



20 November 2023  
EMA/521657/2016  
Human Medicines Evaluation

## List of medicines currently in PRIME scheme

PRIME is a development support scheme for medicines addressing an unmet medical need. Further information on the criteria for eligibility and features of the scheme are available on the EMA website.

This list includes medicines that have been granted eligibility to PRIME and which are still in the scheme as their development is ongoing. For medicines which were granted PRIME eligibility but for which a marketing authorisation application has been submitted or which were withdrawn from the scheme, please refer to the tab 'PRIME products (previous)'.

Name*	Substance type	Therapeutic area	Therapeutic indication	Type of data supporting request	Type of applicant	Date of granting PRIME eligibility
ABSK021	Chemical Medicinal Product	Oncology	Treatment of tenosynovial giant cell tumor (TGCT)	Nonclinical + Clinical exploratory	Other	25/05/2023
Adeno-associated viral vector serotype 8 containing the human MTM1 gene (AT132)	Advanced Therapy Medicinal Product	Other	Treatment of X-linked Myotubular Myopathy	Nonclinical + Clinical exploratory	SME	31/05/2018
Adenovirus associated viral vector serotype 5 containing the human RPGR gene	Advanced Therapy Medicinal Product	Ophthalmology	Treatment of X linked Retinitis Pigmentosa owing to defects in Retinitis Pigmentosa GTPase Regulator	Nonclinical + Clinical exploratory	Other	27/02/2020
<i>Adenovirus associated viral vector serotype 8 containing the human CNGB3 gene (AAV2/8-hCARp.hCNGB3)</i>	<i>Advanced Therapy Medicinal Product</i>	<i>Ophthalmology</i>	<i>Treatment of achromatopsia associated with defects in CNGB3</i>	<i>Nonclinical + Tolerability first in man</i>	<i>Other</i>	<i>22/02/2018</i>

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ADP-A2M4 (transduced CD4+ and CD8+ cells)	Advanced Therapy Medicinal Product	Oncology	Treatment of HLA-A*02 positive patients with inoperable or metastatic synovial sarcoma who have received prior chemotherapy and whose tumour expresses the MAGE-	Nonclinical + Clinical exploratory	Other	23/07/2020
ADV-M-022	Advanced Therapy Medicinal Product	Ophthalmology	Treatment of neovascular(wet) age-related macular degeneration	Nonclinical + Clinical exploratory	SME	23/06/2022
AGTC-501 (laruparetigene zovaparvovec)	Advanced Therapy Medicinal Product	Ophthalmology	Treatment of XLRP	Nonclinical + Clinical exploratory	Other	26/04/2023
Allogeneic multi-virus specific T lymphocytes targeting BK Virus, cytomegalovirus, human herpes virus-6, Epstein Barr virus, and adenovirus (ALVR-105)	Advanced Therapy Medicinal Product	Infectious Diseases	Treatment of serious infections with BK virus, cytomegalovirus, human herpes virus-6, Epstein Barr virus, and/or adenovirus in allogeneic HSCT recipients	Nonclinical + Clinical exploratory	Other	30/01/2020
AMB-05X	Biological Medicinal Product	Oncology	Treatment of tenosynovial giant cell tumour	Nonclinical + Clinical exploratory	SME	15/12/2022
Anti-(insulin receptor) human monoclonal antibody	Biological Medicinal Product	Endocrinology - Gynaecology - Fertility - Metabolism	Treatment of hyperinsulinaemic hypoglycaemia due to congenital hyperinsulinism	Nonclinical + Clinical exploratory	SME	12/10/2023
ANX007	Biological Medicinal Product	Ophthalmology	Treatment of Geographic Atrophy (GA) secondary to age-related macular degeneration (AMD)	Nonclinical + Clinical exploratory	SME	12/10/2023
Apitegromab	Biological Medicinal Product	Neurology	Treatment of spinal muscular atrophy	Nonclinical + Clinical exploratory	SME	25/03/2021

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ApTOLL	Chemical Medicinal Product	Cardiovascular diseases	Acute Ischemic Stroke	Nonclinical + Clinical exploratory	SME	26/01/2023
ARI-0001	Advanced Therapy Medicinal Product	Oncology	Treatment of patients older than 25 years with relapsed/refractory acute lymphoblastic	Nonclinical + Clinical exploratory	Academic sector	16/12/2021
Asunercept	Biological Medicinal Product	Oncology	Treatment of glioblastoma	Nonclinical + Clinical exploratory	Other	18/05/2017
Autologous anti-CD19/CD20 CAR T transduced cells (MB-CART2019.1)	Advanced Therapy Medicinal Product	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	Nonclinical + Clinical exploratory	Other	17/10/2019
Autologous CD34+ cells transduced with lentiviral vector encoding the human beta globin gene (OTL-300)	Advanced Therapy Medicinal Product	Haematology - Haemostaseology	Treatment of transfusion-dependent $\beta$ -thalassaemia	Nonclinical + Clinical exploratory	SME	20/09/2018
Autologous CD34+ enriched cells transduced ex vivo with lentiviral vector carrying the FANCA gene, PGK-FANCA-WPRE (RP-L102)	Advanced Therapy Medicinal Product	Haematology - Haemostaseology	Treatment of Fanconi anaemia Type A	Nonclinical + Clinical exploratory	Other	12/12/2019
Autologous CD4 and CD8 T cells transduced with lentiviral vector containing an affinity-enhanced T cell receptor to target the cancer-testis tumour antigen NY-ESO-1 (NY-ESO-1c259T)	Advanced Therapy Medicinal Product	Oncology	Treatment of HLA-A*0201, HLA-A*0205, or HLA-A*0206 allele positive patients with inoperable or metastatic synovial sarcoma who have received prior chemotherapy and whose tumour expresses the NY-ESO-1 tumour antigen	Nonclinical + Clinical exploratory	Other	21/07/2016

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BCX9250	Chemical Medicinal Product	Other, please specify: Musculoskeletal and connective tissue disorders	Treatment of fibrodysplasia ossificans progressiva	Nonclinical + Tolerability first in man	SME	22/04/2022
BI 456906	Chemical Medicinal Product	Gastroenterology - Hepatology	Treatment of NASH/NAFLD with fibrosis	Non-clinical + Clinical exploratory	Other	09/11/2023
BNT211	Advanced Therapy Medicinal Product	Oncology	Treatment of testicular germ cell tumours	Nonclinical + Clinical exploratory	Other	23/06/2022
Bomedemstat	Chemical Medicinal Product	Haematology - Haemostaseology	Treatment of myelofibrosis	Nonclinical + Clinical exploratory	SME	23/07/2020
Brensocatib	Chemical Medicinal Product	Pneumology- allergology	Treatment of non-cystic fibrosis	Nonclinical + Clinical exploratory	Other	12/11/2020
CD30-directed genetically modified autologous T cells (CD30.CAR-T)	Advanced Therapy Medicinal Product	Oncology	Treatment of classical Hodgkin lymphoma	Nonclinical + Clinical exploratory	Other	17/09/2020
Chikungunya virus virus-like particle vaccine (PXVX0317)	Biological Medicinal Product	Infectious Diseases	Active immunisation to prevent disease caused by chikungunya virus infection in individuals aged 12 years and older	Nonclinical + Clinical exploratory	Other	19/09/2019
CT041 (CAR-CLDN18.2 T cells)	Advanced Therapy Medicinal Product	Oncology	Treatment of patients with advanced gastric cancer who have failed at least 2 prior lines of systemic therapy	Nonclinical + Clinical exploratory	Other	11/11/2021
CTI-1601	Biological Medicinal Product	Neurology	Treatment of Friedreich's Ataxia	Nonclinical + Tolerability first in man	SME	20/05/2021
Deoxycytidine / deoxythymidine	Chemical Medicinal Product	Endocrinology - Gynaecology - Fertility - Metabolism	Treatment of Thymidine Kinase 2 Deficiency	Nonclinical + Clinical exploratory	SME	28/06/2018
DNL310	Biological Medicinal Product	Endocrinology - Gynaecology - Fertility - Metabolism	Treatment of mucopolysaccharidosis type II	Nonclinical + Clinical exploratory	Other	19/05/2022

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DTX401	Advanced Therapy Medicinal Product	Endocrinology - Gynaecology - Fertility - Metabolism	Treatment of glycogen storage disease type Ia (GSDIa, von Gierke disease)	Nonclinical + Clinical exploratory	Other	15/09/2022
ECT-001-CB	Advanced Therapy Medicinal Product	Oncology	Urgent allogeneic haematopoietic stem cell transplantations	Nonclinical + Clinical exploratory	SME	10/12/2020
Efruxifermin (EFX)	Biological Medicinal Product	Gastroenterology - Hepatology	Non-alcoholic steatohepatitis	Nonclinical + Clinical exploratory	SME	15/10/2020
FBX-101	Advanced Therapy Medicinal Product	Neurology	Treatment of Krabbe disease/Globoid Cell Leukodystrophy	Nonclinical + Tolerability first in man	SME	15/12/2022
Fully human anti-BCMA autologous CAR T Cell (CT053)	Advanced Therapy Medicinal Product	Oncology	Treatment of patients with relapsed and/or refractory multiple myeloma (MM) whose prior regimens included a proteasome inhibitor, an immunomodulatory agent and an anti-CD38 monoclonal antibody	Nonclinical + Clinical exploratory	SME	19/09/2019
GBS6 (Group B Streptococcus 6-Valent Polysaccharide Conjugate Vaccine)	Immunological Medicinal Product	Vaccines	Prevention of Group B streptococcal invasive disease due to the vaccine serotypes in infants by active immunization of pregnant women	Nonclinical + Clinical exploratory	Other	22/04/2022
GBS-NN/NN2 vaccine	Immunological Medicinal Product	Infectious Diseases	Prevention of Group B streptococcal invasive disease in infants by active immunization of pregnant women	Nonclinical + Clinical exploratory	SME	15/09/2022
Genetically modified replication-incompetent herpes simplex virus-1 expressing collagen VII (KB103)	Advanced Therapy Medicinal Product	Dermatology	Treatment of Dystrophic Epidermolysis Bullosa	Nonclinical + Clinical exploratory	SME	28/03/2019
Glenzocimab	Biological Medicinal Product	Haematology - Haemostaseology	Treatment of acute ischemic stroke	Nonclinical + Clinical exploratory	SME	21/07/2022

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GNT0003	Advanced Therapy Medicinal Product	Other, Congenital, familial and genetic disorders	Treatment of severe Crigler-Najjar syndrome in adults and children >10 years old, requiring phototherapy	Nonclinical + Clinical exploratory	Other	26/01/2023
Human immunoglobulin G1 constant region - human ectodysplasin-A1 receptor-binding domain fusion protein	Biological Medicinal Product	Dermatology	Treatment of X-linked hypohidrotic ectodermal dysplasia	Nonclinical + Clinical exploratory	Other	12/10/2017
Inaxaplin	Chemical Medicinal Product	Uro-nephrology	Treatment of proteinuric apolipoprotein L1 (APOL1)-mediated chronic kidney disease in patients who have 2 APOL1 genetic mutations (G1/G1, G1/G2 or G2/G2)	Nonclinical + Clinical exploratory	Other	19/05/2022
Iopofosine I-131	Radiopharmaceutical Medicinal Product	Oncology	Treatment of patients with Waldenstrom's macroglobulinemia (WM) who received at least two prior lines of therapy	Nonclinical + Clinical exploratory	SME	14/09/2023
Lacutamab	Biological Medicinal Product	Oncology	Treatment of patients with Sézary Syndrome who have received at least two prior systemic therapies	Nonclinical + Clinical exploratory	Other	12/11/2020
Lonafarnib	Chemical Medicinal Product	Infectious Diseases	Treatment of hepatitis D virus infection	Nonclinical + Clinical exploratory	SME	13/12/2018
Magrolimab	Biological Medicinal Product	Oncology	Myelodysplastic Syndromes	Nonclinical + Clinical exploratory	Other	15/10/2020
MB-107	Advanced Therapy Medicinal Product	Immunology - Rheumatology - Transplantation	Treatment of X-linked severe combined immunodeficiency (XSCID) in newly diagnosed infants	Nonclinical + Clinical exploratory	SME	22/07/2021

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Messenger RNA encoding Cas9, Single guide RNA targeting the human KLKB1 gene	Advanced Therapy Medicinal Product	Cardiovascular diseases	Treatment of hereditary angioedema	Nonclinical + Clinical exploratory	Other	12/10/2023
mRNA-4157, V940	Advanced Therapy Medicinal Product	Oncology	Treatment of melanoma	Nonclinical + Clinical exploratory	Other	30/03/2023
MV-CHIK vaccine	Biological Medicinal Product	Vaccines	Prevention of Chikungunya fever	Nonclinical + Clinical exploratory	SME	31/05/2018
<i>Mycobacterium tuberculosis (MTBVAC)</i>	<i>Immunological Medicinal Product</i>	<i>Vaccines</i>	<i>Active immunization against tuberculosis disease in newborns, adolescents and adults</i>	<i>Nonclinical + Tolerability first in man</i>	<i>SME</i>	<i>28/06/2018</i>
<i>Nangibotide (LR12)</i>	<i>Chemical Medicinal Product</i>	<i>Infectious Diseases</i>	<i>Treatment of septic shock</i>	<i>Nonclinical + Tolerability first in man</i>	<i>SME</i>	<i>09/11/2017</i>
Nicardipine	Chemical Medicinal Product	Neurology	Treatment of non-traumatic subarachnoid haemorrhage in patients undergoing surgery	Nonclinical + Clinical exploratory	SME	13/10/2022
Obecabtagene autoleucel/AUTO1	Advanced Therapy Medicinal Product	Oncology	Treatment of relapsed or refractory B cell acute lymphoblastic leukaemia	Nonclinical + Clinical exploratory	Other	25/03/2021
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	17/09/2020
Pabinafusp alfa (JR-141)	Biological Medicinal Product	Endocrinology - Gynaecology - Fertility - Metabolism	Treatment of Mucopolysaccharidosis type II (MPS II)	Nonclinical + Clinical exploratory	Other	14/10/2021

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PB2452	Biological Medicinal Product	Cardiovascular Diseases	Reversal of antiplatelet effects of ticagrelor in patients with uncontrolled major or life-threatening bleeding or requiring urgent surgery or invasive procedure	Nonclinical + Clinical exploratory	Other	30/01/2020
PF-06823859	Biological Medicinal Product	Immunology - Rheumatology - Transplantation	Treatment of Dermatomyositis	Nonclinical + Clinical exploratory	Other	15/10/2020
PRAX-222	Chemical Medicinal Product	Neurology	Treatment of SCN2A Developmental and Epileptic Encephalopathy (SCN2A-DEE)	Nonclinical + Tolerability first in man	SME	09/11/2023
Purified Inactivated Zika Virus vaccine (TAK-426)	Immunological Medicinal Product	Vaccines	Active immunization for the prevention of disease caused by Zika virus	Nonclinical + Clinical exploratory	Other	28/03/2019
Rebisufligene etisparvovec	Advanced Therapy Medicinal Product	Endocrinology - Gynaecology - Fertility - Metabolism	Treatment of Mucopolysaccharidosis Type IIIA, MPS IIIA (Sanfilippo A Syndrome)	Nonclinical + Clinical exploratory	SME	12/12/2019
RP-A501	Advanced Therapy Medicinal Product	Cardiovascular Diseases	Treatment of Danon disease	Nonclinical + Clinical exploratory	Other	25/05/2023
RP-L201	Advanced Therapy Medicinal Product	Immunology - Rheumatology - Transplantation	Treatment of Leukocyte Adhesion Deficiency-I	Nonclinical + Clinical exploratory	Other	25/03/2021
RP-L301	Advanced Therapy Medicinal Product	Endocrinology - Gynaecology - Fertility - Metabolism	Treatment of pyruvate kinase deficiency	Nonclinical + Clinical exploratory	Other	20/07/2023
Seladelpar (MBX-8025)	Chemical Medicinal Product	Gastroenterology - Hepatology	Treatment of Primary Biliary Cholangitis	Nonclinical + Clinical exploratory	SME	13/10/2016



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Sepofarsen (QR-110)	Chemical Medicinal Product	Ophthalmology	Treatment of Leber's congenital amaurosis due to the p.Cys998X mutation in the CEP290 Gene	Nonclinical + Clinical exploratory	SME	25/07/2019
Setrusumab (Recombinant humanised monoclonal IgG2 lambda antibody against human sclerostin, BPS804)	Biological Medicinal Product	Other	Treatment of osteogenesis imperfecta types I, III and IV	Nonclinical + Clinical exploratory	SME	09/11/2017
Sotatercept	Biological Medicinal Product	Cardiovascular diseases	Treatment of pulmonary arterial hypertension (PAH)	Nonclinical + Clinical exploratory	SME	30/04/2020
Tasadenoturev (Adenovirus serotype 5 containing partial E1A deletion and an integrin-binding domain, DNX-2401)	Advanced Therapy Medicinal Product	Oncology	Treatment of recurrent glioblastoma in patients for which a gross total resection is not possible or advisable, or for those who refuse further surgery	Nonclinical + Clinical exploratory	Other	21/07/2016
Teplizumab	Biological Medicinal Product	Endocrinology - Gynaecology - Fertility - Metabolism	Treatment to delay or prevent clinical Type 1 diabetes in "at-risk" individuals	Nonclinical + Clinical exploratory	SME	17/10/2019
Tominersen (RO7234292)	Chemical Medicinal Product	Neurology	Treatment of Huntington's Disease	Nonclinical + Clinical exploratory	Other	26/07/2018
VLA1553	Immunological Medicinal Product	Vaccines	Prophylaxis against Chikungunya disease	Nonclinical + Clinical exploratory	Other	15/10/2020
VX-880	Advanced Therapy Medicinal Product	Endocrinology - Gynaecology - Fertility - Metabolism	Treatment of type 1 diabetes (T1D) mellitus with impaired hypoglycemia awareness and severe hypoglycemia	Nonclinical + Clinical exploratory	Other	30/03/2023

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4D-150	Advanced Therapy Medicinal Product	Ophthalmology	Treatment of neovascular (wet) age-related macular degeneration (wet AMD)	Nonclinical + Clinical exploratory	SME	12/10/2023

\* 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.

*Product(s) in italic have been granted eligibility to the scheme at early stages of development (proof of principle/proof of mechanism).*