



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

HCPWP/PCWP feedback from CHMP

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An agency of the European Union






Summary (October 2019 – February 2020)

- CHMP opinions
 - New medicines (non-exhaustive list)
 - Community Reviews
 - PRIME eligibility
- HCP/Patients input provided in the context of CHMP activities



Positive opinions – October 2019 – February 2020

EC Authorised **EC** EC decision pending  monitored (supervision) HCP **A** Accelerated assessment

C conditional marketing authorisation **O** orphan **R_X** Restricted prescription ▼ additional monitoring

E Exceptional circumstances **EM** Educational Materials **R_X** Special Restricted prescription



Positive opinions – October 2019 – February 2020

Diabetes

Name	Active S	Indication
Baqsimi (Oct/19)	glucagon	Treatment of severe hypoglycaemia

EC EM

Press release: First non-injectable treatment for severe low blood sugar levels

Name	Active S	Indication
Rybelsus (Jan/20)	semaglutide	Treatment of type 2 diabetes



EC

Press release: First oral glucagon-like peptide 1 treatment for type 2 diabetes

Metabolism

Name	Active S	Indication
Givlaari (Jan/20)	givosiran	Treatment of acute hepatic porphyria



EC



Press release: First treatment for acute hepatic porphyria

PRIME



Positive opinions – October 2019 – February 2020

Infectious diseases

Name	Active S	Indication
Ervebo (Oct/19)	Ebola Zaire Vaccine (rVSVΔG-ZEBOV-GP, live)	Prophylaxis against Zaire Ebola virus disease



EC



Rx



Press release: First vaccine to protect against Ebola

Name	Active S	Indication
Vaxchora (Jan/20)	Cholera vaccine (recombinant, live, oral)	Prophylaxis against disease caused by Vibrio cholerae in adults and children

EC




EM





Positive opinions – October 2019 – February 2020

Neurology

Name	Active S	Indication	EC	EM	Rx
Spravato (Oct/19)	esketamine	Combination treatment in adults with treatment-resistant major depressive disorder	EC	▼ EM	
Mayzent (Nov/19)	siponimod	Treatment of adult patients with secondary progressive multiple sclerosis with active disease	EC	▼ EM	
Sunosi (Nov/19)	solriamfetol	Treatment of excessive daytime sleepiness in narcolepsy and obstructive sleep apnoea	EC	▼	



Positive opinion – October 2019 – February 2020

Cancer

Name	Active S	Indication
Polivy (Nov/19)	polatuzumab vedotin	Treatment of relapsed / refractory diffuse large B-cell lymphoma (DLBCL) in combination with bendamustine and rituximab



EC



Rx

Other areas

Name	Active S	Indication
Evenity (Oct/19)	romosozumab	Treatment of severe postmenopausal osteoporosis
Beovu (dec/19)	brolocizumab	Treatment of neovascular (wet) age-related macular degeneration (AMD)
Staquis (Jan/20)	crisaborole	Treatment of atopic dermatitis

EC



EM

Rx

EC



EM

Rx



EC



Community reviews (Oct2019 – Feb2020)

	Object	Scope
Oct/19 (opinion) (Start Jun/19)	Flurbiprofen Geiser (oromucosal spray) - short-term relief of symptoms of sore throat	ES - Art. 29(4) - Disagreement among EU Member States - laboratory data submitted were not enough to show BE and different composition – Positive outcome
Oct/19 (Start)	Budesonide SUN and associated names - Pulmonary Chronic Obstructive Disease, Asthma	Art. 29(4) - Disagreements on the evidence to establish equivalence
Nov/19 (opinion)	Lemtrada (alemtuzumab) -multiple sclerosis	Art. 20 - Measures to minimise risk of serious side effects due to reports of rare but serious side effects, including deaths
Nov/19 (opinion)	Xeljanz (tofacitinib) - Rheumatoid Arthritis	Art. 20 – to be used with caution in patients at high risk of blood clots - could increase the risk of blood clots in the lungs and in deep veins in patients who are already at high risk



Community reviews (Oct 2019 – Feb 2020)

	Object	Scope
Feb/20 (Start)	Panexcell Clinical Laboratories Priv. Ltd – review of medicines for which studies have been conducted by Panexcell Clinical Laboratories Priv. Ltd at its site in Mumbai, India	DE - Art. 31 - Follows a GCP inspection which raised concerns about the study data used to support some MAA - inspection was carried out AT + DE (october 2019) in the context of the evaluation of MAA.
Feb/20 (Start)	Yondelis (trabectedin)	Art. 20 - Results from ongoing study OVC-3006 - Lack of efficacy in survival in ovarian cancer



PRIME eligibility

Name	Substance type	Therapeutic area	Therapeutic indication	Data of eligibility granted
Teplizumab	Biological	Endocrinology-Gynaecology- Fertility-Metabolism	Treatment to delay or prevent clinical Type 1 diabetes in “at-risk” individuals	Oct/2019
Recombinant adeno-associated virus vector based on the AAV serotype hu37 containing a single stranded DNA genome encoding a form of human FVIII(BAY2599023)	Advanced therapy	Haematology-haemostaseology	Treatment of haemophilia A	Oct/2019
Autologous anti-CD19/CD20 CAR T transduced cells (MB-CART2019.1)	Advanced therapy	Oncology	Treatment of patients with relapsed and refractory diffuse large B-cell lymphoma (DLBCL) after frontline therapy and who are ineligible for autologous stem cell transplantation	Oct/2019
Autologous CD4+ and CD8+ T-cell populations transduced with a genetically-engineered replication-incompetent, self-inactivating lentiviral vector to express a BCMA-specific CAR (JCART125)	Advanced therapy	Oncology	Treatment of relapsed / refractory multiple myeloma whose prior therapies included autologous stem cell transplant if they were eligible, a proteasome inhibitor, an immunomodulatory agent and an anti-CD38 antibody	Nov/2019
Danicopan	Chemical	Haematology - Hemostaseology	Treatment of paroxysmal nocturnal hemoglobinuria not adequately responding to a C5 inhibitor	Nov/2019



PRIME eligibility

Name	Substance type	Therapeutic area	Therapeutic indication	Data of eligibility granted
Autologous CD34+ enriched cells transduced ex vivo with lentiviral vector carrying the FANCA gene, PGK-FANCA-WPRE (RP-L102)	Advanced Therapy	Haematology-haemostaseology	Treatment of Fanconi anaemia Type A	Dec/2019
Rebisufligene etisparovvec	Advanced Therapy	Endocrinology-Gynaecology-Fertility-Metabolism	Treatment of Mucopolysaccharidosis Type IIIA, MPS IIIA (Sanfilippo A Syndrome)	Dec/2019



Interaction between CHMP and HCP - Participation in Scientific Advisory Groups and Ad-hoc Experts Groups – Sep 2019 – Feb 2020

➤ Contributing for decision on recommendations

Name	Product	Indication	Number of HCPs
11/09/2019	Xyndari (glutamine)	Hematology - sickle cell disease	3
17/09/2019	Estradiol-containing (0.01% w/w) medicinal products for topical use	Gynaecology – Art. 31	1
03/10/2019	Evenity (Romosozumab)	Osteoporosis	1
07/10/2019	Revolade (eltrombopag)	Hematology - Thrombocytopenic, Idiopathic Purpura	5
11/10/2019	Translarna (Ataluren)	Neurology - Duchenne muscular dystrophy	3
07/11/2019	Mayzent (siponimod)	Neurology - Relapsing-Remitting Multiple Sclerosis	2
22/01/2020	Hopveus (sodium oxybate)	Psychiatry - Treatment of alcohol dependence	2
27/01/2020	Emgality (galcanezumab)	Neurology - Migraine Disorders	3
18/02/2020	OFEV (nintedanib)	Respiratory - Idiopathic Pulmonary Fibrosis	9
27-28/02/2020	Nitrosamines 5(3)	Other	various



Interaction between CHMP and HCP – reviewed of safety communications – Sep 2019 – Feb 2020 = Total 18

Name	Product	Indication/Scope	Number HCPs
13/11/2019	Xeljanz (tofacitinib)	Rheumatoid Arthritis - Draft EMA safety communication – Art. 20	2
13/11/2019	Lemtrada (alemtuzumab)	Multiple sclerosis - Draft EMA safety communication – Art. 20	2
21/02/2020	Vaxchora Cholera vaccine (recombinant, live, oral)	DRAFT EMA Safety communication - medication errors	2



Interaction between CHMP and Patients' representatives - Participation in CHMP activities– 2019

➤ Contributing for decision on recommendations

Patients involved	CHMP Activity
46 patients (24 meetings)	Scientific Advisory Groups
50 responses / 11 different countries	Written procedures
9 patients (5 procedures)	Oral explanations



Working to proactively identify CHMP procedures that may benefit from patients' involvement

Review of preliminary CHMP agenda (Fatima & Concha)



Identification of procedures of interest for patients' involvement (Fatima & Concha)



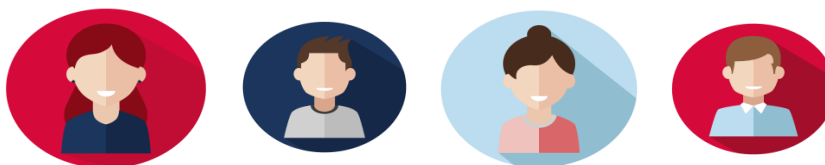
Contact PTL/CHMP Rapporteurs (EMA - Nathalie)



If confirmed, patients are contacted (EMA- Nathalie) → OE
Other



OBRIGADO



¡Muchas gracias por tu interés!

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