EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

PRIME: One year experience and highlights from 19 May Stakeholder meeting

PCWP/HCPWP June 2017

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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.

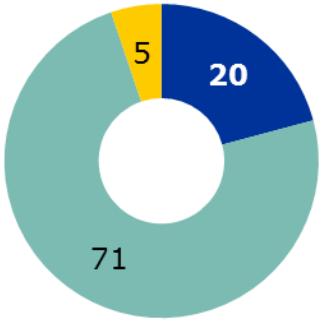
- Potential to address to a significant extent **an unmet medical need**
- Scientific justification, based on data and evidence available from nonclinical and clinical development

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)

One year of PRIME



■ Granted ■ Denied ■ Out of scope*

22 % success rate

108 requests received

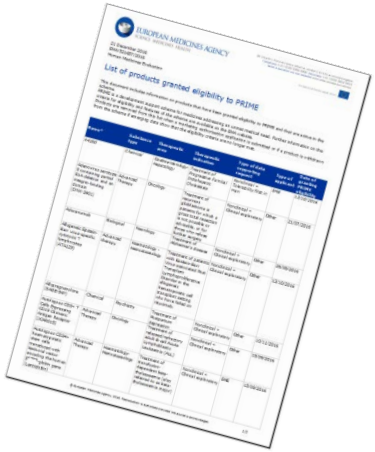
> 90 eligibility requests assessed

> 50 % from SMEs

20 granted*

+

Publication of report and list of products on EMA website



Requests covering wide range of therapeutic areas and product type

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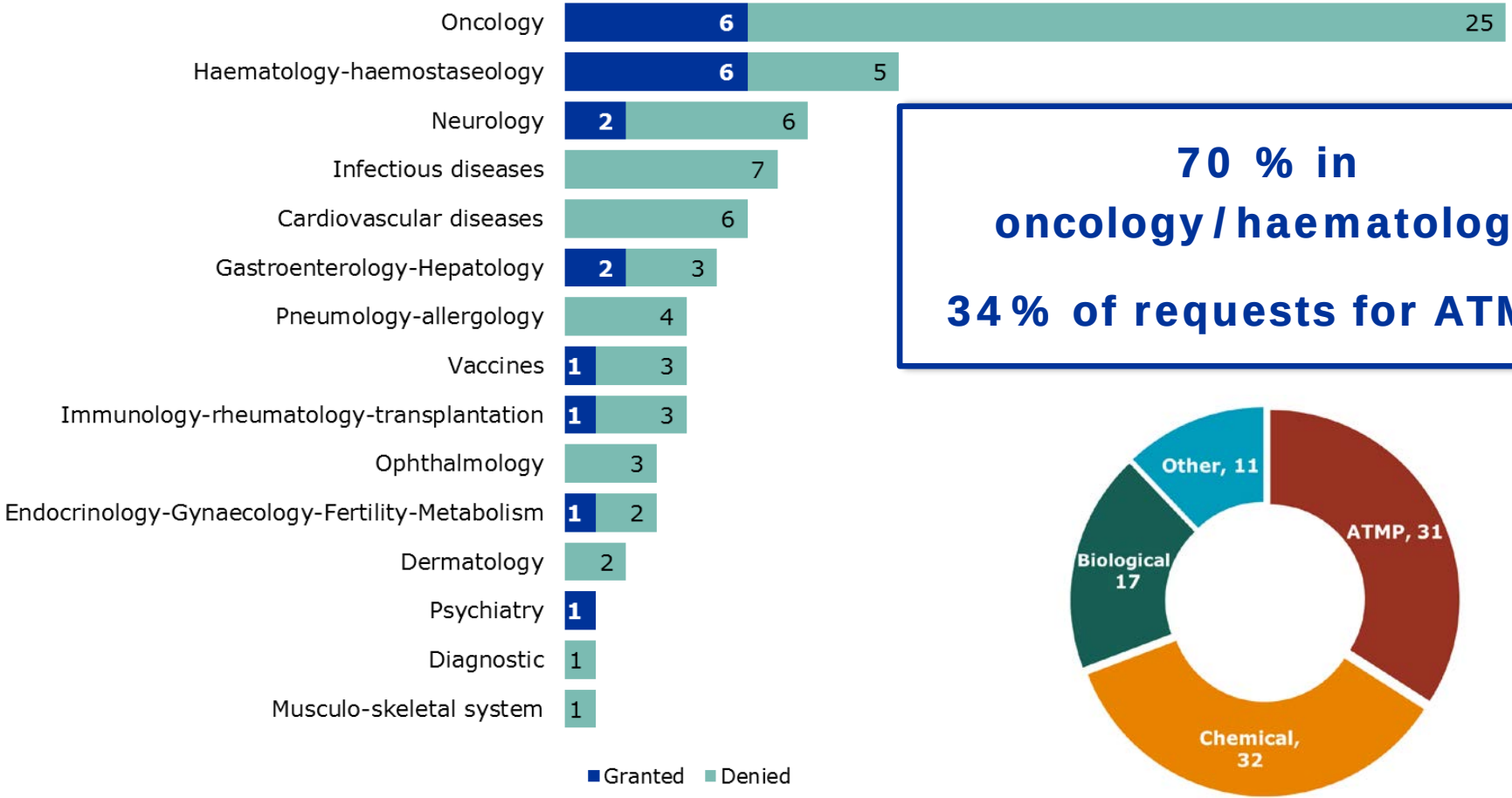


70 % in oncology / haematology

34 % of requests for ATMPs

What do we expect to grant eligibility?

Unmet medical need
No treatment, or clear limitations of existing therapies (e.g. Alzheimer's disease)

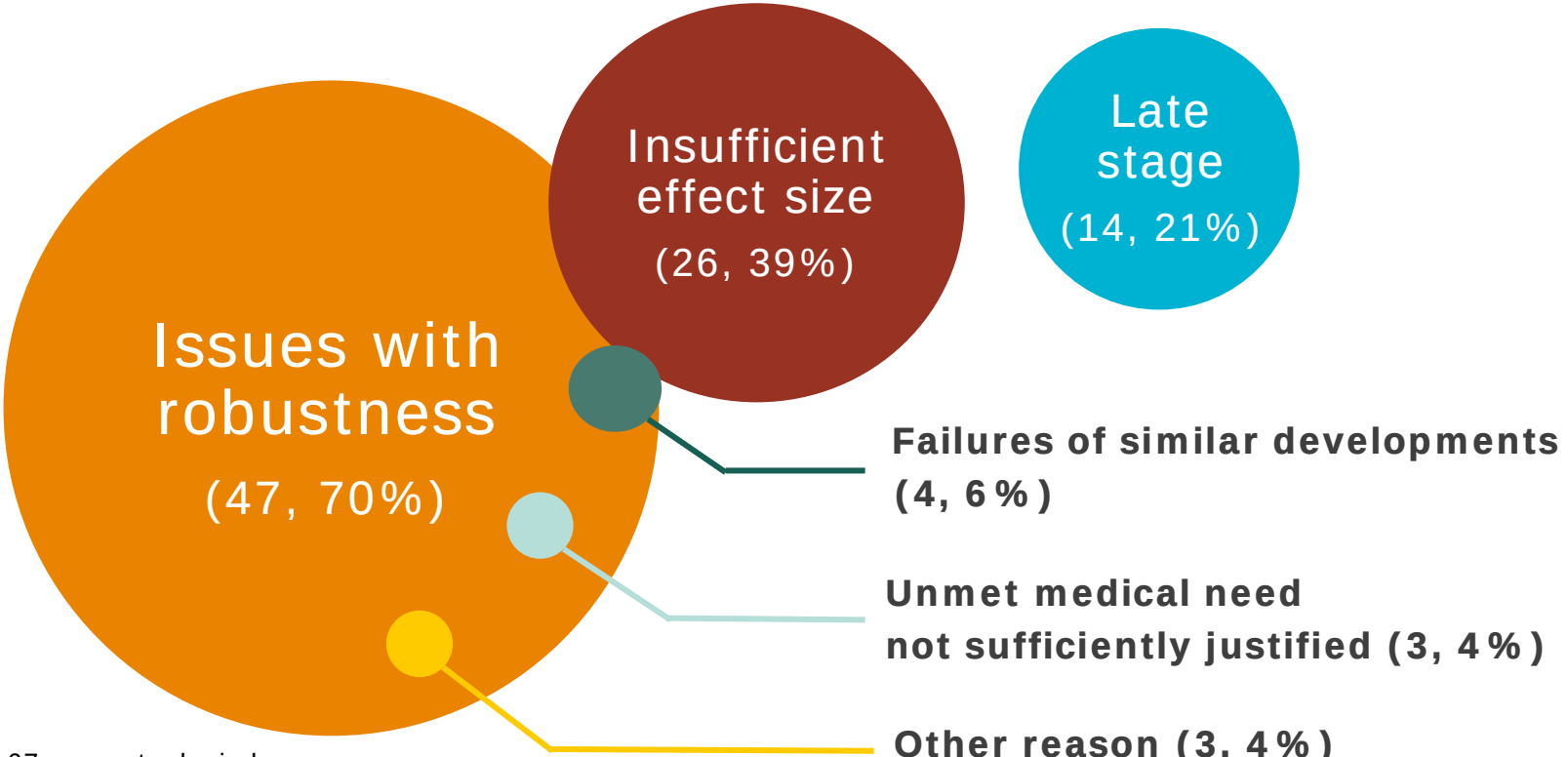
Nonclinical data supporting pharmacological rationale (e.g. gene therapy)

Clinical exploratory data on **relevant endpoint**

If uncontrolled, use **comparable historical control**
i.e. need sufficient information on baseline characteristics

Magnitude of the effect size supporting major therapeutic advantage

Reasons for denial at proof of concept stage



N=67 requests denied

Reasons for denial at proof of concept stage: Examples of robustness issues

Trial design issues eg treatment effect not isolated from other factors, use of concomitant treatments

Failed study

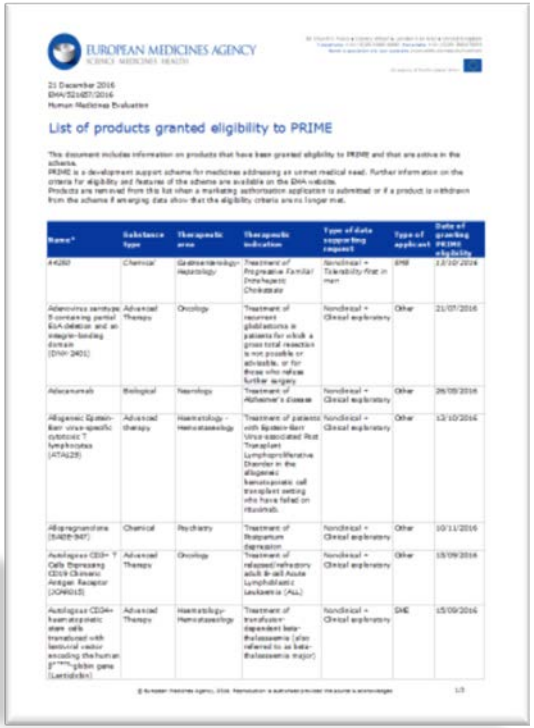
Inconsistency of results
across studies, study groups or endpoints

Claim in subgroup insufficiently justified

Sample issues
size, heterogeneity, insufficient information on baseline

Comparison to inadequate historical control data

Now 25 PRIME products



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21 December 2016
EMA/521407/2016
Human Medicines Evaluation

List of products granted eligibility to PRIME

This document includes information on products that have been granted eligibility to PRIME and that are active in the scheme.
PRIME is a development support scheme for medicines addressing an unmet medical need. Further information on the criteria for eligibility and features of the scheme are available on the EMA website.
Products are removed from this list when a marketing authorisation application is submitted or if a product is withdrawn from the scheme if emerging data show that the eligibility criteria are no longer met.

Name*	Substance type	Therapeutic area	Therapeutic indication	Type of data supporting request	Type of applicant	Date of granting PRIME eligibility
A4250	Chemical	Gastroenterology-Hepatology	Treatment of Progressive Familial Intrahepatic Cholestasis	Nonclinical + Tolerability first in man	EMS	13/10/2016
Adenovirus serotype 5 containing partial E1A deletion and an integrin-binding domain (DNX-2401)	Advanced Therapy	Oncology	Treatment of recurrent glioblastoma in patients for which a gross total resection is not possible or advisable, or for those who refuse further surgery	Nonclinical + Clinical exploratory	Other	21/07/2016
Aducanumab	Biological	Neurology	Treatment of Alzheimer's disease	Nonclinical + Clinical exploratory	Other	26/05/2016
Allogeneic Epstein-Barr virus-specific cytotoxic T lymphocytes (ATA129)	Advanced therapy	Haematology - Haemostaseology	Treatment of patients with Epstein-Barr Virus-associated Post Transplant Lymphoproliferative Disorder in the allogeneic haematopoietic cell transplant setting who have failed on rituximab.	Nonclinical + Clinical exploratory	Other	12/10/2016
Allopregnanolone (SAGE-547)	Chemical	Psychiatry	Treatment of Refractory Status Epilepticus	Nonclinical + Clinical exploratory	Other	10/11/2016
Autologous CD3+ T Cells Expressing CD19 Chimeric Antigen Receptor (JCAR015)	Advanced Therapy	Oncology	Treatment of relapsed/refractory adult B-cell Acute Lymphoblastic Leukaemia (ALL)	Nonclinical + Clinical exploratory	Other	15/09/2016
Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human β^{A-T87Q}-globin gene (Lentiglobin)	Advanced Therapy	Haematology-Haemostaseology	Treatment of transfusion-dependent beta-thalassaemia (also referred to as beta-thalassaemia major)	Nonclinical + Clinical exploratory	SME	15/09/2016

- Some very innovative products with several advanced therapy medicines
- Across therapeutic areas, including rare cancers, Alzheimer's disease
- Most in rare diseases

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.

Enhanced scientific advice

7 products
11 SA requests
following kick-off meetings

Multi-stakeholder
1 EMA/HTA parallel advice
2 with patients involved

Rapporteur involvement
through one of SAWP coordinator

All aspects covered
Quality,
nonclinical, clinical

Flexibility
Shorter pre-submission
3 adopted in 40 days

First anniversary meeting on 19 May

Very positive feedback

A few areas for improvement identified:

- Guidance on eligibility
- Interactions with the Rapporteur
- Regular updates from applicants

Opportunity for further involvement of patients and HTA in scientific advice

In summary,

Eligibility review: robust, short time, in writing

Rapporteur appointment enables
early identification of potential issues

Scheme triggers discussions across product type / class

Excellent collaboration across committees

Iterative scientific advices with opportunity for patients' involvement

Thank you for your attention

Further information

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