



Press Release N°62/2021

Advanced therapies: a new management model to quickly reach the patient's bedside; the on-going alliance between the ISS and ASSOBIOTEC-Federchimica

The 14 Advanced Therapies approved by EMA

Source IV Report ATMP Forum (update 31/08/2021)

ATMP	MA EU	COMPANY	INDICATION
ChondroCelect	05/10/2009	TiGenix NV	Repair of individual symptomatic cartilage lesions of the femoral condyle of the knee (Grade III or IV), International Cartilage Repair Society (ICRS) in adults.
Glybera	25/10/2012	uniQure biopharma B.V.	Lipoprotein lipase deficiency, LPLD) with severe or repeated attacks of pancreatitis in spite of low fat diet
Maci	27/06/2013	Vericel Denmark ApS	Repair of full-thickness symptomatic cartilage defects in human knees (Grade III and IV of the modified Outerbridge scale) of 3-20 cm ² in adult patients with mature skeleton
Provenge	06/09/2013	Dendreon UK Ltd	Treatment of symptomatic or slightly symptomatic metastatic castration-resistant prostate cancer (non visceral) in adult men for whom chemotherapy is not clinically indicated yet.
Holoclar	17/02/2015	Holostem Terapie Avanzate	Moderate to severe deficiency of limbus stem cells (defined by the presence of neovascularization in at least two quadrants of the cornea with unilateral or bilateral involvement of the central part of the cornea and severely damaged visual acuity) caused by physical or chemical burns of the eye. A biopsy requires at least 1-2 mm ² of undamaged limbus tissue.
Imlygic	16/12/2015	Amgen Europe B.V.	Treatment of adult patients affected by inoperable melanoma with regional or distal metastases (Stage IIB, IIC and IVM1a) without involvement of the bone, brain, lung or of other viscera.
Strimvelis	26/05/2016	Orchard Therapeutics (Netherlands) BV	Treatment of patients with Severe Combined Immunodeficiency due to Adenosine Deaminase for whom there is no compatible family donor of Haematopoietic Stem Cells (HSC) who shares the Human Leukocytic Antigene (HLA) antigen
Zalmoxis	18/08/2016	MolMed SpA	Additional treatment in Haploidentical Hematopoietic Stem Cell Transplant in adults with high risk hematologic malignant neoplasms
Spherox	10/07/2017	CO.DON AG	Repair of symptomatic articular cartilage defects of the femoral condyle and of the patella of the knee (Grade III or IV) with defects measuring up to 10 cm ² in adults.
ATMP	MA EU	COMPANY	INDICATION
Alofisel	23/03/2018	Takeda Pharma A/S	Treatment of complex perianal fistulas in adult patients with non-active/slightly active Crohn's Disease, in cases where the fistulas have not responded adequately to at least one conventional or biological treatment. Alofisel must be used only at the end of the conditioning of the fistulas.
Kymriah	22/08/2018	Novartis Europharm Limited	Kymriah is indicated for the treatment of: <ul style="list-style-type: none"> - paediatric patients and young adults up to the age of 25 included, with refractory B-cell Acute Lymphoblastic Leukaemia (ALL), relapse after transplant or second or more relapses;

			<ul style="list-style-type: none"> - adult patients with refractory or relapsing Diffuse Large B-Cell Lymphoma (DLBCL) after two or more lines of systemic treatment.
Yescarta	23/08/2018	Kite Pharma EU B.V./Gilead	Treatment of adult patients with refractory or relapsing Diffuse Large B-Cell Lymphoma (DLBCL) and Primitive Mediastinum Large B-Cell Lymphoma that are refractory to or have relapsed after two or more lines of systemic treatment
Luxturna	22/11/2018	Novartis Europharm Limited	Treatment of adult and paediatric patients with visual loss due to hereditary retinal dystrophy caused by confirmed bi-allelic mutations of the RPE65 gene and who have sufficient vital retinal cells.
Zynteglo	29/05/2019	Bluebird bio (Netherlands) B.V.	Treatment of patients aged 12 or more suffering from transfusion dependent beta-thalassemia (TDT) who have a β^0/β^0 genotype for which Hematopoietic Stem Cell (HSC) transplant is appropriate but there is no compatible family donor who shares the Human Leukocyte Antigen HLA
Zolgensma	18/05/2020	Novartis Gene Therapies EU Limited	<p>Treatment of:</p> <ul style="list-style-type: none"> - patients with Spinal Muscular Atrophy with a bi-allelic mutation of the SMN1 gene and a clinical diagnosis of SMA type-1, or - patients with SMA 5q with a bi-allelic mutation of the SMN1 gene and up to three copies of the SMN2 gene
Tecartus	14/12/2020	Kite Pharma EU B.V./Gilead	Treatment of adult patients with relapsing or resistant Mantle Cell Lymphoma (MCL) after two or more lines of systemic treatment which includes Bruton's Tyrosine Kinase (BTK) inhibitor.
Libmeldy	17/12/2020	Orchard Therapeutics (Netherlands) BV	<p>Metachromatic Leukodystrophy Treatment (MLD) characterized by bi-allelic mutations of the Arysulphatase A (ARSA) gene which entails a reduction in the enzymatic activity of ARSA;</p> <ul style="list-style-type: none"> -in children with the late infantile form and the juvenile form, without the clinical manifestations of the disease; -in children with the early juvenile form, who preserve the ability to walk autonomously and before the start of cognitive decline
Skysona	16/07/2021	Bluebird bio (Netherlands) B.V.	Treatment of early Cerebral Adrenoleukodystrophy (CALD) in patients below the age of 18, with mutation of the ABCD1 gene for whom there is no compatible family donor of Haematopoietic Stem Cells (HSC) who shares the Human Leukocyte Antigen HLA
Abecma	18/18/2021	Celgene Europe BV	Adult patients with relapsing resistant Multiple Myeloma treated with at least 3 therapies including an immunomodulatory agent, a proteasome inhibitor and an anti-CD38 antibody, with evidence of disease progression after the last treatment.

ATMP in the process of being approved by the EMA

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ATMP	COMPANY	INDICATION	STARTING DATE OF CAT ASSESSMENT
Upstaza <i>Eladocagene exuparvovec</i>	PTC Therapeutics	Aromatic L-Amino acid Decarboxylase deficiency (AADC)	19/02/2020
Breyanzi <i>Lisocabtagene maraleucel (liso-cel)</i>	Bristol Myers Squibb	Adults with relapsing resistant form of Diffuse Large B-cell Lymphoma (DLBCL), Primary Mediastinum Large B-cell Lymphoma (PMBCL) and Follicular Lymphoma Grade III B (FL3B)	09/09/2020
Gliovac <i>Glioma inactivated autologous and allogeneic cells and lysates of autologous cells and of glioma inactivated allogeneic cells</i>	Epitopoietic Research Corporation	Glioma	07/10/2020
Lumevoq <i>lenadogene nolparvavec</i>	GenSight Biologics	Treatment of the loss of eye-sight in patients with Leber's Hereditary Optical Neuropathy (LHON) with mutation of the ND4 mitochondrial gene	01/12/2020
Ciltacabtagene <i>Autoleucel (cilta-cel)</i>	Janssen	Refractory and relapsing Multiple Myeloma	16/06/2021
Roctavian <i>Valoctocogene roxaparvavec</i>	Biomarin	Haemophilia-A	14/07/2021