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Press office

Recommendations on eligibility to PRIME scheme

Adopted at the CHMP meeting of 17 September 2020

During its September 2020 meeting, the CHMP reviewed 9 recommendations for eligibility to PRIME: 6 were granted and 3 were denied. The individual outcomes adopted this month are listed below.

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Eligibility granted

Name*	Substance type	Therapeutic area	Therapeutic indication	Type of data supporting request	Type of applicant
CD30-directed genetically modified autologous T cells (CD30.CAR-T)	Advanced Therapy Medicinal Product	Oncology	Treatment of classical Hodgkin lymphoma	Nonclinical+ Clinical exploratory	Other
AT-GTX-501 (adeno-associated viral vector, serotype 9, containing the human CLN6 gene)	Advanced Therapy Medicinal Product	Neurology	Slowing disease progression in paediatric patients with variant late infantile neuronal ceroid lipofuscinosis 6 (vLINCL6)	Nonclinical+ Clinical exploratory	Other
Autologous CD34+ cell-enriched population from patients with sickle cell disease that contains haematopoietic stem cells transduced with BB305 lentiviral vector encoding the βA-T87Q-globin gene (bb1111)	Advanced Therapy Medicinal Product	Haematology-haemostaseology	Treatment of Sickle Cell Disease	Nonclinical+ Clinical exploratory	Other
CTX001 (Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the BCL11A gene)	Advanced Therapy Medicinal Product	Haematology - Hemostaseology	Treatment of Sickle Cell Disease	Nonclinical+ Clinical exploratory	Other
Iptacopan	Chemical Medicinal Product	Endocrinology-Gynaecology-Fertility-Metabolism	Treatment of C3 glomerulopathy (complement-driven renal disease)	Nonclinical+ Clinical exploratory	Other

Name*	Substance type	Therapeutic area	Therapeutic indication	Type of data supporting request	Type of applicant
OTL-203 (Autologous CD34+ hematopoietic stem and progenitor cells genetically modified with the lentiviral vector (IDUA LV) encoding for the alpha-L-iduronidase gene)	Advanced Therapy Medicinal Product	Endocrinology-Gynaecology-Fertility-Metabolism	Treatment of Mucopolysaccharidosis type I (MPS-1)	Nonclinical+ Clinical exploratory	SME

* Name of the active substance, INN, common name, chemical name or company code.

SME applicants are micro-, small-and medium-sized-enterprises registered with the Agency's SME office. Other types of applicants are those not qualifying or not registered as SME or not fulfilling the definition of academic sponsors.

Eligibility denied

Substance type	Therapeutic area	Therapeutic indication	Type of data supporting request	Type of applicant
Advanced Therapy Medicinal Product	Oncology	Treatment of relapse/refractory lymphoplasmacytic lymphoma and Waldenstrom's Macroglobulinemia.	Nonclinical+ Clinical exploratory	SME
Advanced Therapy Medicinal Product	Neurology	Treatment of Duchenne Muscular Dystrophy	Nonclinical+ Clinical exploratory	Other
Advanced Therapy Medicinal Product	Neurology	Treatment of Parkinson's Disease	Nonclinical+ Clinical exploratory	Other

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Cumulative overview of recommendations on PRIME eligibility requests adopted by 17 Speptember 2020