EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

PRIME

SME workshop, 3 October 2016

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Scientific and Regulatory Management Department

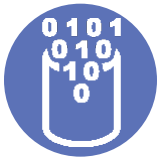
An agency of the European Union

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

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

Building on existing framework;

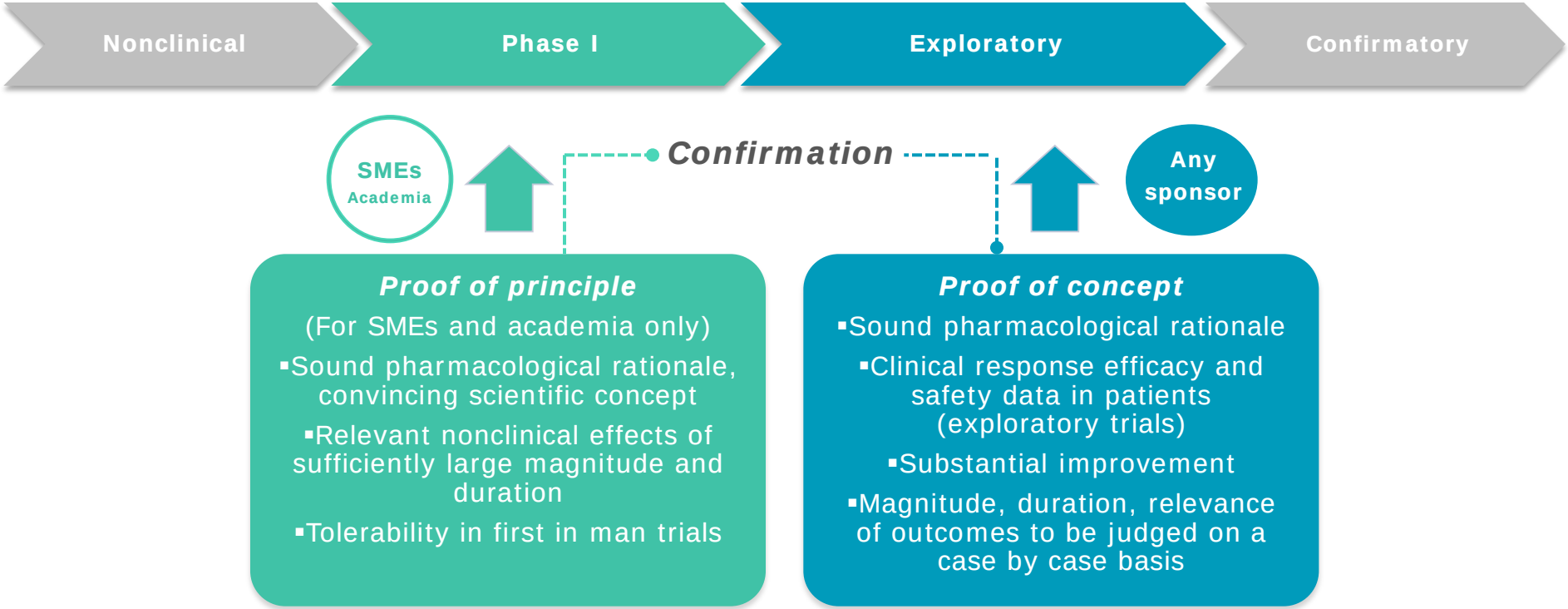
Eligibility according to existing ‘Accelerated Assessment criteria’

Features of the PRIME scheme

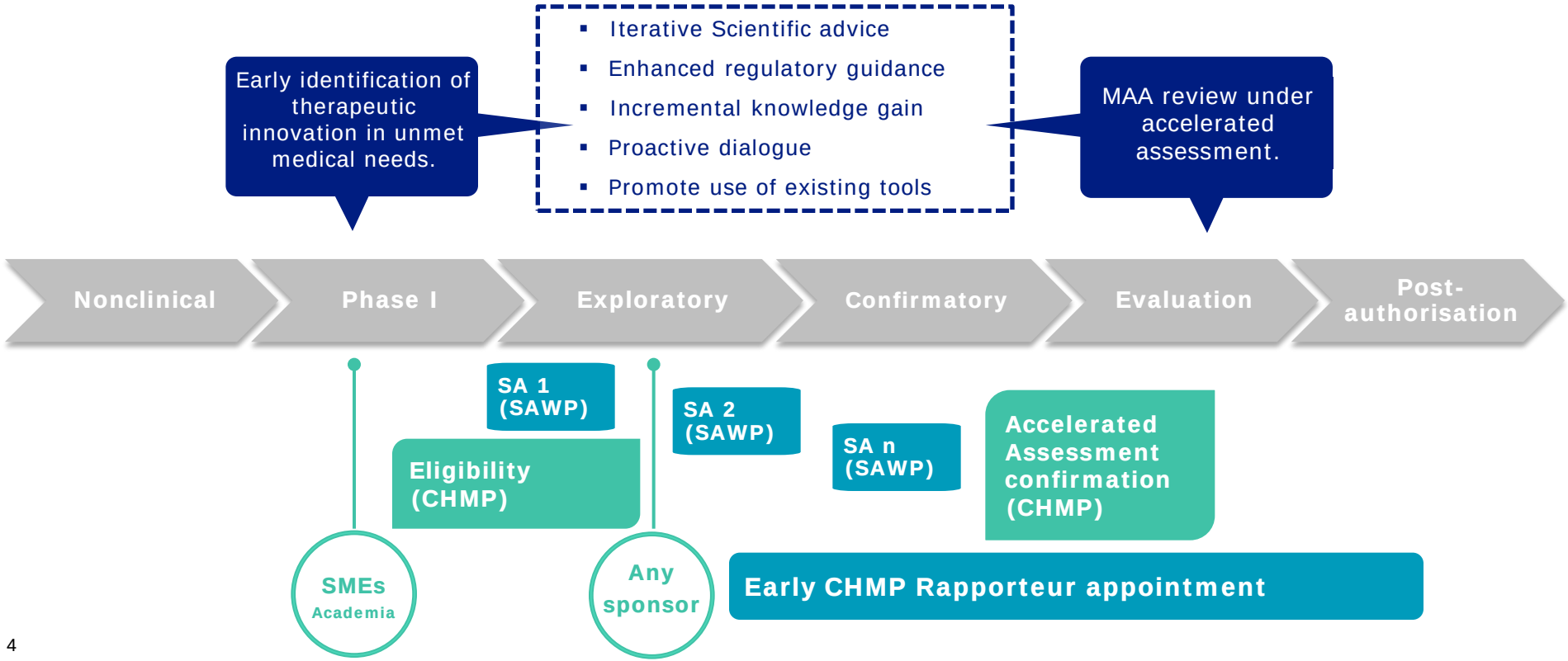
Early access tool, supporting patient access to innovative medicines.

- **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.

Entry points PRIME eligibility and required evidence



Overview of PRIME scheme



PRIME eligibility criteria and request

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, 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

Justification assessed by EMA's scientific committees

Justification for eligibility to PRIME


Date

PRIME eligibility request – Applicant's justification

<(Invented) Name>

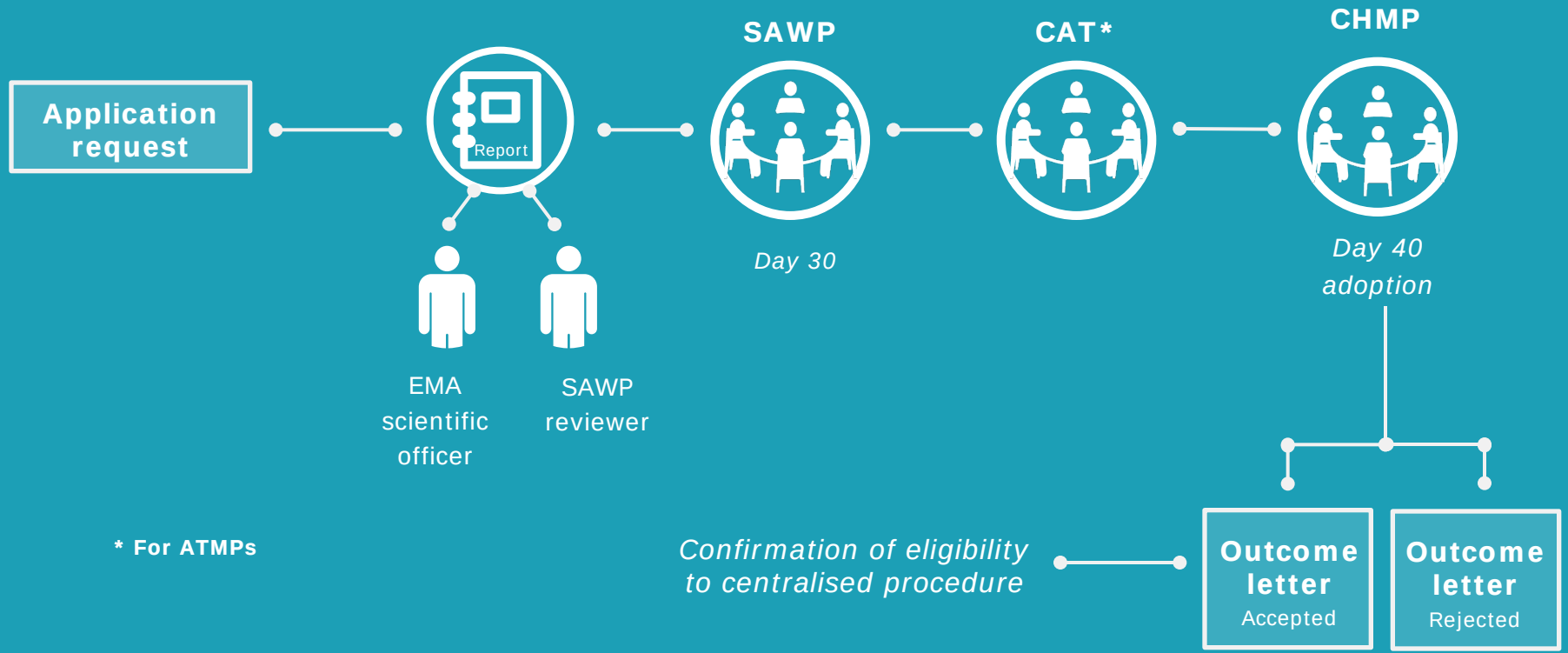
Product information	
Active substance (INN or common name or company code):	<Chemical> <Biological> <Advanced therapy>
Substance Type:	
Description of the product & mechanism(s) of action:	
Therapeutic indication:	
Applicant:	
UPL number/EMA number:	

- Short background on disease & product
- Unmet medical need
  - Epidemiology of disease
  - Available treatment
- Supportive evidence
  - Nonclinical pharmacology
  - Clinical data (eg exploratory efficacy + safety)
  - Conclusion on claim of major public health interest
  - Is there an unmet medical need in the proposed indication?
  - Is the data sufficient to support product's potential to significantly address unmet medical need?

Focus on nonclinical data & proof of principle

- Sound pharmacological rationale, convincing scientific concept
  - But, new pharmacological target or mechanism of action is not sufficient
- Relevant nonclinical effects
  - in vitro and in vivo data from relevant models, with comparison to results from other products if possible
  - Observed effects: sufficiently large and/or of long duration
  - Compelling results to outweigh many uncertainties of very early stage
- Early clinical data from first in man trials
  - Acceptable tolerability
  - PK to confirm sufficient exposure so that nonclinical effect may be observed in man

Assessment of Eligibility: 40-day procedure



*** For ATMPs**

Transparency


EUROPEAN MEDICINES AGENCY
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27 July 2016
EMA/434593/2016
Press office

Recommendations on eligibility to PRIME scheme
Adopted at the CHMP meeting of 18-21 July 2016

Eligibility granted

Name*	Substance type	Therapeutic area	Therapeutic indication	Type of data supporting request	Type of applicant
Autologous CD4 and CD8 T cells transduced with lentiviral vector	Advanced Therapy	Oncology	Treatment of HLA-A*0201, HLA-A*0205, or HLA-A*0206 allele positive patients with inoperable or metastatic synovial sarcoma who have received prior chemotherapy and whose	Nonclinical + Clinical exploratory	SME

Eligibility denied

Substance type	Therapeutic area	Therapeutic indication	Type of data supporting request	Type of applicant
Biological	Oncology	Treatment of relapsed and refractory multiple myeloma.	Nonclinical + Clinical exploratory	Other
Biological	Oncology	Treatment of diffuse intrinsic pontine glioma (DIPG)	Nonclinical + Clinical exploratory	SME
Biological	Haematology - Hemostaseology	Treatment of hemophilia A	Nonclinical + Clinical exploratory	Other
Chemical	Haematology - Hemostaseology	Treatment of iron overload in patients with beta thalassaemia	Nonclinical + Clinical exploratory	SME
Advanced Therapy	Gastroenterology-Hepatology	Treatment of active moderate to severe Crohn's Disease (CD)	Nonclinical + Clinical exploratory	SME
Chemical	Infectious Diseases	Treatment of invasive fungal infections	Non clinical + tolerability first in man	SME
Biological	Pneumology-Allergology	Treatment of peanut allergy	Clinical exploratory	Other

SME applicants are micro-, small-and medium-sized-enterprises registered with the Agency's SME office. Other type of applicants are those not qualifying or not registered as SME or not fulfilling the definition of academic sponsors.

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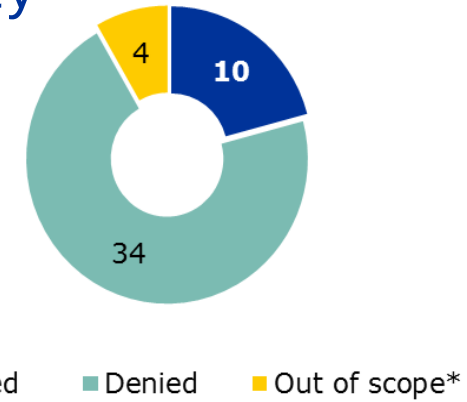
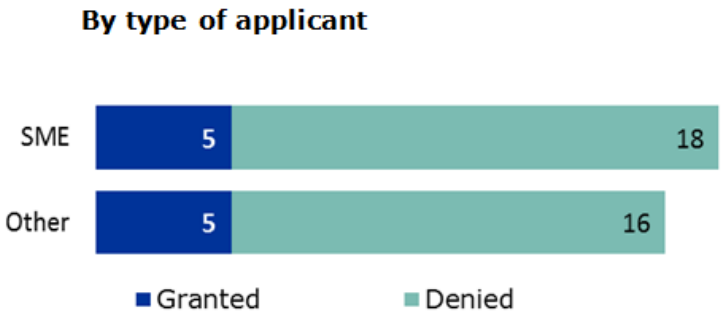
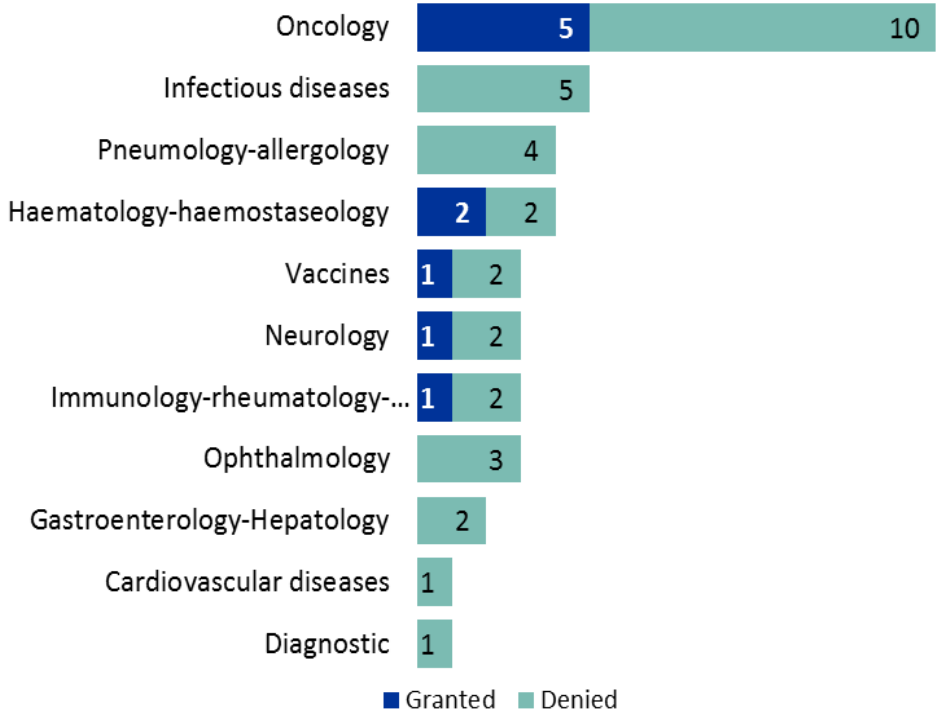
- Monthly report in CHMP highlights
- Broad characteristics
- Active substance/INN for eligible products
- High-level statistics regularly updated

PRIME eligibility requests received as of 29 June 2016





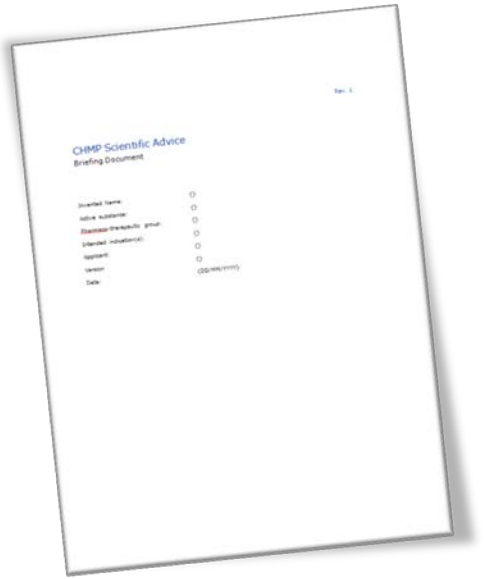


Support to eligible products

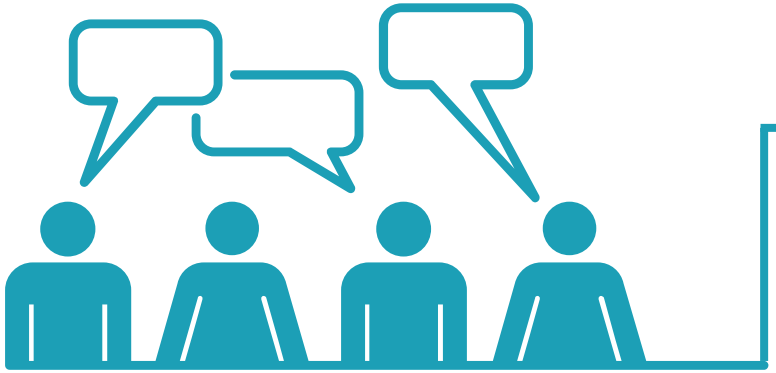
What happens next?

Rapporteur nomination

Kick-off meeting



Scientific advice



Kick-off meeting

- Multi disciplinary meeting with relevant experts from SAWP and CHMP and other committees;
- To take place, at EMA, shortly after eligibility confirmation;

- Facilitate initial interaction between applicant and EU regulatory network;
- Introduction of product and development status by applicant;
- Discuss the overall development plan and regulatory strategy;
- Regulatory guidance and awareness on requirements
- Provide recommendation on milestones and identify issues for scientific advice;
- Plan interactions with regulators.

Support to be channelled through Scientific Advice by SAWP/CHMP

- Discuss detailed development plan, design of pivotal studies
- Discuss key issues for MAA, at major milestones
- Expectation of iterative advice, higher frequency of interactions
- Continuity will facilitate sharing of knowledge from development to life-cycle
- Fee waivers for SMEs and academia

Role of SAWP coordinator

- One SAWP coordinator to follow all iterative Scientific Advice
- Recommend next milestones for SA requests

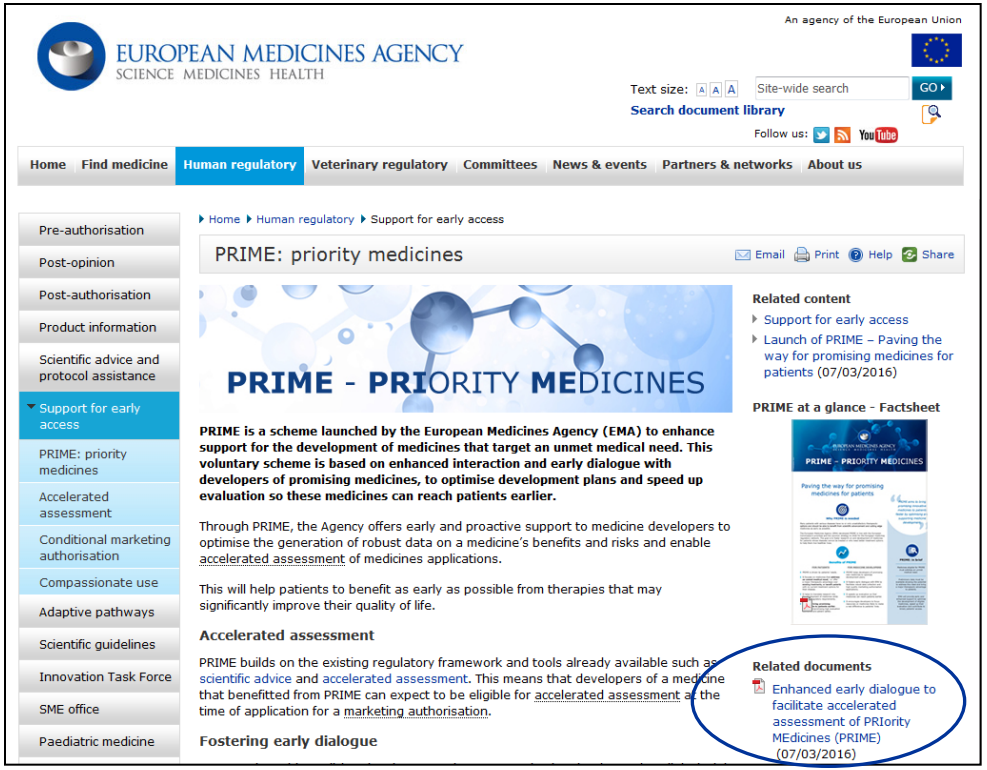
Early CHMP Rapporteur appointment

- Continuity with life-cycle approach
- Dialogue on regulatory pathway/MAA requirements
- Promote use of tools/initiatives
- Knowledge gained during development to facilitate accelerated assessment

In summary

- PRIME aims at **strengthening support** to medicines that target an unmet medical need.
- For medicines that may offer a major therapeutic advantage over existing treatments, or benefit patients with no treatment options.
- EMA will offer early, proactive and enhanced scientific and regulatory support to optimise the generation of robust data and enable accelerated assessment.
- This will allow **patients to benefit from therapies** that may significantly improve their quality of life as early as possible

PRIME webpage and supporting documents

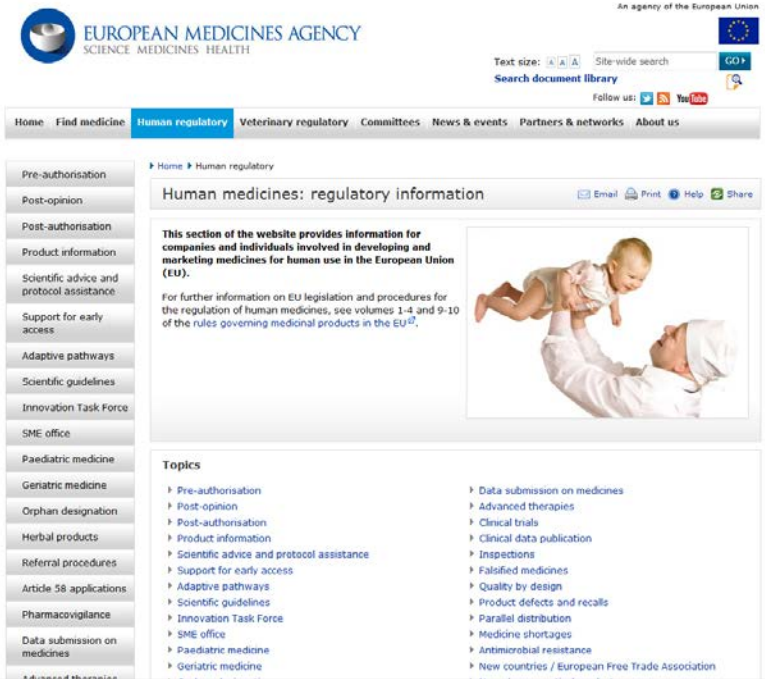


Factsheet in lay language

Q&A, templates, application form for applicants

prime@ema.europa.eu

If PRIME is not the right tool



EMA still can provide support through…

- EU innovation network
- Innovation Task Force
- Scientific Advice
- SME office
- ATMP certification
- Paediatric early interaction meetings
- Accelerated Assessment
- Pre-submission meetings

Thank you for your attention

Further information

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