

27 June 2019 EMA/CHMP/SAWP/367140/2019 Press Office

Scientific advice and protocol assistance

Adopted during the CHMP meeting 24 - 27 June 2019

Pre-authorisation: scientific advice and protocol assistance EMA centralised procedures

	1995 - 2018	2019	Overall total
Scientific Advice	3866	188	4054
Follow-up to Scientific Advice	1150	71	1221
Protocol Assistance	932	56	988
Follow-up to Protocol Assistance	477	25	502
EMA/EUnetHTA parallel consultation advice	139	12	151
Qualification of novel methodologies	126	7	133
	6689	359	7048

Outcome of the June 2019 CHMP meeting in relation to scientific advice procedures

Final scientific advice procedures

			Type of request New Follow-up				Topic			
Substance	Intended indications	SA	PA	SA	PA	Quality	Preclinical	Clinical	Significant benefit	
Chemical	Treatment of Wilson disease		х					х		
Chemical	Treatment of small cell lung cancer		x			х		x		
Chemical	Treatment of acute myeloid leukaemia				х			х		
Chemical	Treatment of Duchenne Muscular Dystrophy		x				х	х	x	



Biological	Treatment of Pompe's disease		Х				Х	Х	
Advanced Therapy	Treatment of β-Thalassaemia		Х			Х			
Chemical	Treatment of spinal muscular atrophy		х				х	х	
Biological	Treatment of diffuse large B- cell lymphoma		х					Х	
Advanced Therapy	Treatment of multiple myeloma				x			Х	Х
Advanced Therapy	Treatment of multiple myeloma		х			х		Х	х
Chemical	Treatment of Niemann-Pick disease				x	Х	х		х
Chemical	Treatment of transthyretin amyloidosis		х			х			х
Biological	Treatment of thrombocytopenia		х					х	Х
Advanced Therapy	Treatment of haemophilia A		х			х	х	х	
Chemical	Treatment of Alagille syndrome		х			x			
Chemical	Treatment of progressive familial intrahepatic cholestasis		х			x	x	x	
Biological	Treatment of Pompe's disease		Х				Х	х	
Biological	Treatment of Type 2 diabetes mellitus	х						х	х
Chemical	Cutaneous treatment of actinic keratosis	х						х	
Biological	Prevention of the development of graft failure	Х				х		х	
Chemical	Treatment of urinary tract infections and gonorrhoea.	х				х			
Biological	Treatment of Niemann Pick Diseas			х		х			
Chemical	Treatment of solid tumours	х						х	
Biological	Treatment of Crohn's disease			Х				х	
Chemical	Treatment of axial spondyloarthritis	х						х	
Chemical	Treatment of heart failure	Х				Х		Х	
Biological	Treatment of rheumatoid arthritis; treatment of systemic juvenile idiopathic arthritis; treatment of idiopathic polyarthritis; treatment of giant cell arteritis; treatment of severe or life-threatening cytokine release syndrome			×				×	
Chemical	Treatment of pulmonary hypertension	Х						Х	
Biological	Treatment of acute GVHD	Х						Х	
Chemical	Treatment of chronic myeloid leukemia			x				x	
Chemical	Treatment of WHIM Syndrome			x			x	х	

Chemical	Treatment of mitochondrial DNA m.3243A>G mutation	Х		Х	Х	Х	
Biological	Treatment of neovascular (wet) age-related macular degeneration, macular edema following retinal vein occlusion, diabetic macular edema and diabetic retinopathy		х			х	
Biological	Prevention of respiratory tract infection		x			x	
Chemical	Treatment of thalassaemia		х	х	Х	х	
Biological	Treatment of neovascular (wet) age-related macular degeneration, macular edema following retinal vein occlusion, diabetic macular edema and diabetic retinopathy		x	x	х	x	
Chemical/ Innovative product or therapy	Treatment of pneumonia	x				x	
Chemical	Treatment of acute myeloid leukaemia		х		х	х	
Advanced Therapy	Repair of symptomatic articular cartilage defects	х		х			
Biological	Treatment of plaque psoriasis		х			х	
Chemical	Treatment of systemic sclerosis	х		х		х	
Biological	Treatment of haemophilia A		Х			х	
Chemical	Prevention and treatment of oral mucositis	х		х	х	х	
Chemical	Treatment of non-small cell lung cancer	х		х			
Chemical	Treatment of transthyretin amyloidosis	х				х	
Chemical	Treatment of achondroplasia	Х			Х	Х	
Chemical	Treatment of cancer	х				х	
Biological	Treatment of ulcerative colitis	х				х	
Chemical	Treatment of pulmonary arterial hypertension	х				x	
Chemical	Decrease of the incidence of severe oral mucositis	x			x	х	
Biological	Restoration and maintenance of circulating blood volume	x		x	x	x	
Biological	Treatment of osteoporosis	Х		х	Х	Х	
Chemical	Treatment of HIV-1 infection	х		х		х	
Biological	Treatment of myelodysplastic syndrome	x		x	x	×	
Chemical	Treatment of ulcerative colitis	х				x	
Advanced Therapy	Treatment of phenylketonuria	x		x	x	x	
Chemical	Treatment to slow the progression of myopia	х				x	

Treatment of psoriatic arthritis, rheumatoid arthritis, juvenile rheumatoid arthritis, ulcerative colitis, Crohn's disease, psoriasis, ankylosing spondylitis Treatment of glant cell tumour of bone, osteoporosis, bone loss and to reduce the risk of bone fractures and bone complications Chemical Improvement of walking in multiple sclerosis Chemical Treatment of non-alcoholic steatochepatitis Advanced Treatment of solid tumors x x x x x x x X X X X X X X X X X X X								
Biological biological cell tumour of bone, osteoporosis, bone loss and to reduce the risk of bone fractures and bone complications Chemical Improvement of walking in multiple sclerosis Chemical Steatohepatitis Steatohepa	Biological	arthritis, rheumatoid arthritis, juvenile rheumatoid arthritis, ulcerative colitis, Crohn's disease, psoriasis, ankylosing	х		x		x	
Chemical multiple sclerosis X Chemical Treatment of non-alcoholic steatohepatitis Advanced Treatment of solid tumors X Advanced Treatment of HIV-1 infection X Treatment of giant cell tumour of bone, osteoporosis, bone loss and to reduce the risk of bone fractures and bone complications Chemical Treatment of common cold symptoms Biological Treatment of non-small cell lung cancer Biological Treatment of moderate-to-severe atopic dermatitis Biological Treatment of non-alcoholic x x x x x Biological Treatment of non-alcoholic x x x x x Biological Treatment of non-alcoholic x x x x x x x x x x x x x x x x x x x	Biological	tumour of bone, osteoporosis, bone loss and to reduce the risk of bone fractures and bone	х		х		х	
Advanced Treatment of solid tumors x x x x x x x x x x x x x x x x x x x	Chemical		х				Х	
Therapy Chemical Treatment of HIV-1 infection x x x x x x x x x x x x x x x x x x x	Chemical		х		х	х	х	
Biological osteoporosis, bone loss and to reduce the risk of bone fractures and bone complications Chemical Treatment of common cold symptoms Biological Treatment of non-small cell lung cancer Biological Treatment of moderate-to-severe atopic dermatitis Biological Treatment of non-alcoholic steatohepatitis Chemical Relief of moderate to severe pain Reduction in the duration of neutropenia and the incidence of febrile neutropenia Chemical Treatment of non-small cell x x x x x Reduction in the duration of neutropenia and the incidence of febrile neutropenia Chemical Treatment of glioblastoma x x x x x Biological Treatment of non-small cell lung cancer Chemical Treatment of thyroid x x x x x Chemical Treatment of thyroid x x x x x Biological Treatment of thyroid x x x x x Chemical Treatment of perianal fistula(s) Biological Treatment of perianal fistula(s) Treatment of aplastic anaemia Biomarker declaration of acute myeloid x x x x x Chemical Treatment of acute myeloid x x x x x		Treatment of solid tumors	х		х	х	х	
Biological tumour of bone, osteoporosis, bone loss and to reduce the risk of bone fractures and bone complications Chemical Treatment of common cold symptoms Biological Treatment of non-small cell lung cancer Biological Treatment of moderate-to-severe atopic dermatitis Biological Treatment of non-alcoholic steatohepatitis Chemical Relief of moderate to severe pain Reduction in the duration of neutropenia and the incidence of febrile neutropenia Chemical Treatment of pon-small cell lung cancer Chemical Treatment of pon-small cell lung cancer Chemical Treatment of hon-small cell lung cancer Chemical Treatment of hon-small cell lung cancer Chemical Treatment of thyroid carcinoma Advanced Treatment of thyroid carcinoma Advanced Treatment of perianal fistula(s) Biological Treatment of aplastic anaemia Biomarker Mechanistic predictions of drug PK and/or PD Chemical Treatment of acute myeloid	Chemical	Treatment of HIV-1 infection	Х		Х	Х	Х	
Biological symptoms x x x x x x x x x x x x x x x x x x x	Biological	tumour of bone, osteoporosis, bone loss and to reduce the risk of bone fractures and bone	х		х		х	
Biological lung cancer Treatment of moderate-to-severe atopic dermatitis Biological Treatment of non-alcoholic steatohepatitis Chemical Relief of moderate to severe pain Reduction in the duration of neutropenia and the incidence of febrile neutropenia Chemical Treatment of glioblastoma x Biological Treatment of non-small cell lung cancer Chemical Treatment of hor-small cell reatment of thyroid carcinoma Advanced Treatment of perianal fistula(s) Biological Treatment of aplastic anaemia Biomarker Reduction in the duration of neutropenia and the incidence of febrile neutropenia and the incidence of febrile neutropenia and the incidence of febrile neutropenia of febrile neutropenia and the incidence of febrile neutropenia of febrile neutropen	Chemical		x				x	
Biological severe atopic dermatitis X X X X X X X X X X X X X X X X X X X	Biological		х			х	х	
Steatohepatitis Steatohepatitis Relief of moderate to severe pain Reduction in the duration of neutropenia and the incidence of febrile neutropenia Chemical Treatment of glioblastoma Treatment of non-small cell lung cancer Chemical Treatment of thyroid carcinoma Advanced Treatment of perianal fistula(s) Treatment of aplastic anaemia Biomarker Treatment of caute myeloid Treatment of acute myeloid Treatment of acute myeloid	Biological		x		х			
Reduction in the duration of neutropenia and the incidence of febrile neutropenia and the incidence of febrile neutropenia and the incidence of febrile neutropenia of febrile neutrope	Biological		x			х	x	
Biological neutropenia and the incidence of febrile neutropenia	Chemical		x			х	x	
Biological Treatment of non-small cell lung cancer Chemical Treatment of thyroid x x x x x x Advanced Treatment of perianal fistula(s) Biological Treatment of aplastic anaemia Biomarker Mechanistic predictions of drug PK and/or PD Treatment of acute myeloid x x x x x x x x x x x x x x x x x x x	Biological	neutropenia and the incidence	x		x	х	x	
Chemical lung cancer x x x x x x x x x x x x x x x x x x x	Chemical	Treatment of glioblastoma	Х				Х	
Advanced Treatment of perianal Therapy fistula(s) Biological Treatment of aplastic anaemia Biomarker Mechanistic predictions of drug PK and/or PD Chemical Treatment of acute myeloid Treatment of acute myeloid	Biological		x		х	х	х	
Therapy fistula(s) x x x x Biological Treatment of aplastic anaemia x x x x x x x x x x x x x x x x x x x	Chemical	3	х		Х		Х	
Biological anaemia x x x Biomarker mechanistic predictions of drug PK and/or PD x x Chemical Treatment of acute myeloid x x		fistula(s)	x		x			
drug PK and/or PD Treatment of acute myeloid X X X X X X Y X Y X X Y X X	Biological	anaemia	х		х			
	Biomarker	drug PK and/or PD	х				х	
	Chemical		x			х	x	

SA: Scientific Advice PA: Protocol Assistance

The above-mentioned 43 Scientific Advice letters, 14 Protocol Assistance letters, 12 Follow-up Scientific Advice, 3 Follow-up Protocol Assistance, 1 Qualifications of novel methodologies and 4 EMA/EUnetHTA parallel consultation advices, were adopted at the 24 – 27 June 2019 CHMP meeting.

New requests for scientific advice procedures

The Committee accepted 52 new Requests for which the procedure started at the SAWP meeting held on 11 – 14 June 2019. The new requests are divided as follows: 31 Initial Scientific Advice, 9 Follow-up Scientific Advice, 8 Initial Protocol Assistance, 3 Follow-up Protocol Assistance, 0 Qualification of novel methodologies and 1 EMA/EUnetHTA parallel consultation advices.