Overview of ATMPs submitted for marketing authorisation at the European Medicines Agency (update: June 2025)

Updated until June 2025 by Christine van Hattem (PhD candidate, Utrecht University) based on the initial overview by Eleanor van Dijk (during her MSc research project at Utrecht University)

Brand name (chronological order)	Active substance / INN	Cell / gene / tissue	Therapeutic Area	Initial indication, if granted, otherwise requested *	Orphan (initial MA)	PRIME	CHMP opinion	MA date	MA granted	Status
Cerepro (2007)	sitimagene ceradenovec	GTMP – in vivo	oncology	[Requested] For use in conjunction with ganciclovir sodium for the treatment of patients with operable high-grade glioma	yes (at MAA)	no	withdrawn during re-examination, 13-7-2007	-	-	-
Advexin	contusugene ladenovec	GTMP – in vivo	oncology	[Requested] For the treatment of Li-Fraumeni cancer patients	yes (at MAA)	no	withdrawn before opinion, 17-12-2008	-	-	-
Contusugene Ladenovec Gendux	contusugene ladenovec	GTMP – in vivo	oncology	[Requested] In adults for the treatment of patients with recurrent or refractory squamous cell carcinoma of the head and neck as monotherapy	no (at MAA)	no	withdrawn before opinion, 12-6-2009	-	-	-
ChondroCelect	characterised viable autologous cartilage cells expanded ex vivo expressing specific marker proteins	TEP	cartilage defect	Repair of single symptomatic cartilage defects of the femoral condyle of the knee (International Cartilage Repair Society [ICRS] grade III or IV) in adults. Concomitant asymptomatic cartilage lesions (ICRS grade I or II) might be present.	no	no	positive	5-10-2009	standard	MA withdrawn 30-11-2016
Cerepro (2010)	sitimagene ceradenovec	GTMP – in vivo	oncology	[Requested] For use in conjunction with ganciclovir sodium for the treatment of patients with operable high-grade glioma	yes (at MAA)	no	withdrawn during re-examination, 8-3-2010	-	-	-
Glybera	alipogene tiparvovec	GTMP – in vivo	cardiovascular disease	For adult patients diagnosed with familial lipoprotein lipase deficiency (LPLD) and suffering from severe or multiple pancreatitis attacks despite dietary fat restrictions.	yes	no	positive (after re-examination)	25-10-2012	exceptional	MA expired 28-10-2017
Hyalograft C autograft	characterized viable autologous chondrocytes expanded in vitro, seeded and cultured on a hyaluronan based scaffold	TEP; cATMP	cartilage defect	[Requested] For use in repairing cartilage defects at the end of the femur (the thigh bone), where the bone forms part of the knee joint. It was for use in adults experiencing symptoms caused by sudden or repetitive trauma to the cartilage.	no (at MAA)	no	withdrawn before opinion, 14-1-2013	-	-	-
OraNera (CAOMECS)	autologous oral mucosal epithelial cells	TEP	ophthalmology	[Requested] To treat limbal stem cell deficiency (LSCD) in adults.	no (at MAA)	no	withdrawn before opinion, 14-3-2013	-	-	-
MACI	matrix-applied characterised autologous cultured chondrocytes	TEP	cartilage defect	For the 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	no	positive	27-6-2013	standard	MA expired 1-7-2018

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Provenge	sipuleucel-T	CTMP	oncology	For treatment of asymptomatic or minimally symptomatic metastatic (nonvisceral) castrate resistant prostate cancer in male adults in whom chemotherapy is not yet clinically indicated.	no	no	positive	6-9-2013	standard	MA withdrawn 6-5-2015
Holoclar	ex vivo expanded autologous human corneal epithelial cells containing stem cells	TEP	ophthalmology	For treatment of adult patients with moderate to severe limbal stem cell deficiency, unilateral or bilateral, due to physical or chemical ocular burns.	yes	no	positive	17-2-2015	conditional	authorised
Heparesc	human heterologous liver cells	CTMP	metabolic disorder	[Requested] For treatment of paediatric patients from birth to less than 6 years of age with urea cycle disorders (UCD) caused by carbamoylphosphate synthetase 1 deficiency, ornithine transcarbamylase deficiency, argininosuccinate synthetase deficiency (citrullinaemia type 1), argininosuccinate lyase deficiency (argininosuccinic aciduria), or arginase deficiency (hyperargininaemia).	yes (at MAA)	no	negative (after re-examination), 22-10-2015	-	-	-
Imlygic	talimogene laherparepvec	GTMP – in vivo	oncology	For the treatment of adults with unresectable melanoma that is regionally or distantly metastatic (Stage IIIB, IIIC and IVM1a) with no bone, brain, lung or other visceral disease	no	no	positive	16-12-2015	standard	authorised
Strimvelis	autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human ADA cDNA sequence	GTMP – ex vivo	metabolic disorder	For the treatment of patients with severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID), for whom no suitable HLA-matched related stem cell donor is available	yes	no	positive	26-5-2016	standard	authorised
Zalmoxis	allogeneic T cells genetically modified with a retroviral vector encoding for a truncated form of the human low affinity nerve growth factor receptor (ΔLNGFR) and the herpes simplex I	CTMP	haemato-oncology	As adjunctive treatment in haploidentical HSCT of adult patients with high-risk haematological malignancies	yes	no	positive	18-8-2016	conditional	MA withdrawn 9-10-2019

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	virus thymidine kinase (HSV-TK Mut2)									
Spherox	spheroids of human autologous matrix-associated chondrocytes	TEP	cartilage defect	For repair of symptomatic articular cartilage defects of the femoral condyle and the patella of the knee (International Cartilage Repair Society [ICRS] grade III or IV) with defect sizes up to 10 cm^2 in adults.	no	no	positive	10-7-2017	standard	authorised
Alofisel	darvadstrocel	CTMP	autoimmune disease	For the treatment of complex perianal fistulas in adult patients with nonactive/mildly active luminal Crohn's disease, when fistulas have shown an inadequate response to at least one conventional or biologic therapy.	yes	no	positive	23-3-2018	standard	MA withdrawn 13-12-2024
Raligize	axalimogene filolisbac	GTMP (unclear)	oncology	[Requested] for the treatment of adult women who progress beyond first-line therapy of persistent, recurrent or metastatic carcinoma of the cervix.	no (at MAA)	no	withdrawn before opinion, 10-7-2018	-	-	-
Yescarta	axicabtagene ciloleucel	GTMP – ex vivo	haemato-oncology	For the treatment of adult patients with relapsed or refractory DLBCL and PMBCL, after two or more lines of systemic therapy	yes	yes	positive	23-8-2018	standard	authorised
Kymriah	tisagenlecleucel	GTMP – ex vivo	haemato-oncology	For the treatment of: · Paediatric and young adult patients up to 25 years of age with B-cell acute lymphoblastic leukaemia (ALL) that is refractory, in relapse post-transplant or in second or later relapse. · Adult patients with relapsed or refractory DLBCL after two or more lines of systemic therapy.	yes	yes	positive	23-8-2018	standard	authorised
Luxturna	voretigene neparvovec	GTMP – in vivo	ophthalmology	For the treatment of adult and paediatric patients with vision loss due to inherited retinal dystrophy caused by confirmed biallelic RPE65 mutations and who have sufficient viable retinal cells.	yes	no	positive	22-11-2018	standard	authorised
Zynteglo	betibeglogene autotemcel	GTMP – ex vivo	haematology	For the treatment of patients 12 years and older with TDT who do not have a β0/β0 genotype, for whom HSCT is appropriate but a HLA-matched related HSC donor is not available.	yes	yes	positive	29-5-2019	conditional	MA withdrawn 15-4-2022
Luxceptar	viable T-cells	CTMP	haemato-oncology	[Requested] As an adjunctive treatment to a haploidentical, T-cell depleted HSCT in adult patients with high-risk haematological malignancies in complete remission.	yes (at MAA)	no	withdrawn before opinion, 6-11-2019	-	-	-

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Zolgensma	onasemnogene abeparvovec	GTMP – in vivo	musculoskeletal disease	For the treatment of: · patients with 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the SMN1 gene and a clinical diagnosis of SMA Type 1, or · patients with 5q SMA with a bi-allelic mutation in the SMN1 gene and up to 3 copies of the SMN2 gene.	yes	yes	positive	18-5-2020	conditional	authorised (standard MA 15-5-2022)
Roctavian (2020)	valoctocogene roxaparvovec	GTMP – in vivo	haematology	[Requested] for the treatment of severe haemophilia A (congenital factor VIII deficiency)	yes (at MAA)	yes	withdrawn before opinion, 4-11-2020	-	-	-
Artobend	autologous human chondrocytes in vitro expanded	TEP	cartilage defect	[Requested] To repair defects in the cartilage of the knee in patients who are experiencing symptoms such as pain and problems moving the knee. For adults and children whose leg bones are no longer growing (growth-plate closure), when the affected area was no greater than 10 cm^2.	no (at MAA)	no	withdrawn before opinion, 18-11-2020	-	-	-
Tecartus	brexucabtagene autoleucel	GTMP – ex vivo	haemato-oncology	For the treatment of adult patients with relapsed or refractory mantle cell lymphoma (MCL) after two or more lines of systemic therapy including a Bruton's tyrosine kinase (BTK) inhibitor	yes	yes	positive	14-12-2020	conditional	authorised
Libmeldy	atidarsagene autotemcel	GTMP – ex vivo	metabolic disorder	For the treatment of metachromatic leukodystrophy (MLD) characterized by biallelic mutations in the arysulfatase A (ARSA) gene leading to a reduction of the ARSA enzymatic activity: · in children with late infantile or early juvenile forms, without clinical manifestations of the disease, · in children with the early juvenile form, with early clinical manifestations of the disease, who still have the ability to walk independently and before the onset of cognitive decline.	yes	no	positive	17-12-2020	standard	authorised
Skysona	elivaldogene autotemcel	GTMP – ex vivo	metabolic disorder	For the treatment of early cerebral adrenoleukodystrophy in patients less than 18 years of age, with an ABCD1 genetic mutation, and for whom a human leukocyte antigen (HLA)-matched sibling HSC donor is not available.	yes	yes	positive	16-7-2021	standard	MA withdrawn 18-11-2021

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Abecma	idecabtagene vicleucel	GTMP – ex vivo	haemato-oncology	For the treatment of 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	yes	positive	18-8-2021	conditional	authorised (standard MA 19-3-2024)
Breyanzi	lisocabtagene maraleucel	GTMP – ex vivo	haemato-oncology	For the treatment of adult patients with relapsed or refractory DLBCL, PMBCL and follicular lymphoma grade 3B (FL3B), after two or more lines of systemic therapy.	no	yes	positive	4-4-2022	standard	authorised
Carvykti	ciltacabtagene autoleucel	GTMP – ex vivo	haemato-oncology	For the treatment of 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	yes	positive	25-5-2022	conditional	authorised (standard MA 19-4-2024)
Upstaza	eladocagene exuparvovec	GTMP – in vivo	metabolic disorder	For the treatment of patients aged 18 months and older with a clinical, molecular, and genetically confirmed diagnosis of aromatic L-amino acid decarboxylase (AADC) deficiency with a severe phenotype.	yes	no	positive	18-7-2022	exceptional	authorised
Sitoiganap	autologous glioma tumor cells, inactivated	CTMP	oncology	[Requested] For the treatment of adult patients with recurrent glioma.	yes (at MAA)	no	withdrawn before opinion, 2-5-2022	-	-	-
Roctavian (2022)	valoctocogene roxaparvovec	GTMP – in vivo	haematology	For the treatment of severe haemophilia A (congenital factor VIII deficiency) in adult patients without a history of factor VIII inhibitors and without detectable antibodies to adeno associated virus serotype 5 (AAV5).	yes	yes	positive	24-8-2022	conditional	authorised
Ebvallo	tabelecleucel	CTMP	haemato-oncology	As monotherapy for treatment of adult and paediatric patients 2 years of age and older with relapsed or refractory Epstein-Barr virus positive post-transplant lymphoproliferative disease (EBV+ PTLD) who have received at least one prior therapy.	yes	yes	positive	16-12-2022	exceptional	authorised
Hemgenix	etranacogene dezaparvovec	GTMP – in vivo	haematology	For the treatment of severe and moderately severe haemophilia B (congenital factor IX deficiency) in adult patients without a history of factor IX inhibitors.	yes	yes	positive	20-2-2023	conditional	authorised

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Lumevoq	lenadogene nolparvovec	GTMP – in vivo	ophthalmology	[Requested] For the treatment of patients with vision loss due to Leber Hereditary Optic Neuropathy (LHON) caused by a confirmed G11778A mutation in the ND4 mitochondrial gene.	yes (at MAA)	no	withdrawn before opinion, 20-5-2023	-	-	-
Casgevy	exagamglogene autotemcel	GTMP – ex vivo	haematology	For the treatment of: · TDT in patients 12 years of age and older for HSCT is appropriate and a HLA-matched related HSC donor is not available. · severe sickle cell disease (SCD) in patients 12 years of age and older with recurrent vaso-occlusive crises (VOCs) for whom HSCT is appropriate and a HLA-matched related HSC donor is not available	yes	yes	positive	9-2-2024	conditional	authorised
Beqvez (previously Durveqtix)	fidanacogene elaparvovec	GTMP – in vivo	haematology	For the treatment of severe and moderately severe haemophilia B (congenital factor IX deficiency) in adult patients without a history of factor IX inhibitors and without detectable antibodies to variant AAV serotype Rh74.	yes (at MAA)	yes	positive	24-7-2024	conditional	MA withdrawn 16-5-2025
Vyjuvek	beremagene geperpavec	GTMP – in vivo	dermatology	For the treatment of wounds in patients with dystrophic epidermolysis bullosa (DEB) with mutation(s) in the collagen type VII alpha 1 chain (COL7A1) gene, from birth.	yes	yes	positive	25-4-2025	standard	authorised

cATMP: combined ATMP; CHMP: Committee for Medicinal Products for Human Use; CTMP: somatic cell therapy medicinal product; DLBCL: diffuse large B-cell lymphoma; GTMP: gene therapy medicinal product; HLA: human leukocyte antigen; HSCT: haematopoietic stem cell transplantation; HSC: haematopoietic stem cell; MA: marketing authorisation; PMBCL; primary mediastinal large B-cell lymphoma; TDT: transfusion-dependent β-thalassaemia; TEP: tissue engineered product.

** The indication as stated here is shortened for legibility purposes; the full official indication can be found in the corresponding Excel file.*