (HLH)	Nonclinical + Clinical exploratory	SME

* Name of the active substance, INN, common name, chemical name or company code.
SME applicants are micro-, small-and medium-sized-enterprises registered with the Agency's SME office. Other types of applicants are those not qualifying or not registered as SME.

Eligibility denied

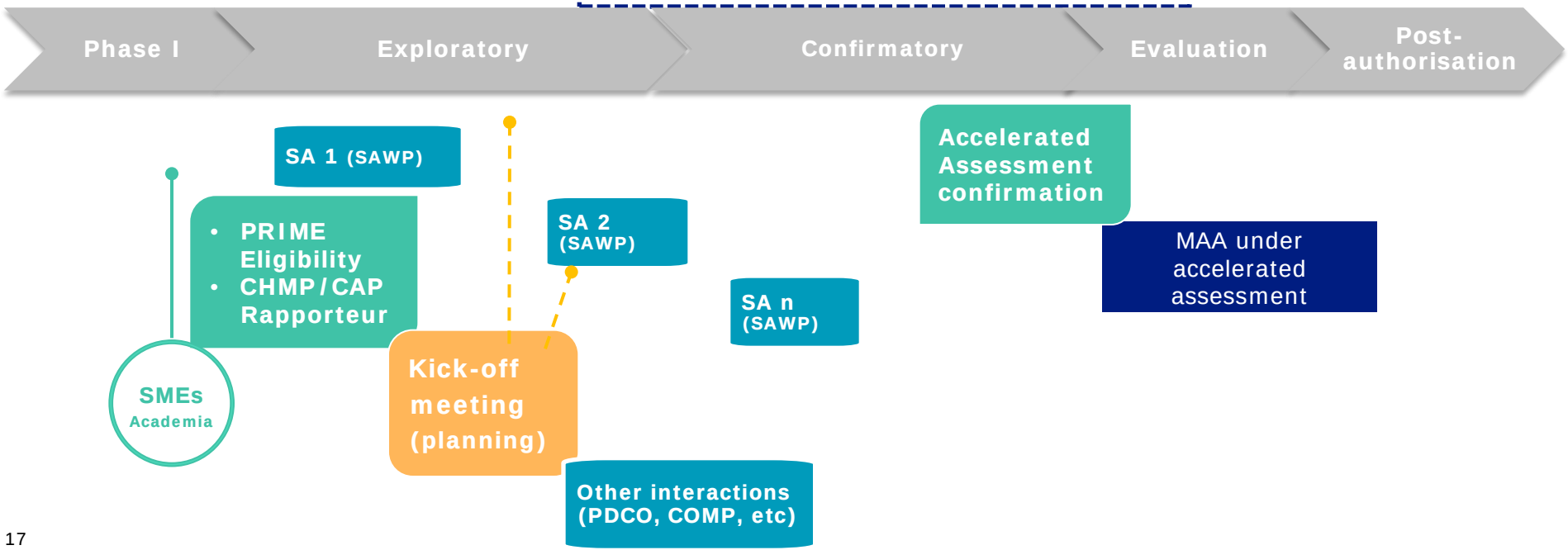
Substance type	Therapeutic area	Therapeutic indication	Type of data supporting request	Type of applicant
Advanced Therapy	Oncology	Treatment of adult patients with primary mediastinal B-cell lymphoma (PMBCL)	Non clinical + Clinical exploratory	SME
Advanced Therapy	Oncology	Treatment of adult patients with transformed follicular lymphoma (TFL)	Non clinical + tolerability first in man	SME
Chemical	Infectious Diseases	Treatment of serious bacterial infections	Nonclinical + Clinical exploratory + Clinical confirmatory	Other
Chemical	Infectious Diseases	Prevention of poliomyelitis	Nonclinical + Clinical exploratory	SME
Biological	Infectious Diseases	Treatment of adult patients with active recurrent Clostridium difficile infection	Nonclinical + Clinical exploratory	SME
Biological	Vaccines	Prevention of respiratory syncytial virus (RSV) disease in adults 60 years of age and older	Nonclinical + Clinical exploratory	Other
Biological	Vaccines	Prevention of lower respiratory tract infection due to RSV in infants ≤6 months of age	Nonclinical + Clinical exploratory	Other
Biological	Pneumology-Allergology	Treatment of peanut allergy	Nonclinical + Clinical exploratory	SME
Biological	Pneumology-Allergology	Treatment of peanut allergy	Nonclinical + Clinical exploratory	Other
Herbal	Pneumology-Allergology	Prevention of acute attacks of hereditary angioedema	Nonclinical + Clinical exploratory	Other
Biological	Immunology-Rheumatology-Transplantation	Treatment of steroid-resistant acute graft-versus-host disease	Nonclinical + Clinical exploratory	SME
Chemical	Neurology	Adjunctive treatment of super-refractory status epilepticus	Nonclinical + Clinical exploratory	Other

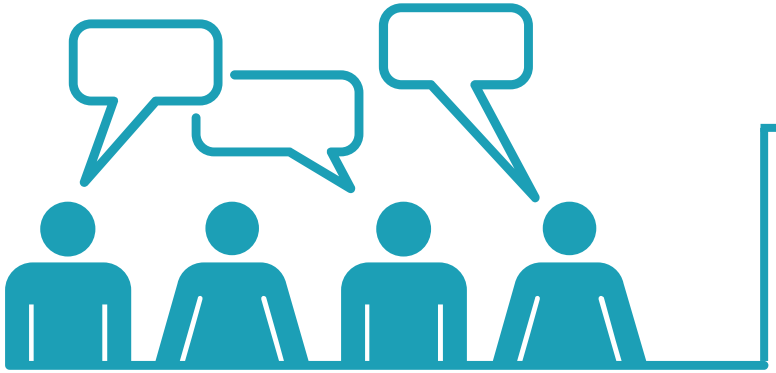
- Publication of recommendations on eligibility to PRIME (both granted and denied)
- Broad characteristics
- Active substance/INN for eligible products
- Monthly statistics published after CHMP
- EMA will share information with relevant partners and stakeholders

PRIME Support

PRIME scheme support

- Early CHMP/CAT Rapporteur appointment
- Kick-off meeting
- Iterative Scientific advice
- Proactive dialogue and early and enhanced scientific and regulatory support





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 CHMP/CAT Rapporteur appointment;
- To be held at EMA, where feasible e.g. in the context of SA procedure;
- Facilitate initial interaction between applicant and EU regulatory network;
- Discuss the overall development plan and regulatory strategy;
- Provide recommendation on milestones and topics for SA.

With PRIME

Planning and facilitating accelerated assessment

- Plan for coordinated interactions with regulators
- Identify issues to be addressed early in development
- Support preparation of robust marketing authorisation application
- Adequate post-approval planning

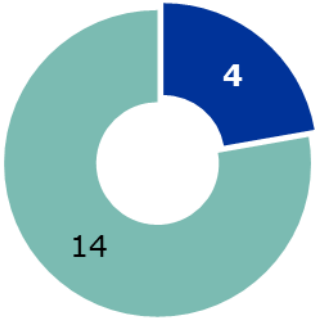
Make better use of existing tools for early access

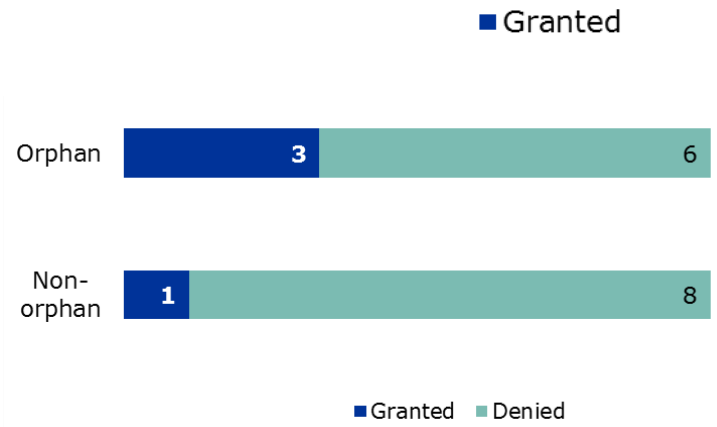
PRIME - Monitoring of development

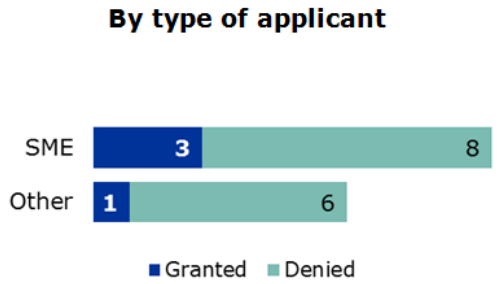
- Monitoring within iterative Scientific Advice
- Regular updates from applicants expected
- Possibility to withdraw products from scheme if criteria are no longer met
- Accelerated assessment to be re-confirmed prior to MAA submission

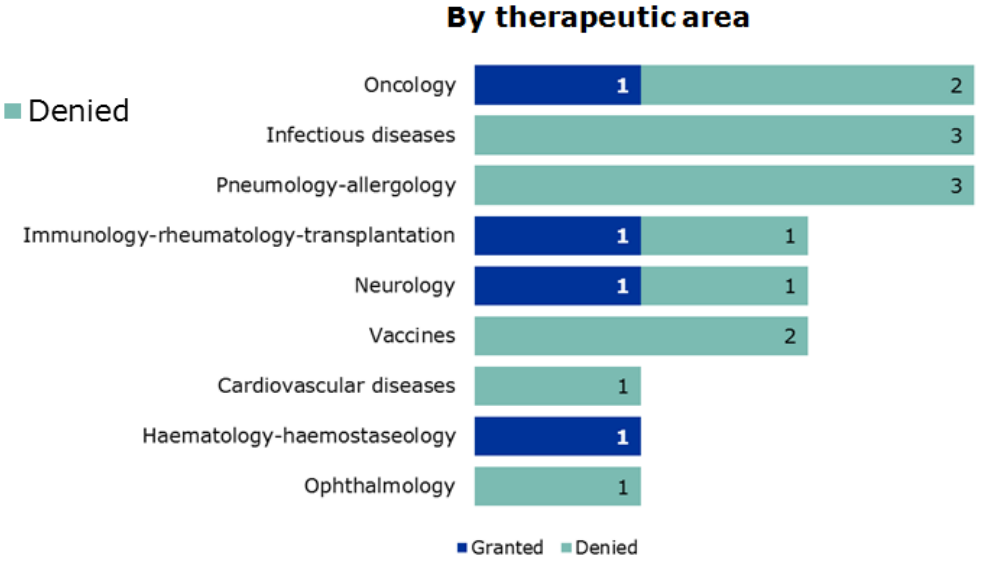
PRIME Live

PRIME eligibility requests received 7 March- 6 April 2016









A few observations…

- ✓ All applications in scope
- ✓ All of adequate quality to allow assessment
- ⚠ Most requests received on the day of the deadline or the day before
- ⚠ No need to submit separate form for eligibility to centralised procedure
- ⚠ PIP or waiver not yet submitted in 12/18

In conclusion

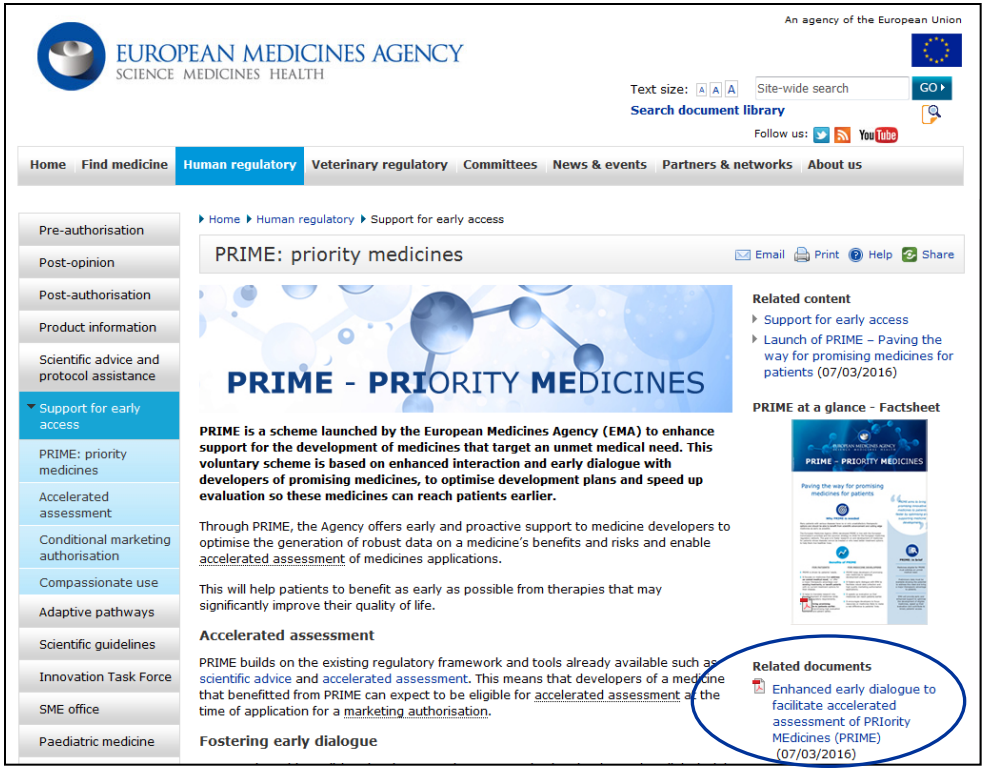
In summary,

PRIME created to foster the development of *medicines with high public health potential*

- Reinforce scientific and regulatory advice
- Optimise development for robust data generation
- Enable accelerated assessment

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

PRIME webpage and supporting documents



Factsheet in lay language

Q&A, templates, application form for applicants

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Thank you for your attention

Further information

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