



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

PRIME – update from experience

5th STAMP meeting

Presented by Sonia Ribeiro on 28 June 2016
Head of Regulatory Affairs Office
Human Medicines Research Development and Support Division

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

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.



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)

PRIME webpage and supporting documents

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Orphan designation
Herbal products

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PRIME: priority medicines

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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.

Fostering early dialogue

By engaging with medicine developers early on, PRIME is aimed at improving clinical trial designs so that the data generated is suitable for evaluating a marketing-authorisation application.

Early dialogue and scientific advice also ensure that patients only participate in trials designed to provide the data necessary for an application, making the best use of

Related content

- ▶ Support for early access
- ▶ Launch of PRIME – Paving the way for promising medicines for patients (07/03/2016)

PRIME at a glance - Factsheet

Related documents

- ▶ Enhanced early dialogue to facilitate accelerated assessment of Priority Medicines (PRIME) (07/03/2016)
- ▶ European Medicines Agency guidance for applicants seeking access to PRIME scheme (07/03/2016)
- ▶ PRIME eligibility requests:

PRIME - PRIORITY MEDICINES

Paving the way for promising medicines for patients

Why PRIME is needed

Many patients with serious diseases have no or only unsatisfactory therapeutic options and need to wait to benefit from scientific advancement and receive new medicines as early as possible.

The European Medicines Agency (EMA) developed PRIME in line with the European Commission's priorities and the mission strategy to 2020 for the European Medicines Regulatory Authority. The goal is to foster research on and development of medicines for patients whose diseases cannot be treated or who have better response options to help them live healthier lives.

Benefits of PRIME

FOR PATIENTS

- PRIME is driven by patients' needs.
- It focuses on medicines that address an unmet medical need, for either a new indication, or a new patient population, or a new formulation, or a new pathway to certain treatment options for their disease.
- It helps to identify research gaps in the development of medicines early.
- It aims to bring promising medicines to patients sooner, without any paying top-valuation threshold or patent delay.

FOR MEDICINE DEVELOPERS

- PRIME helps developers of promising medicines to understand the Agency's expectations and to align their development plans.
- It helps early medicine developers with EMA to better understand the Agency's high quality marketing authorisation requirements.
- It speeds up evaluation as PRIME medicines can reach patients earlier.
- It encourages developers to focus research on medicines that have the greatest potential to benefit patients.

PRIME: in brief

Medicine developers for PRIME must submit a letter of intent to EMA.

Medicine developers can benefit from PRIME by working to address the need and bring a major therapeutic advantage to patients.

EMA will provide early and expert support to ensure the development of single medicinal products for rare, highly innovative, and timely patient access.

**Factsheet
in lay
language**

**Q&A,
templates,
application
form for
applicants**

7 March 2016
EMA/11216/2015
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 scope and features of PRIME. It provides an overview of the procedure to obtain support through the scheme and gives guidance to companies in preparing their requests. This guidance will be updated regularly to reflect new developments as experience is gained with the scheme.

It should be read in conjunction with:

- Guidance on [enhanced early dialogue to facilitate accelerated assessment of Priority Medicines \(PRIME\)](#)
- Guidance on [accelerated assessment](#)

[European Medicines Agency: Feedback for applicants and EMA committees, advice and contact information](#)

If you require further information on any of the included 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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PRIME webpage and supporting documents

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 polyangiitis) 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.

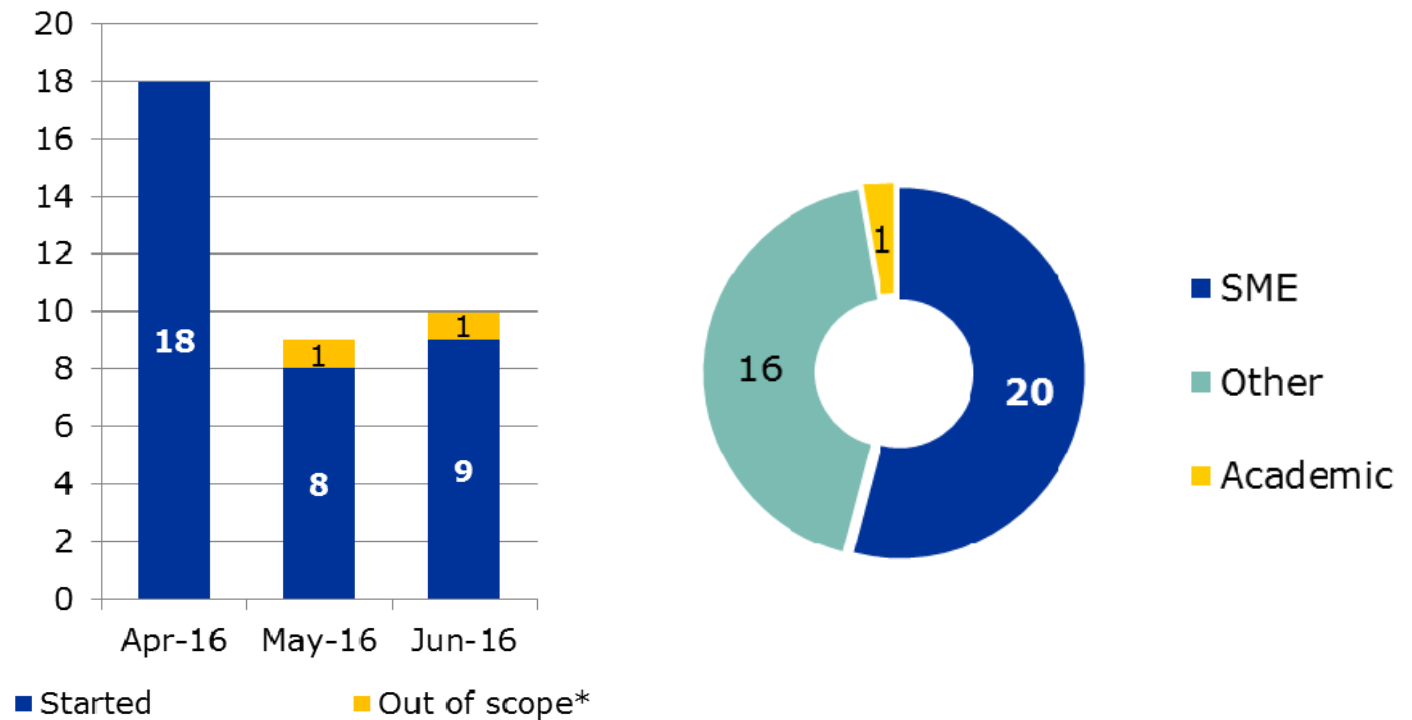
Eligibility denied

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 Nonclinical + Clinical exploratory + Clinical confirmatory	Other
Chemical	Infectious Diseases	Treatment of serious bacterial infections	Nonclinical + Clinical exploratory	SME
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	Other
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	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

- Monthly publication of recommendations on eligibility to PRIME (both granted and denied), after CHMP
- Broad characteristics
- Active substance/INN for eligible products

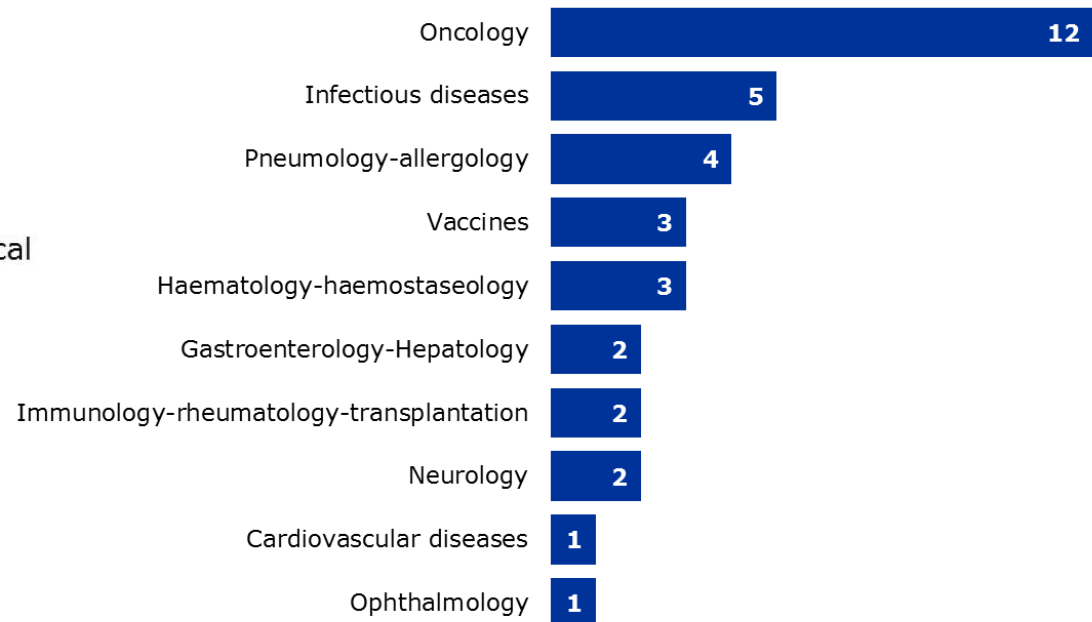
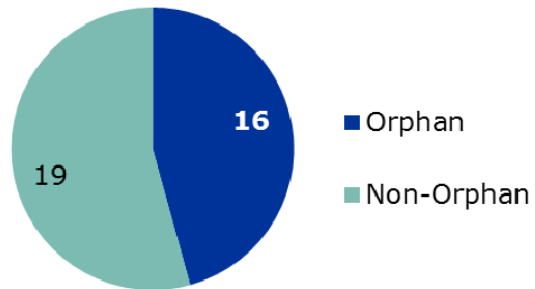
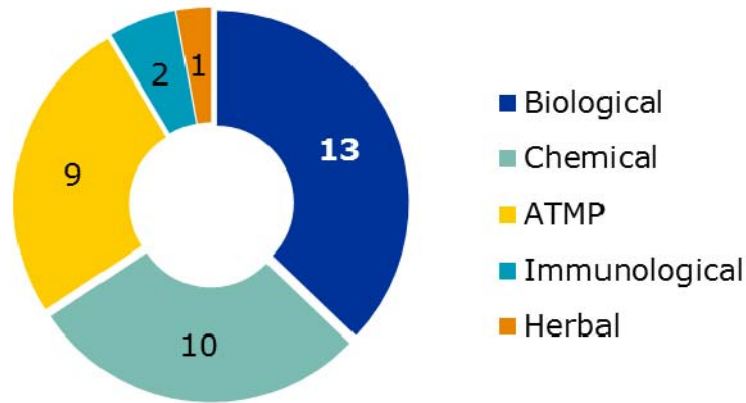


PRIME Experience





PRIME Experience

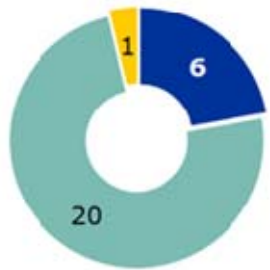




PRIME Experience

Outcomes

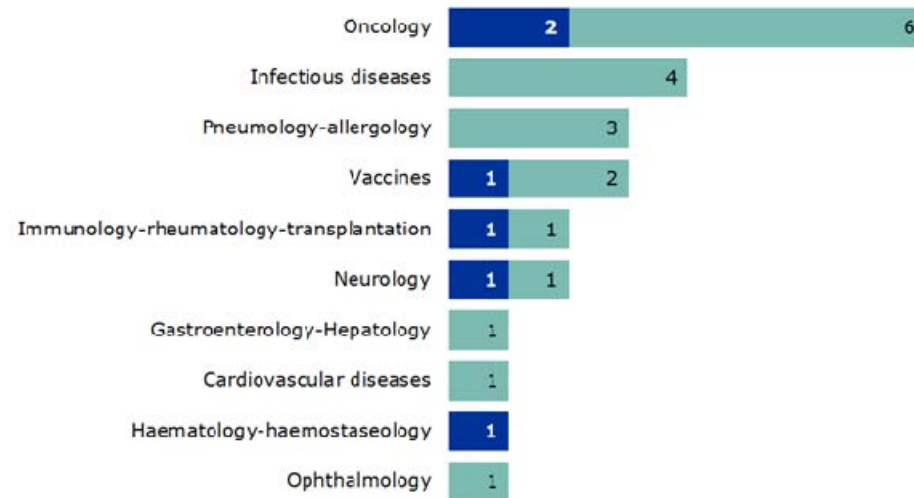
Adopted in May and June 2016



■ Granted
 ■ Denied
 ■ Out of scope*

* This indicates eligibility requests received but not started by EMA as they were deemed outside the scope of the scheme. These are not included in the breakdown by type of applicant or by therapeutic area.

By therapeutic area



By type of applicant





First 6 products granted eligibility

CCX-168

Treatment of patients with active ANCA-associated vasculitis (GPA and MPA)
Orphan

KTE-C19

ATMP
Treatment of DLBCL, PMBCL, TFL
Orphan

CTL019

ATMP
Treatment of paediatric patients with relapsed or refractory B cell acute lymphoblastic leukaemia
Orphan

Emapalumab

Treatment of primary haemophagocytic lymphohistiocytosis (HLH)
Orphan

Adacunumab

Alzheimer's disease

rVSVΔG-ZEBOV-GP, live attenuated

Vaccination against Ebola (Zaire strain)



Some reflections on PRIME experience so far

- ✓ Number of requests received confirms **high interest** from industry, particularly **SME**
- ✓ **Cross-committee collaboration** enables scrutiny from our scientific committees and oversight group to ensure consistency and discussing policy aspects of implementation.
- ✓ A number of products are in **late stage** of development -> this may be due to recent launch of the scheme.
- ✓ Next phase of the scheme: Support to applicants with **kick-off meeting** being organised.
- ✓ **HTA engagement** during development through parallel advice procedure will be of key importance.



Thank you for your attention

Further information

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