

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

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

Biological	Treatment of Pompe's disease		x				x	x	
Advanced Therapy	Treatment of β -Thalassaemia		x			x			
Chemical	Treatment of spinal muscular atrophy		x				x	x	
Biological	Treatment of diffuse large B-cell lymphoma		x					x	
Advanced Therapy	Treatment of multiple myeloma				x			x	x
Advanced Therapy	Treatment of multiple myeloma		x			x		x	x
Chemical	Treatment of Niemann-Pick disease				x	x	x		x
Chemical	Treatment of transthyretin amyloidosis		x			x			x
Biological	Treatment of thrombocytopenia		x					x	x
Advanced Therapy	Treatment of haemophilia A		x			x	x	x	
Chemical	Treatment of Alagille syndrome		x			x			
Chemical	Treatment of progressive familial intrahepatic cholestasis		x			x	x	x	
Biological	Treatment of Pompe's disease		x				x	x	
Biological	Treatment of Type 2 diabetes mellitus	x						x	x
Chemical	Cutaneous treatment of actinic keratosis	x						x	
Biological	Prevention of the development of graft failure	x				x		x	
Chemical	Treatment of urinary tract infections and gonorrhoea.	x				x			
Biological	Treatment of Niemann Pick Diseases			x		x			
Chemical	Treatment of solid tumours	x						x	
Biological	Treatment of Crohn's disease			x				x	
Chemical	Treatment of axial spondyloarthritis	x						x	
Chemical	Treatment of heart failure	x				x		x	
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			x				x	
Chemical	Treatment of pulmonary hypertension	x						x	
Biological	Treatment of acute GVHD	x						x	
Chemical	Treatment of chronic myeloid leukemia			x				x	
Chemical	Treatment of WHIM Syndrome			x			x	x	

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

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