Table 1: Name, type, indication, manufacture and efficacy of ATMPs licensed for use across the UK

Name of ATMP	Type of ATMP	Indication	Product manufacture and administration	Efficacy (major outcome trials)
Atidarsagene autotemcel (Libmeldy)	Gene therapy medicinal product	Metachromatic leukodystrophy with mutations in the arylsulfatase A (ARSA) gene	• Autologous gene therapy manufactured with patient's own CD34+ stem cells, collected by leukapheresis or bone-marrow harvest, which are modified *ex vivo* using a lentiviral vector to insert a functional ARSA gene; • Single-dose administration.	Results indicate it is effective in modifying the disease course of early-onset metachromatic leukodystrophy in most patients
Autologous anti-CD19-transduced CD3+ cells (Tecartus)	Gene therapy medicinal product	Relapsed or refractory mantle cell lymphoma	• Autologous CAR-T cell therapy manufactured using the patient's own T-lymphocytes collected by leukapheresis; • The T cells are genetically modified *ex vivo* using a retroviral vector to produce anti-CD19 CAR-T cells; • The CAR-T cells are expanded and infused back into patient; • Single-dose administration.	In the phase 2 ZUMA-2 trial, after a minimum of 7 months of follow up, 93% of patients had an objective response, with 67% achieving complete response
Axicabtagene ciloleucel (Yescarta)	Gene therapy medicinal product	Relapsed/refractory diffuse large B-cell lymphoma and primary (DLBCL) mediastinal large B-cell lymphoma following two or more previous systemic treatments	• Product manufacture is similar to that described for Tecartus; • Single-dose administration.	In the phase 2 ZUMA-1 trial, after a median follow up of 24 months, 74% of patients achieved an objective response and 54% a complete response
Tisagenlecleucel (KYMRIAH)	Gene therapy medicinal product	Relapsed or refractory B-cell acute lymphoblastic leukaemia (r/r ALL) in patients aged under 25 years Refractory DLBCL after more than 2 previous therapies	• Autologous CAR-T cell therapy manufactured using the patient's own T-lymphocytes collected by leukapheresis. The T cells are genetically modified *ex vivo* using a lentiviral vector encoding an anti-CD19 CAR protein; • Single-dose administration.	• In the ELIANA trial, children and young adults with r/r ALL had an overall remission rate of 82.3%; • In the JULIET trial, adult patients with DLBCL demonstrated an overall response rate 53% at a median follow-up of 40.3 months.
Talimogene laherparepvec (Imlygic)	Gene therapy medicinal product	Adult patients with unresectable metastatic melanoma	• Produced by recombinant DNA technology in Vero cells; • The exact mechanism of action is unknown, although when talimogene is injected into melanoma, it causes tumor lysis and release of tumor-derived antigens. This together with virally derived granulocyte-macrophage colony-stimulating factor (GM-CSF) promotes an anti-tumor immune response.	In the OPTiM study, talimogene laherparepvec monotherapy versus GM-CSF showed a statistically significant durable response rate (19% vs. 1.4%) and overall response rate (31.5% vs. 6.4%). Median overall survival was 23.3 versus 18.9 months. 50 patients (16.9%) vs 1 patient (0.7%) achieved complete response in each group
Autologous human corneal epithelial cells (Holoclar)	Tissue-engineered medicinal product	Adult patients with moderate to severe limbal stem cell deficiency	• Autologous limbal stem cells. Collected at biopsy, expanded and implanted into the cornea; • Process takes several weeks.	The HLSTM01 trial reported 72.1% of patients had successful Holoclar transplant at 12 months after therapy
Voretigene neparvovec (Luxturna)	Gene therapy medicinal product	Inherited retinal dystrophies caused by RPE65 gene mutations	Extracted from naturally occurring adeno-associated virus and recombinant DNA techniques	The phase 3 301 study reported improvement in the visual acuity of at least 0.3 logMAR in 55% of first-treated eyes and 20% of second-treated eyes after 12 months
Autologous CD34+ enriched cell fraction containing CD34+ cells transduced with retroviral vector encoding for human adenosine deaminase cDNA sequence (Strimvelis)	Gene therapy medicinal product	Adenosine deaminase deficiency (severe combined immunodeficiency)	• Autologous bone-marrow-derived cells (CD34+ cells) collected and modified to make functional adenosine deaminase enzyme; • Single-dose administration.	The AD1115611 trial reported a 100% survival rate at 3 years
Autologous chondrocyte implantation (ACI) using chondrosphere (Spherox)	Tissue-engineered medicinal product	Symptomatic articular cartilage defects of the knee	• Patient's own chondrocytes isolated from healthy cartilage then cultured *in vitro* for autologous use; • Entire process takes six to eight weeks.	A phase 3 licensing study demonstrated the non-inferiority of ACI (compared with microfracture) at 60 months follow-up
Onasemnogene abeparvovec (Zolgensma)	Gene therapy medicinal product	Spinal muscular atrophy	• Derived from human embryonic kidney cells by r-DNA technology; • Single-dose administration.	In the AVXS-101-CL-303 study, 21/22 patients survived event-free (without permanent ventilation) to ≥10.5 months of age and 20 survived to 18 months of age