

Table 1: Overview of the ATMPs that have been submitted for Marketing Authorization Application (MAA) at the EMA. [latest update: June 2022]

Brand name	Active substance	Cell, gene, or tissue therapy	Current status*	Indication, if granted, otherwise requested	Classified as orphan (initial MAA)	MAA outcome (CHMP opinion)	Date of MAA outcome	Type of MA
ChondroSelect	Characterised viable autologous cartilage cells expanded ex vivo expressing specific marker proteins	TEP	Withdrawn post-authorisation, 30-11-2016	Repair of single symptomatic cartilage defects of the femoral condyle of the knee (International Cartilage Repair Society [ICRS] grade III or IV) in adults.	No	positive	5-10-2009	standard
Advexin	Adenoviral vector containing human p53 gene	GTMP	Not authorised	(Requested) Li-Fraumeni cancer in patients from the age of 18 years.	Yes	withdrawn	17-12-2008	
Contusogene Ladenovec Gendux	Contusogene ladenovec	GTMP	Not authorised	(Requested) Recurrent or refractory squamous cell carcinoma of the head and neck.	No	withdrawn	12-6-2009	
Cerepro	Adenovirus-mediated Herpes simplex virus-thymidine kinase gene	GTMP	Not authorised	(Requested) Patients with operable high-grade glioma.	Yes	withdrawn	8-3-2010	
Glybera	Alipogene tiparvovec	GTMP	Withdrawn post-authorisation, 28-10-2017	Adult patients diagnosed with lipoprotein lipase deficiency demonstrating hyperchylomicronaemia or having a history of acute pancreatitis.	Yes	positive	25-10-2012	exceptional
Hyalograft C autograft	Characterized viable autologous chondrocytes expanded in vitro, seeded and cultured on a hyaluronan based scaffold	TEP	Not authorised	(Requested) Surgical repair of symptomatic cartilage defects of the femoral condyle (medial, lateral) or trochlea, caused by acute or repetitive trauma in adults	No	withdrawn	14-1-2013	
OraNera (CAOMECS)	Autologous oral mucosal epithelial cells	TEP	Not authorised	(Requested) Restoration of the ocular epithelial surface in patients with Limbal Stem Cell Deficiency (LSCD) without stromal involvement	No	withdrawn	14-3-2013	
MACI	Autologous cultured chondrocytes	TEP	Withdrawn post-authorisation, 1-7-2018	Repair of symptomatic, full-thickness cartilage defects of the knee (grade III and IV of the Modified Outerbridge Scale) of 3-20 cm ² in skeletally mature adult patients.	No	positive	27-6-2013	standard
Provenge	Autologous peripheral-blood mononuclear cells including a minimum of 50 million autologous CD54+ cells activated with prostatic acid phosphatase granulocyte-macrophage colony-stimulating factor	CTMP	Withdrawn post-authorisation, 6-5-2015	Asymptomatic or minimally symptomatic metastatic (non-visceral) castrate resistant prostate cancer in male adults in whom chemotherapy is not yet clinically indicated	No	positive	6-9-2013	standard

Holoclar	Ex vivo expanded autologous human corneal epithelial cells containing stem cells	TEP	Authorised	Moderate to severe limbal stem cell deficiency (LSCD) due to physical or chemical burns to the eye(s) in adults	Yes	positive	17-2-2015	conditional
Heparesc	Human heterologous liver cells	CTMP	Not authorised	(Requested) Children from birth up to 3 years of age with specific urea cycle disorders	Yes	negative	21-12-2015	
Imlygic	Talimogene laherparepvec	GTMP	Authorised	Adults with melanoma that cannot be removed by surgery and that has spread either to the surrounding area or to other areas of the body (regionally or distantly metastatic) without affecting the bones, brain, lung or other internal organs	No	positive	16-12-2015	standard
Strimvelis	Autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human adenosine deaminase (ADA) cDNA sequence from human haematopoietic stem/progenitor (CD34+) cells	GTMP	Authorised	Children with severe combined immunodeficiency due to ADA-deficiency (ADA-SCID), who have no matching donor for a stem cell transplant.	Yes	positive	26-5-2016	standard
Zalmoxis	Allogeneic T cells genetically modified with a retroviral vector encoding for a truncated form of the human low affinity nerve growth factor receptor (Δ LNGFR) and the herpes simplex I virus thymidine kinase (HSV-TK Mut2)	CTMP	Withdrawn post-authorisation, 9-10-2019	Adjunctive treatment in haploidentical haematopoietic stem cell transplantation (HSCT) of adult patients with high-risk haematological malignancies	Yes	positive	18-8-2016	conditional
Spherox	Spheroids of human autologous matrix-associated chondrocytes	TEP	Authorised	Symptomatic articular cartilage defects of the femoral condyle and the patella of the knee	No	positive	10-7-2017	standard
Alofisel	Darvadstrocel	CTMP	Authorised	Complex perianal fistulas in patients with Crohn's disease	Yes	positive	23-3-2018	standard
Yescarta	Axicabtagene ciloleucel	GTMP	Authorised	Adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL) and primary mediastinal large B-cell lymphoma, after two or more lines of systemic therapy	Yes	positive	23-8-2018	standard
Kymriah	Tisagenlecleucel	GTMP	Authorised	Paediatric and young adult patients (up to 25 years of age) with B-cell acute lymphoblastic leukaemia (ALL) that is refractory or in second or later relapse, and in adult patients with relapsed or	Yes	positive	22-8-2018	standard

				refractory DLBCL after two or more lines of systemic therapy				
Raligize	Axalimogene filolisbac	GTMP	Not authorised	(Requested) Women with cervical cancer whose disease had not responded to, or had come back after, initial treatment.	No	withdrawn	10-7-2018	
Luxturna	Voretigene neparvovec	GTMP	Authorised	Adults and children suffering from inherited retinal dystrophy caused by RPE65 gene mutation, a rare genetic disorder which causes vision loss and usually leads to blindness	Yes	positive	22-11-2018	standard
Zynteglo	Autologous CD34+ cell enriched population that contains hematopoietic stem cells transduced with lentiglobin BB305 lentiviral vector encoding the beta-A-T87Q-globin gene	GTMP	Withdrawn post- authorisation, 15-4-2022	Patients 12 years and older with transfusion dependent β thalassaemia (TDT) who do not have a $\beta 0/\beta 0$ genotype, for whom haematopoietic stem cell (HSC) transplantation is appropriate but a human leukocyte antigen (HLA) matched related HSC donor is not available	Yes	positive	29-5-2019	conditional
Luxceptar	Viable T-Cells	CTMP	Not authorised	(Requested) Improve survival in patients with blood cancers being treated with a specific type of blood stem cell transplant	Yes	withdrawn	6-11-2019	
Zolgensma	Onasemnogene abeparvovec-xioi	GTMP	Authorised	Babies and young children who have a rare, serious inherited condition called spinal muscular atrophy (SMA)	Yes	positive	18-5-2020	conditional
Libmeldy	Atidarsagene autotemcel	GTMP	Authorised	Children with the 'late infantile' or 'early juvenile' forms of metachromatic leukodystrophy (MLD).	Yes	positive	17-12-2020	standard
Tecartus	Autologous peripheral blood T cells CD4 and CD8 selected and CD3 and CD28 activated transduced with retroviral vector	GTMP	Authorised	Relapsed or refractory Mantle Cell Lymphoma.	Yes	positive	14-12-2020	conditional
Roctavian	Valoctocogene roxaparvovec	GTMP	Not authorised	(Requested) Severe haemophilia A	Yes	withdrawn	4-11-2020	
Artobend	Autologous human chondrocytes in vitro expanded	GTMP	Not authorised	(Requested) Cartilage defects in the knee	No	withdrawn	18-11-2020	
Skysona	Elivaldogene autotemcel	GTMP	Withdrawn post- authorisation, 18-11-2021	Children under 18 years of age with early cerebral adrenoleukodystrophy (CALD).	Yes	positive	16-7-2021	standard
Abecma	Idecabtagene vicleucel	GTMP	Authorised	Relapsed and refractory multiple myeloma	Yes	positive	18-8-2021	conditional

Breyanzi	Lisocabtagene maraleucel	GTMP	Authorised	Adults with different types of blood cancer, incl. DLBCL, PMBCL and FL3B	Yes	positive	4-4-2022	standard
Carvykti	Ciltacabtagene autoleucel	GTMP	Authorised	Adult patients with relapsed and refractory multiple myeloma, who have received at least three prior therapies, including an immunomodulatory agent, a proteasome inhibitor and an anti-CD38 antibody and have demonstrated disease progression on the last therapy	Yes	positive	25-5-2022	conditional
Sitoiganap	Allogeneic glioma tumor cell lysates (inactivated), allogeneic glioma tumor cells (inactivated), autologous glioma tumor cell lysates (inactivated), autologous glioma tumor cells (inactivated)	CTMP	Not authorised	(Requested) Adults with a type of brain cancer called malignant glioma, that is progressive (continues to grow) or recurrent (has come back) after treatment.	Yes	withdrawn	2-5-2022	
Upstaza	Eladocagene exuparvovec	GTMP	Authorised	Adults and children aged 18 months and older with severe aromatic L-amino acid decarboxylase (AADC) deficiency with a genetically confirmed diagnosis.	Yes	positive	18-7-2022	exceptional

**The current status describes the corresponding date if the ATMP is withdrawn post-authorisation. Abbreviations: CHMP: Committee for Medicinal Products for Human Use, CTMP: Cell therapy medicinal product, GTMP: Gene therapy medicinal product, MA: marketing authorisation, MAA: marketing authorisation application, TEP: Tissue engineered product.*