EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

HCPWP feedback from CHMP

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Summary

- CHMP opinions (overview Oct 2017 – Mar 2018)
  - New medicines
  - Scientific Advices/Protocol Assistance
  - PRIME eligibility
- HCP/P input provided in the context of CHMP activities

Positive opinion on new medicines – Oct 17 – Mar 18

Name	DS	Indication	
Adynovi	rurioctocog alfa pegol	Haemophilia A	EC ▼ Rx
Ad hoc expert group (included 2 Patients' representatives) Post-authorisation safety study (PASS): In order to investigate potential effects - Q1 2019			
Hemlibra	emicizumab	Haemophilia A - factor VIII inhibitors	EC ▼ Rx
EP / EM - Physician, Patient/Carer, and Laboratory professionals educational material			
Intrarosa	prasterone	Vulvar and vaginal atrophy in postmenopausal women	EC ▼ Rx
Crysvita	burosumab	X-linked hypophosphataemia C	EC O ▼ Rx
Lamzede	velmanase alfa	non-neurological manifestations of mild to moderate alpha-mannosidosis E	EC O ▼ Rx
Ad hoc expert group (included 2 Patients' representatives) **Post-MA**: Long-term data on effectiveness and safety data from a registry of patients + Annual reports/ annual re-assessment + Paediatric Study - Final Study report: November 2020			

Positive opinion on new medicines – Oct 17 – Mar 18

Name	DS	Indication	
Fasenra	benralizumab	Severe eosinophilic asthma	EC ▼ Rx
Jorveza	budesonide	Eosinophilic esophagitis	EC Rx
Alofisel	darvadstrocel	Complex perianal fistulas in patients with Crohn's disease	EC ▼ Rx
EP / EM - Guide for pharmacists with instructions on the appropriate reception and storage. Guide (video) for surgeons and other HCP involved in the preparation and administration. Guide for surgeons and other HCP describing the method of administration and providing information on potential for microbial information and advice on steps to follow in case a positive culture is identified. Relevant information on the risk of medication errors and the potential for transmission of infectious agents and details on how to minimise these, including reception, storage and administration instructions (i.e. fistula conditioning, preparation and injection). **Post-MA**: To follow-up on the efficacy of Alofisel, the MAH should submit the results of a Phase III randomised double-blind, placebo-controlled study investigating a single administration - 2Q/3Q 2022			
Ocrevus	ocrelizumab	Relapsing forms of MS and primary progressive MS	EC ▼ Rx
SAG (included 2 Patients' representatives)			

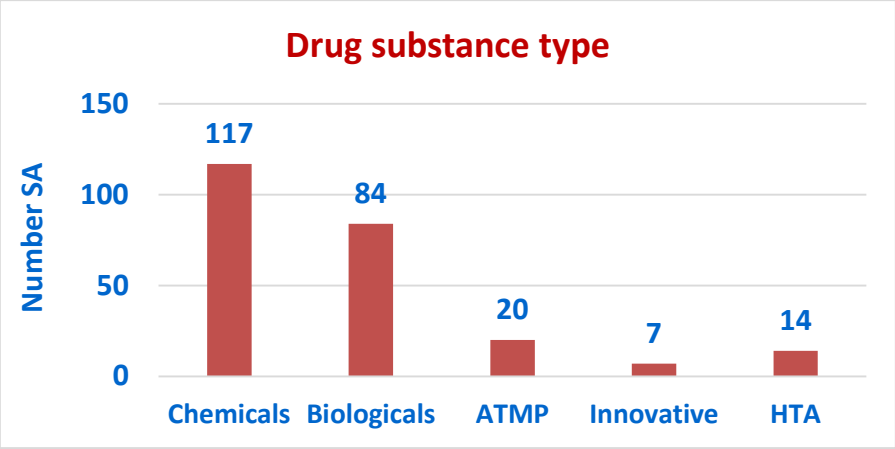
Positive opinion on new medicines – Oct 17 – Mar 18

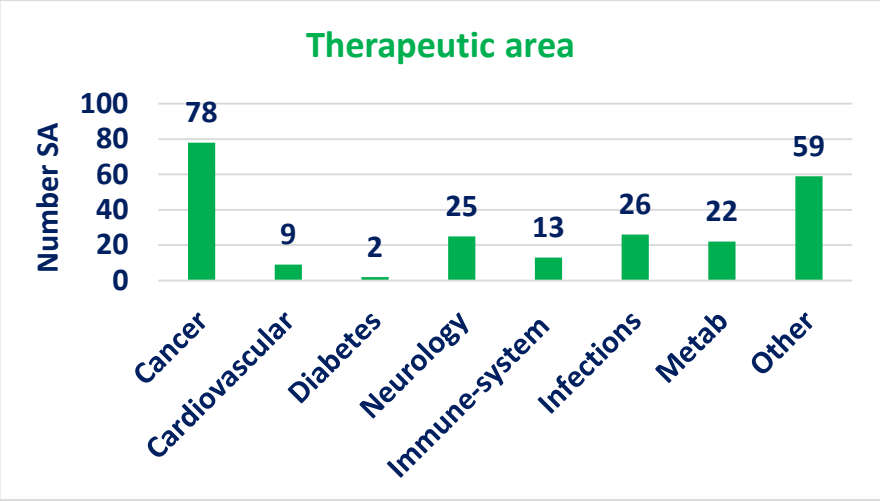
Name	DS	Indication	
Ozempic	semaglutide	Type 2 diabetes	EC ▼
Steglatro	ertugliflozin	Type 2 diabetes	EC ▼
Segluromet	ertugliflozin / metformin	Type 2 diabetes	EC ▼
Steglujan	ertugliflozin / sitagliptin	Type 2 diabetes	EC ▼
Amglidia	glibenclamide	Neonatal diabetes	EC  ▼
Measure to minimise medication errors - Number of presentations for the two different dose strengths and syringes **EM** - Visual educational material for physicians and pharmacists 			
Alkindi	hydrocortisone	Replacement therapy of adrenal insufficiency in infants, children and adolescents	EC

Positive opinion on new medicines – Oct 17 – Mar 18

Name	DS	Indication	
Prevymis	letermovir	Prophylaxis of CMV reactivation and disease in adult CMV-seropositive recipients of HSCT	EC    
Post-MA: In order to optimise the sterility assurance level implement the measures concerning development, validation and introduction of terminal sterilisation.			
Shingrix	herpes zoster vaccine (recombinant, adjuvanted)	Prevention of herpes zoster (HZ) and post-herpetic neuralgia (PHN), in adults > 50 years	EC 
Alpivab	peramivir	Uncomplicated influenza	EC
Juluca	dolutegravir / rilpivirine	HIV infection	EC  
Mylotarg	gemtuzumab ozogamicin	Acute myeloid leukaemia	EC   
Rubraca	rucaparib	Relapsed or progressive ovarian cancer 	EC  
SAG (included 2 Patients' representatives)			

Scientific Advice / Protocol assis. (Oct 17 – Mar 18)





PRIME eligibility – Oct 17 – Mar 2018

Name	Substance type	Therapeutic área	Therapeutic indication	Data of eligibility granted
Entrectinib	Chemical	Oncology	Treatment of NTRK fusion-positive, locally advanced or metastatic solid tumours in adult and paediatric patients who have either progressed following prior therapies or who have no acceptable standard therapy	10-2017
Humanized antibody targeting B cell maturation antigen conjugated with maleimidocaproyl monomethyl auristatin F	Biological	Oncology	Treatment of relapsed and refractory multiple myeloma patients whose prior therapy included a proteasome inhibitor, an immunomodulatory agent and an anti-CD38 antibody	10-2017
Human immunoglobulin G1 constant region - human ectodysplasin-A1 receptor-binding domain fusion protein	Biological	Dermatology	Treatment of X-linked hypohidrotic ectodermal dysplasia	10-2017

PRIME eligibility – Oct 17 – Mar 2018

Name	Substance type	Therapeutic área	Therapeutic indication	Data of eligibility granted
Autologous T lymphocyte-enriched population of cells transduced with a lentiviral vector encoding a chimeric antigen receptor targeting human B cell maturation antigen with 4-1BB and CD3-zeta intracellular signalling domains (bb2121)	Advanced therapy	Oncology	Treatment of relapsed and refractory multiple myeloma patients whose prior therapy included a proteasome inhibitor, an immunomodulatory agent and an anti-CD38 antibody	11-2017
LR12	Chemical	Infectious Diseases	Treatment of Septic Shock	11-2017
Recombinant humanised monoclonal IgG2 lambda antibody against human sclerostin (BPS804)	Biological	Other	Treatment of osteogenesis imperfecta types I, III and IV	11-2017

PRIME eligibility – Oct 17 – Mar 2018

Name	Substance type	Therapeutic área	Therapeutic indication	Data of eligibility granted
Adenovirus associated viral vector serotype 8 containing the human CNGB3 gene (AAV2/8-hCARp.hCNGB3)	Advanced therapy	Oncology	Treatment of achromatopsia associated with defects in CNGB3	02-2018
Lumasiran	Chemical	Uro-nephrology	Treatment of Primary Hyperoxaluria Type 1	03-20178

Interaction between CHMP & HCP & Patients

Name	Indication	Consultation	Outcome
Adynovi	haemophilia A	Ad-Hoc (2 Pts)	√
Ocrevus	multiple sclerosis	SAG (2 Pts)	√
Alcover (A-32)	alcohol dependence	Ad-Hoc	X
Fanaptum	schizophrenia	Ad-Hoc (2 Pts)	X
Lamzede	alpha-mannosidosis	Ad-Hoc (2 Pts)	√
Nerlynx	early breast cancer	SAG (2 Pts)	X
Onzeald	advanced breast cancer	SAG (2 Pts)	X
Opdivo (ext. ind.)	met. colorectal cancer	SAG (2 Pts)	**Withdrawn**
Rubraca	prog. ovarian cancer	SAG (2 Pts)	√
Raxone (ext. ind.; after re-exm)	Duchenne muscular dystrophy	SAG (2 Pts)/CHMP	X
Eladynos	osteoporosis	Ad-Hoc (2 Pts)	X
Aplidin (after re-exam)	multiple myeloma	SAG (2 Pts)	X