EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

HCPWP/PCWP feedback from CHMP

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28 June 2017

Summary

- CHMP opinions
  - New medicines (March - May 2017)
  - Scientific Advices/Protocol Assistance (March – May 2017)
  - PRIME eligibility (March – May 2017)
- HCP/Patients input provided in the context of CHMP activities

Positive opinion on new active substances – March – May 2017

Cancer

Name	Active S	Indication	
Axumin	fluciclovine (18F)	Diagnostic agent for the detection of recurrence of prostate cancer with positron emission tomography (PET) imaging	EC
Besponsa	inotuzumab ozogamicin	Treatment of acute lymphoblastic leukaemia	O Rx EC

EC Authorised EC EC decision pending monitored (supervision) HCP

C conditional marketing authorisation O orphan Rx Restricted prescription ▼ additional monitoring

Positive opinion on new active substances – March – May 2017

Haematology

Name	Active S	Indication	
Refixia	nonacog beta pegol	Treatment of haemophilia B	O Rx EC

EC Authorised EC EC decision pending monitored (supervision) HCP

C conditional marketing authorisation O orphan Rx Restricted prescription ▼ additional monitoring

Positive opinion on new active substances – March – May 2017

Vaccine

Name	Active S	Indication	
Trumenba	meningococcal group B vaccine (recombinant, adsorbed)	Prophylaxis against invasive meningococcal disease caused by meningococcal serogroup B bacteria	▼ EC

EC Authorised EC EC decision pending monitored (supervision) HCP

C conditional marketing authorisation O orphan Rx Restricted prescription ▼ additional monitoring

Positive opinion on new active substances – March – May 2017

Neurology

Name	Active S	Indication
Brineura	cerliponase alfa	Treatment of neuronal ceroid lipofuscinosis type 2 (CLN2) disease

O Rx 

C EC

Press release: New medicine for rare neurodegenerative disorder in children

Name	Active S	Indication
Spinraza	nusinersen	Treatment of spinal muscular atrophy

O Rx 

EC

Press release: First medicine for spinal muscular atrophy

Positive opinion on new active substances – March – May 2017

Psychiatry

Name	Active S	Indication	
Reagila	cariprazine	Treatment of schizophrenia	EC

Positive opinion on new active substances – March – May 2017

Immuno-system

Name	Active S	Indication		
Kevzara	sarilumab	Treatment of rheumatoid arthritis	Rx EC	
Kyntheum	brodalumab	Treatment of moderate to severe plaque psoriasis	Rx	

Positive opinion on new active substances – March – May 2017

Ophthalmology

Name	Active S	Indication
Oxervate	cenegermin	Treatment of moderate to severe neurotrophic keratitis

EC

Metabolism

Name	Active S	Indication
Veltassa	patiromer	Treatment of hyperkalaemia

EC

Positive opinion on new active substances – March – May 2017

Bone defect

Advanced Therapy

Name	Active S	Indication
Spherox	spheroids of human autologous matrix-associated chondrocytes	Repair of certain cartilage defects of the knee

EC

Press release: New advanced therapy to repair cartilage defects in the knee

Negative opinions – March – May 2017

Cancer

Name	Active S	Indication
Adlumiz	anamorelin hydrochloride	Treatment of anorexia, cachexia or unintended weight loss in patients with non-small cell lung cancer

- Studies proof marginal efficacy and no effect on patients' quality of life.
- Following an inspection at clinical study sites - safety data had not been recorded adequately - evaluation of potential risks with Adlumiz was not possible.

Negative opinions – March – May 2017

Cancer

Name	Active S	Indication
Human IgG1 monoclonal antibody specific for human interleukin-1 alpha XBiotech	human IgG1 monoclonal antibody specific for human interleukin-1 alpha	Treatment of advanced colorectal cancer

- Did not show clear improvements in either lean body mass or quality of life.
- Increased risk of infection in patients taking the medicine, which was not considered acceptable in vulnerable patients receiving palliative care.
- Inadequate controls of the manufacturing process to ensure the medicine would have the same quality as the product used in clinical trials.

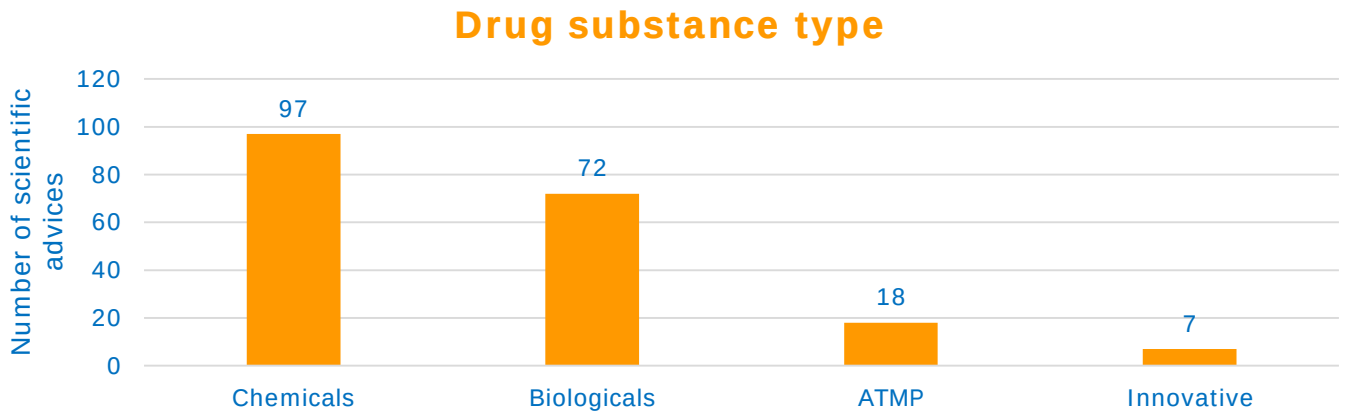
Negative opinions – March – May 2017

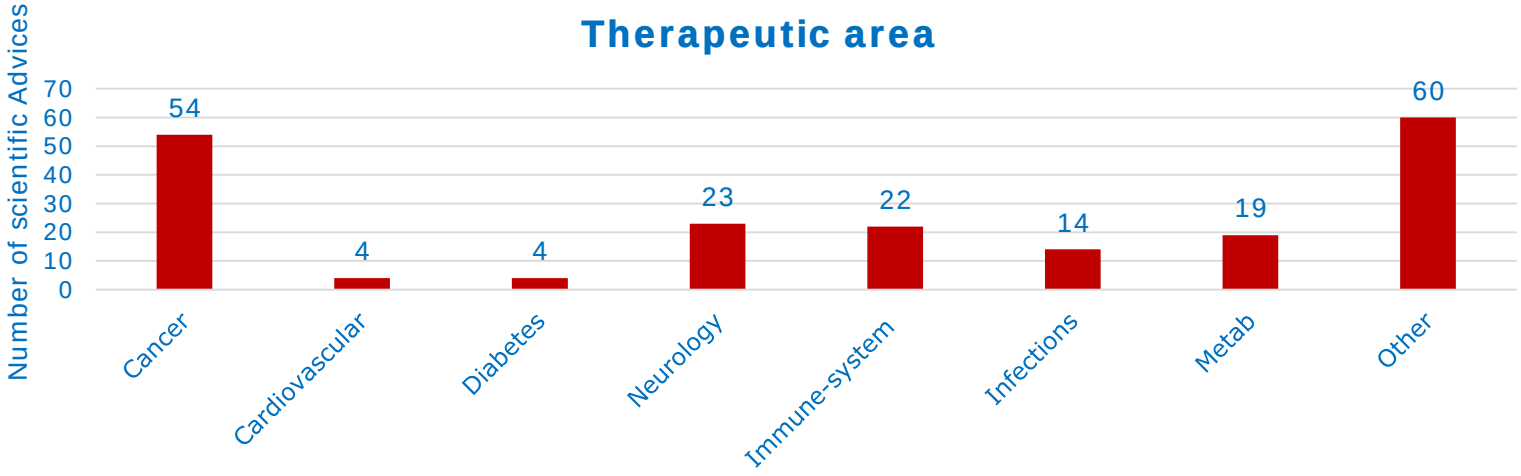
Haematology

Name	Active S	Indication
Masipro	masitinib	Treatment of systemic mastocytosis

- Questioned reliability of the study results due to serious fails observed during a routine GCP (good clinical practice) inspection at the study sites.
- Major changes were made to the study design while the study was ongoing, which made the results difficult to interpret.
- Limited safety data of the medicine with concerns regarding the adverse effects: neutropenia (low levels of white blood cells) and harmful effects on the skin and liver, of relevance particularly because the medicine was to be used long term.

Scientific Advice (January- May 2017)

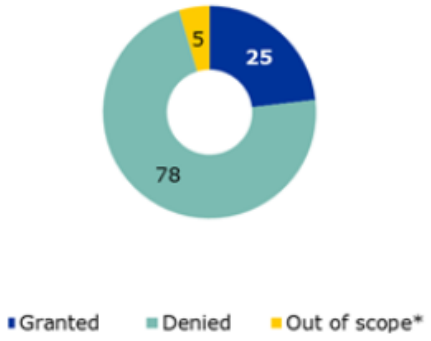


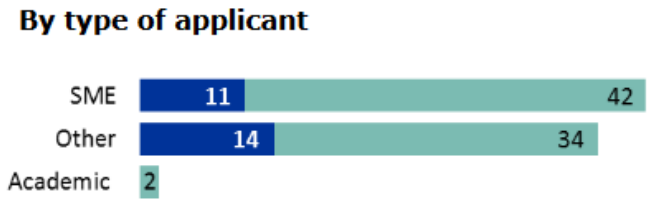


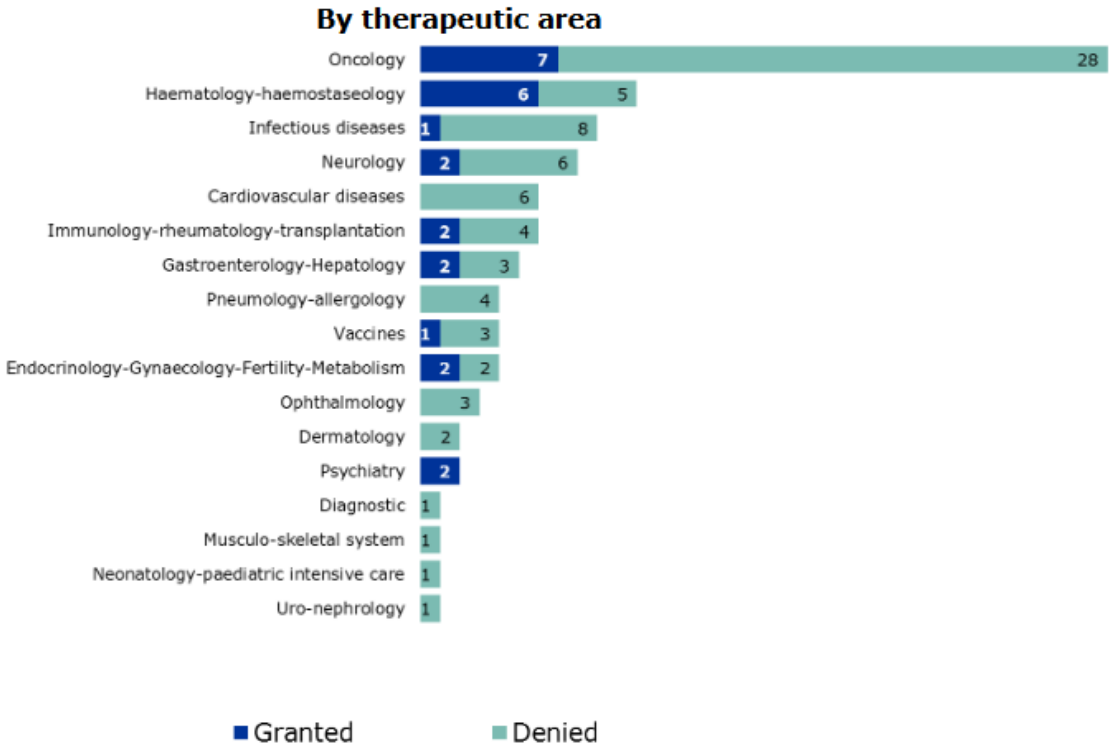
PRIME

- Enhance support for the development of medicines that target an unmet medical need.
- Interaction and early dialogue with developers of promising medicines, to optimise development plans and speed up evaluation so these medicines can reach patients earlier.
- Built on the existing regulatory framework and tools already available such as scientific advice and accelerated assessment.
- Improving clinical trial designs - data generated suitable for evaluating a MAA
- Patients only participate in trials designed to provide the data necessary for an application - best use of limited resources.

PRIME eligibility up to 18 May 2017







* One eligible product has subsequently been withdrawn from the scheme at the applicant's request
Out of scope indicates eligibility requests received but not started by EMA as they were deemed outside the scope of the scheme or with a format and content inadequate to support their review. These are not included in the breakdown by type of applicant or by therapeutic area.

PRIME eligibility – March – May 2017

Name	Substance type	Therapeutic área	Therapeutic indication	Data of eligibility granted
Adeno-associated viral vector serotype 5 containing human factor IX gene (AMT-060)	Advanced therapy	Haematology - Hemostaserology	Treatment of severe haemophilia B	04-2017
Asunercept	Biological	Oncology	Treatment of glioblastoma	05-2017
Olipudase alfa	Biological	Endocrinology - Gynaecology - Fertility - Metabolism	Treatment of non-neurological manifestations of acid sphingomyelinase deficiency	05-2017
Rapastinel	Chemical	Psychiatry	Adjunctive treatment of major depressive disorder	05-2017

PRIME eligibility – March – May 2017

Name	Substance type	Therapeutic área	Therapeutic indication	Data of eligibility granted
Synthetic 47-amino-acid N-myristoylated lipopeptide, derived from the preS region of hepatitis B virus	Chemical	Infectious Diseases	Treatment of chronic hepatitis D infection	05-2017
Recombinant IgG degrading enzyme of Streptococcus pyogenes	Biological	Immunology - Rheumatology - Transplantation	Prevention of graft rejection following solid organ transplantation)	05-2017

Interaction between CHMP and HCP - Participation in Scientific Advisory Groups and ad-hoc Experts Groups

- **Contributing for decision on recomendations**

Name	Active S	Indication
Refixia	nonacog beta pegol	Treatment of haemophilia B
Brineura	cerliponase alfa	Treatment of neuronal ceroid lipofuscinosis type 2 (CLN2) disease
Reagila	cariprazine	Treatment of schizophrenia

Interaction between CHMP and Patient's representatives - Participation in CHMP plenary sessions

➢ Contributing for decision on recomendations (Adlumiz)

Name	Active S	Indication
Adlumiz	anamorelin hydrochloride	Treatment of anorexia, cachexia or unintended weight loss in patients with non-small cell lung cancer

- **Patients' participation at the oral explanation for Adlumiz was perceived very positive by CHMP members - patients' view major help for CHMP's deliberations.**
- **Adlumiz was a classical benefit-risk discussion, with the possibility that Adlumiz may help (a little) in a few patients, with quite some uncertainty on the safety side.**
- **It was important for the Committee to hear from patients a clear "for this product, we do not want to be given the choice".**