EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

PRIME – update from experience

5th STAMP meeting

Presented by Sonia Ribeiro on 28 June 2016
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Human Medicines Research Development and Support Division

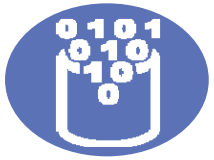
EUROPEAN MEDICINES AGENCY

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

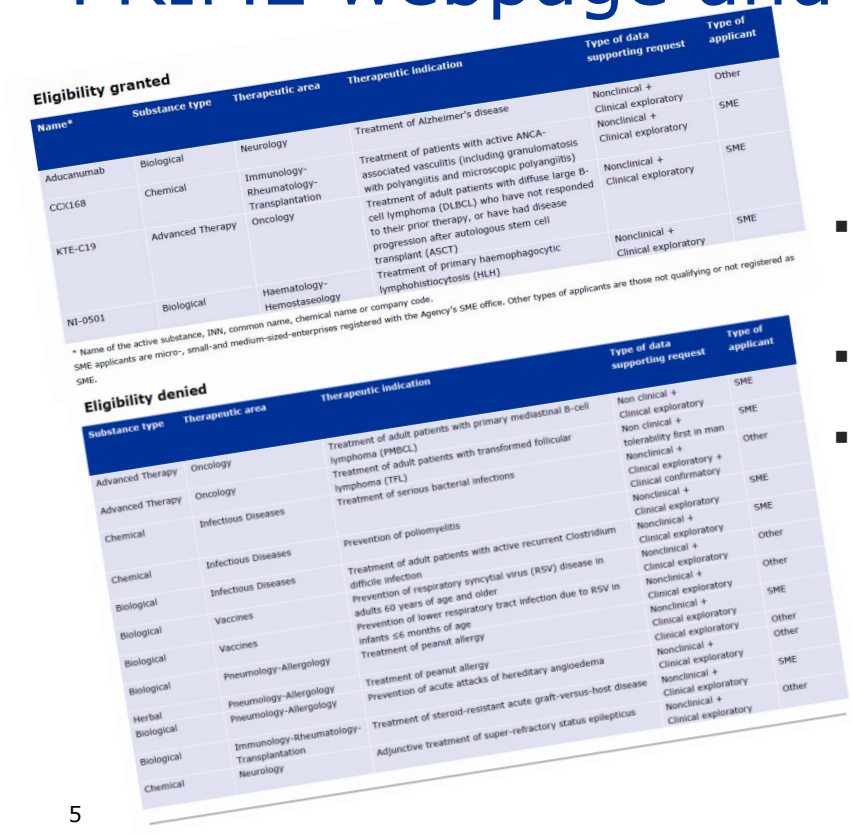
Meaningful improvement of efficacy (impact on onset, duration, improving morbidity, mortality)

PRIME webpage and supporting documents

Factsheet in lay language

Q&A, templates, application form for applicants

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 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

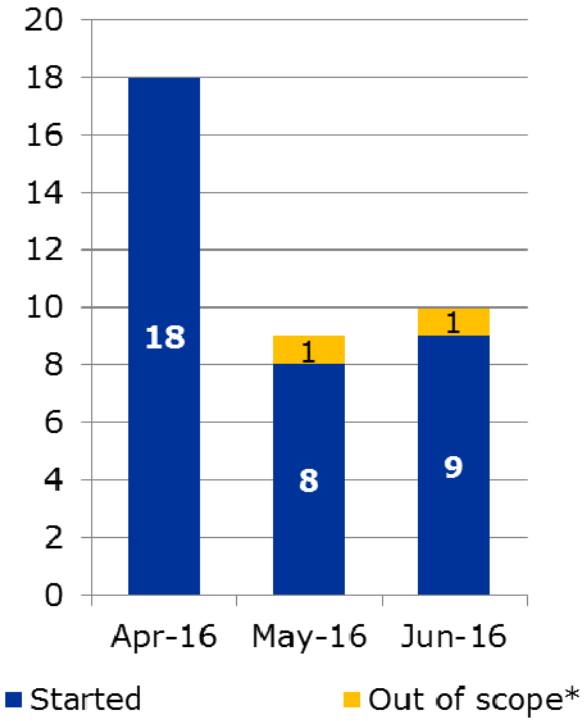
* Name of the active substance, INN, common name, chemical name or company code.
SME applicants are micro-, small-and medium-sized-enterprises registered with the Agency's SME office. Other types of applicants are those not qualifying or not registered as SME.

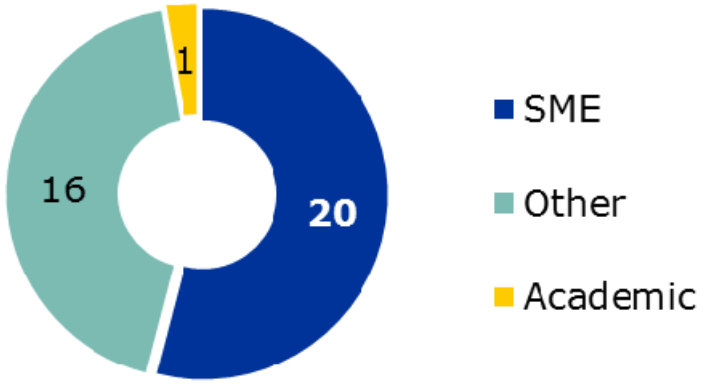
Eligibility denied

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
Herbal	Pneumology-Allergology	Treatment of peanut allergy	Nonclinical + Clinical exploratory	Other
Biological	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

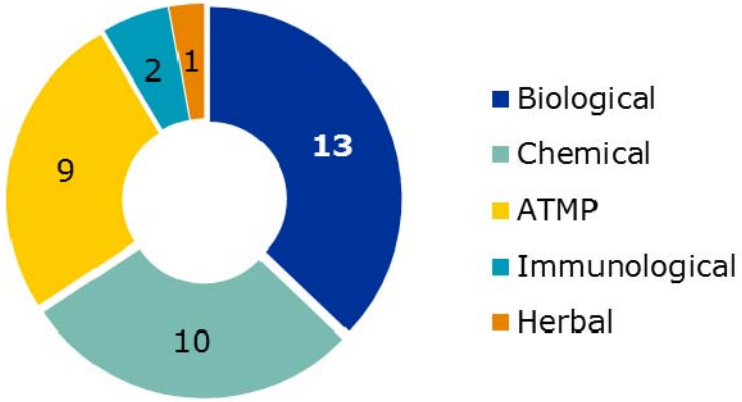
PRIME Experience

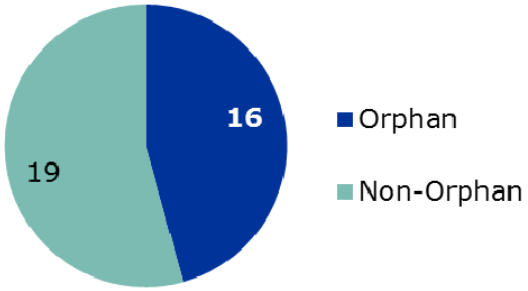


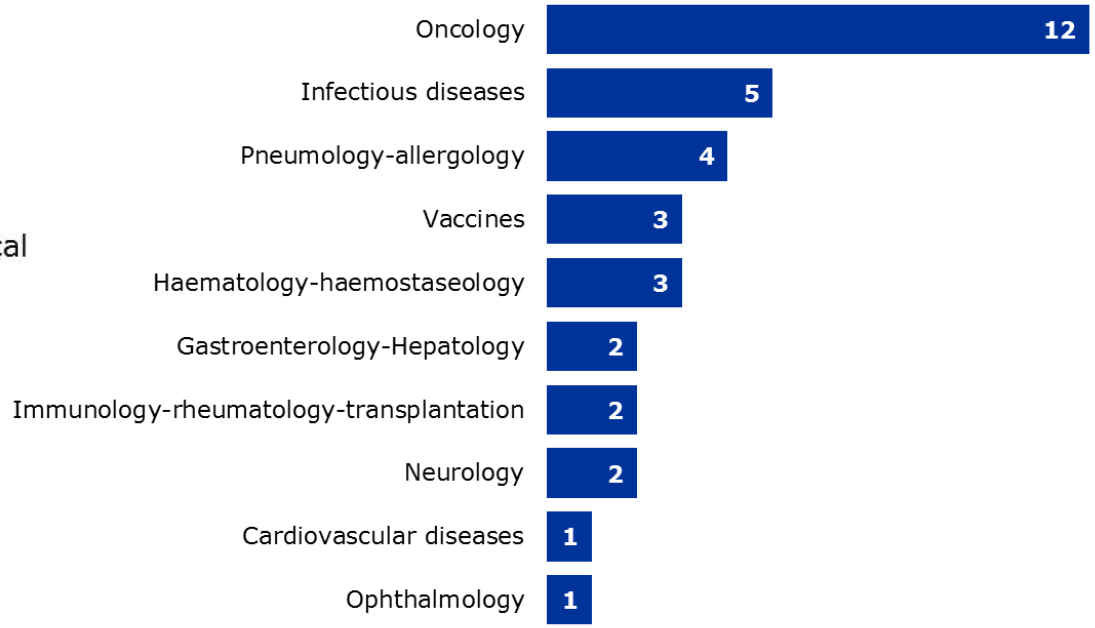


* Requests received but not started by EMA as they were deemed outside the scope of the scheme

PRIME Experience





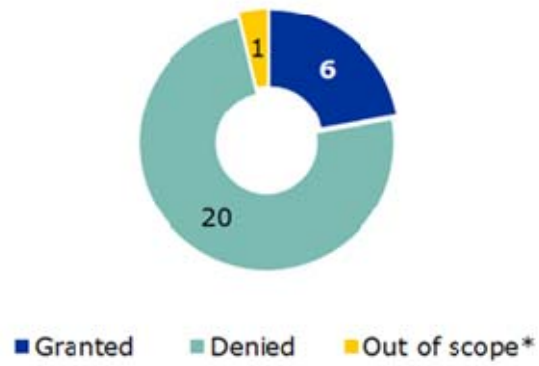


Data excluding out of scope applications

PRIME Experience

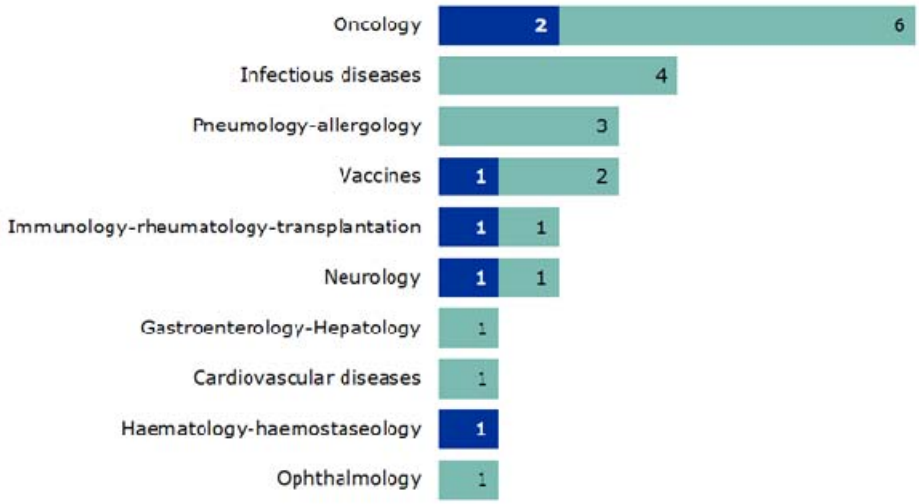
Outcomes

Adopted in May and June 2016



* 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.

EUROPEAN MEDICINES AGENCY

Thank you for your attention

Further information

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