



Advanced Therapies in Wales

Availability, Access and Utilisation

2019/20 – 2025/26

Executive Summary

This report examines the availability, access and utilisation of advanced therapies in Wales between 2019/20 and 2025/26.

Between 2019/20 and 2025/26, access to advanced therapies in Wales expanded significantly, reflecting the growing availability of innovative gene and cell-based treatments within the NHS. Although the current treatment portfolio remains dominated by CAR-T therapies for blood cancers, advanced therapies are increasingly being used to treat a wider range of rare genetic, neurological, ophthalmological and inherited conditions. As of April 2026, NICE had issued 19 positive recommendations covering 15 advanced therapies across 14 disease indications, all of which are available to Welsh patients through national commissioning arranged by the NHS Wales Joint Commissioning Committee.

Use of advanced therapies has grown rapidly over the reporting period, with the number of annual treated patients increasing from 8 in 2019/20 to 29 in 2025/26. In total, 131 Welsh patients received an advanced therapy, the vast majority for haematological cancers. Patients receiving treatment were drawn from all age groups and all seven health boards, demonstrating national access to these highly specialised therapies. While most treatments were delivered within Wales, established cross-border specialist networks remain essential to ensuring timely access to services that cannot be sustainably provided locally.

Overall, the findings suggest that national commissioning arrangements have enabled equitable access to advanced therapies for patients across Wales, despite the challenges associated with rare conditions, small patient numbers and the need for highly specialised treatment centres. However, further work is needed to better understand variation in referral pathways, treatment uptake and equity of access. Continued growth in the advanced therapy pipeline is likely to increase demand further, highlighting the importance of ongoing service planning, workforce development, data collection and monitoring to ensure Welsh patients continue to benefit from these transformative treatments.

Availability of NICE-Recommended Advanced Therapies

For the purpose of this report, Advanced Therapies refer to Advanced Therapy Medicinal Products (ATMPs) that use genes, cells or tissues to target the root cause of disease. Definitions of ATMPs can be found in Appendix 1. Like all new medicines, ATMPs are assessed by the [Medicines and Healthcare products Regulatory Agency](#) (MHRA) for safety, quality and effectiveness before being appraised by the [National Institute for Health and Care Excellence](#) (NICE) for clinical and cost effectiveness. Treatments that receive a positive NICE recommendation are commissioned by the NHS Wales Joint Commissioning Committee (NWJCC) on behalf of all seven health boards, ensuring equitable access across Wales. Advanced therapies accessed through clinical trials are not commissioned by NWJCC and are therefore outside the scope of this report.

As of April 2026, NICE has issued 19 positive recommendations covering 15 advanced therapies across 14 disease indications. The full list of NICE recommendations and disease areas can be found in Appendix 2. The number of available therapies continues to grow, with a substantial pipeline of new treatments expected in the coming years.

The majority of advanced therapy recommendations published by NICE have enabled patient access within the NHS (Figure 1). However, two treatments were not recommended for routine use: darvadstrocel (Alofisel) for treating complex perianal fistulas in Crohn's disease ([TA556](#), January 2019), and axicabtagene ciloleucel (Yescarta) for treating relapsed or refractory follicular lymphoma ([TA894](#), June 2023). Several other advanced therapies have entered the NICE work programme but have resulted in terminated or discontinued appraisals, typically where the company did not complete, or withdrew its evidence submission.

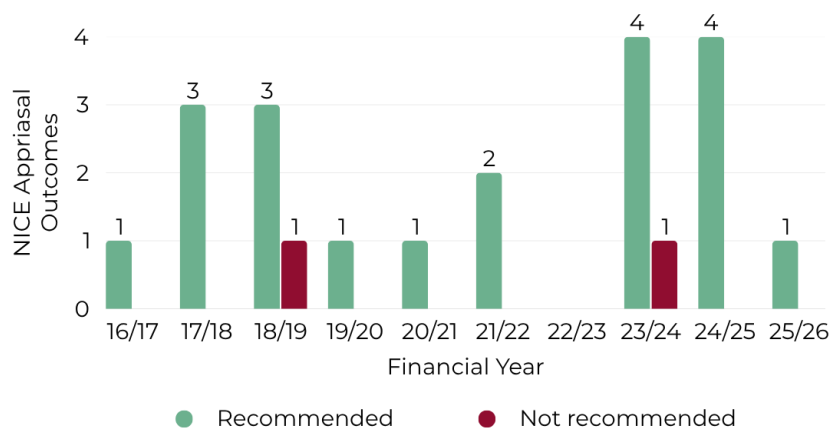


Figure 1 – Annual NICE Appraisal Outcomes for Advanced Therapies by Financial Year (2016/17 – 2025/26)

Figure 2 shows the cumulative distribution of NICE-recommended advanced therapies by ATMP type. Most recommendations relate to *ex vivo* gene therapies, followed by *in vivo* gene therapies. There are currently two recommended tissue engineered products and no recommended somatic cell therapies.

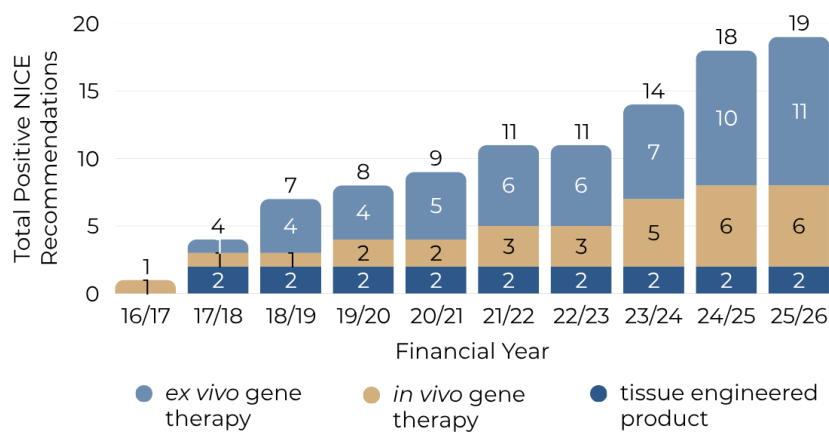


Figure 2 – Total Positive NICE Recommendations for Advanced Therapies by Financial Year (2016/17 – 2025/26)

Most NICE-recommended advanced therapies are currently used to treat blood cancers. However, treatments are also available for a growing range of conditions in areas including neurology, ophthalmology, inherited blood disorders and rare genetic diseases (Table 1). The majority of recommendations relate to *ex vivo* gene therapies, particularly CAR-T therapies for blood cancers (Figure 3).

Speciality	16/17	17/18	18/19	19/20	20/21	21/22	22/23	23/24	24/25	25/26
Oncology	1	1	1	1	1	1	1	1	1	1
Orthopaedics	0	1	1	1	1	1	1	1	1	1
Immunodeficiency	0	1	1	1	1	1	1	1	1	1
Ophthalmology	0	1	1	2	2	2	2	2	2	2
Haemato-oncology	0	0	3	3	4	4	4	5	6	7
Neurology	0	0	0	0	0	2	2	4	4	4
Haematology	0	0	0	0	0	0	0	0	3	3
All	1	4	7	8	9	11	11	14	18	19

Table 1 – NICE Recommended Advanced Therapies by Clinical Speciality and Financial Year

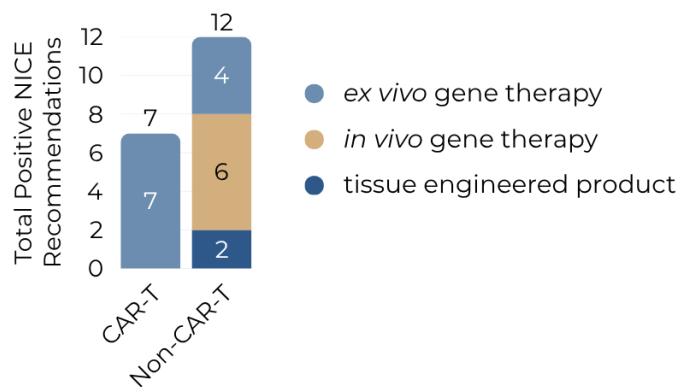


Figure 3 – NICE Recommended Advanced Therapies by ATMP Type

Activity and Utilisation

Patient numbers have increased steadily since the introduction of nationally commissioned advanced therapies in Wales. Although some treatments were available from 2016, this report focuses on activity from the 2019/2020 financial year onwards. This reflects the introduction of CAR-T therapies and the start of routine national commissioning of advanced therapies by the Welsh Health Specialised Services Committee (WHSSC), now the NHS Wales Joint Commissioning Committee (NWJCC).

Between 1st April 2019 and 31st March 2026, 131 patients received an NWJCC-commissioned advanced therapy, with annual patient numbers increasing by 263%, from 8 patients in 2019/20 to 29 patients in 2025/26 (Figure 4).

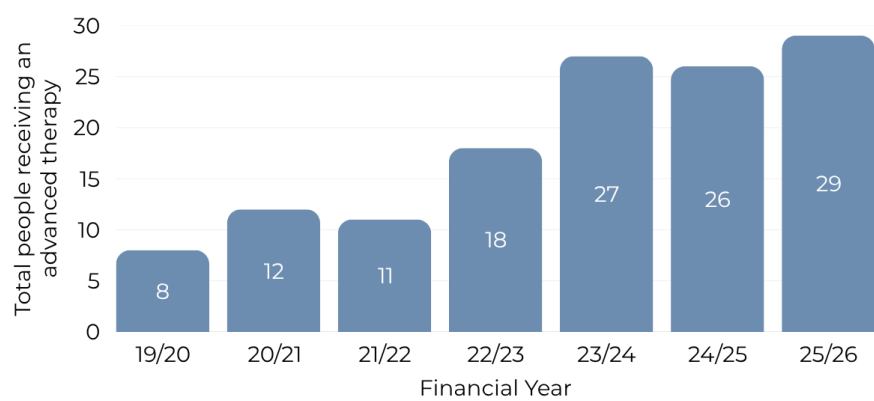


Figure 4 – Number of People Treated with Advanced Therapies by Financial Year

Most treatment activity related to blood cancers, with 123 patients (94%) receiving CAR-T therapy for B-cell lymphomas, B-cell Acute Lymphoblastic Leukaemia (ALL) or Mantle Cell Lymphoma (MCL). The remaining 8 patients (6%) received an *in vivo* gene therapy for rare non-cancer conditions, including Spinal Muscular Atrophy (SMA), retinal dystrophy or haemophilia B (Figure 5).

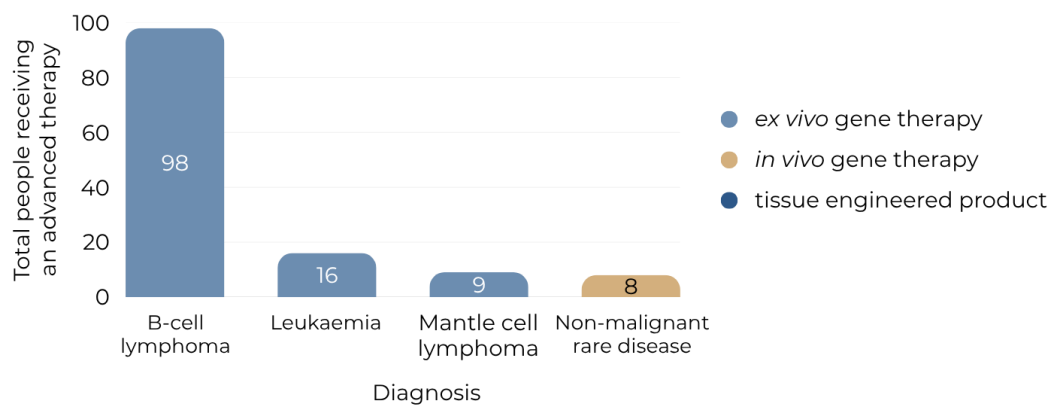


Figure 5 – Number of People Treated with Advanced Therapies by Diagnosis and Treatment Type

Patient Demographics

Patients ranged from infants to older adults, although most treatments were given to older people (Figure 6). Three quarters of patients (98 of 131) were aged 50 years or over. All age groups were represented, including 11 children aged under 10 years who received treatment for childhood cancers or rare genetic conditions.

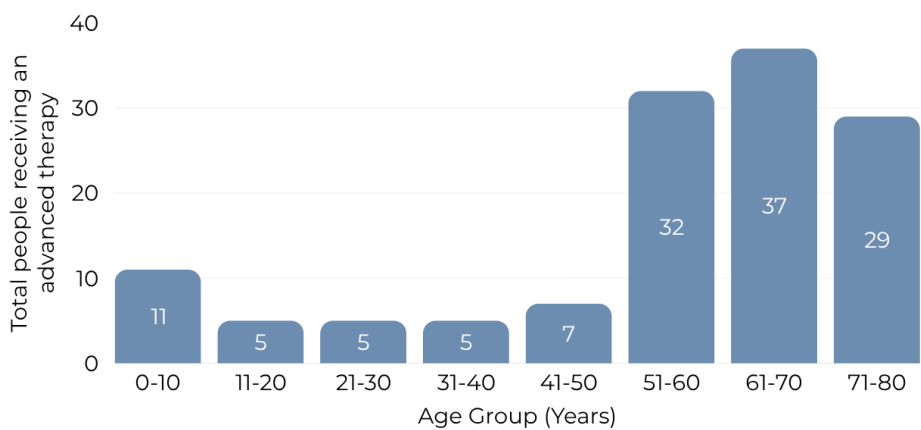


Figure 6 – Number of People Treated with Advanced Therapies by Age Group

The treated population was predominantly male, with 89 men (68%) and 42 women (32%) receiving treatment (Figure 7). This largely reflects the current treatment portfolio, which is dominated by CAR-T therapies for blood cancers such as diffuse large B-cell lymphoma, a condition that becomes more common with age and is slightly more common in men¹. Overall, the patient population receiving advanced therapies in Wales reflects the current concentration of commissioned treatments within haemato-oncology, while also demonstrating the ability of these innovative therapies to treat rare and severe conditions across all stages of life.

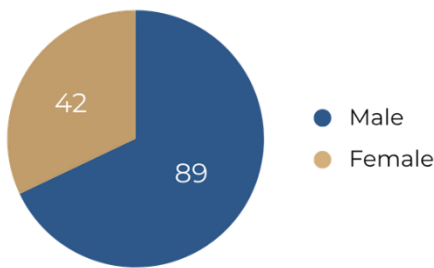


Figure 7 – Number of People Treated with Advanced Therapies by Sex

¹ <https://lymphoma-action.org.uk/sites/default/files/media/documents/2025-07/LYMweb0018DLBCL2025v11.pdf>

Delivery of Advanced Therapies in Wales

Currently, five of the nineteen positive NICE-recommendations are delivered within Wales. The South Wales Blood and Marrow Transplant Programme (SWBMT) provides CAR-T therapies for adults with large B-cell lymphoma (second- and third-line treatment) and mantle cell lymphoma, using lisocabtagene maraleucel, axicabtagene ciloleucel and brexucabtagene autoleucel. These services are provided for patients from six of the seven health boards in Wales. The haematology team at University Hospital Wales also delivers the gene therapy etranacogene dezaparvovec for haemophilia B for patients from South Wales. Patients from Betsi Cadwaladr University Health Board can access both CAR-T and etranacogene dezaparvovec via a closer, designated specialist centre in England.

Although these therapies are routinely available within Wales, patients may occasionally be referred to alternative designated centres within the UK to manage service capacity to ensure timely access to treatment. All other advanced therapies commissioned by NWJCC are currently accessed through specialist centres outside Wales. As many of these therapies treat very rare conditions, patient numbers are often too small to support sustainable local services, while others require highly specialist expertise or facilities that are not currently available in Wales. UK specialist centres provide the expertise and facilities needed to deliver these treatments safely and effectively.

Treatment Location and Distance Travelled

During the reporting period, 80 patients (61%) received treatment in Wales, while 51 patients (39%) received treatment in England (Figure 8A). This reflects established UK-wide commissioning arrangements and the concentration of expertise and infrastructure required to safely deliver these highly specialised treatments. Despite the need for patients to travel to specialist centres, most travelled less than 60 miles to access treatment (Figure 8B). Approximately one-third of patients (43; 33%) travelled less than 30 miles, while a further 47 patients (36%) travelled between 31 and 60 miles. Longer journeys were less common, with only 20 patients (15%) travelling over 100 miles for treatment.

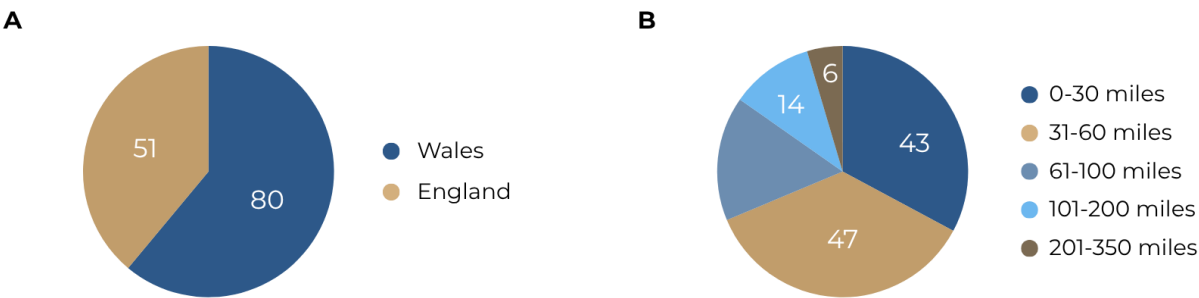


Figure 8 – Treatment Location and Distance Travelled by Patients Receiving Advanced Therapies

These findings demonstrate the importance of regional and cross-border specialist treatment networks in enabling access to advanced therapies for Welsh patients. While over a third of treatments were delivered outside Wales, almost 70% of patients travelled fewer than 60 miles to receive treatment. However, these data relate

only to patients who ultimately received an advanced therapy and do not capture individuals who may have declined treatment, withdrawn from the pathway or experienced other barriers to access. Further work is therefore required to understand the extent to which travel distance and treatment location may influence referral patterns, treatment uptake and equity of access.

Geographical Distribution

Patients who received advanced therapies were resident in all seven health board areas, demonstrating national access to these highly specialised treatments (Figure 9). Aneurin Bevan University Health Board (ABUHB) accounted for the largest number of treated patients (41, 31%) followed by Cardiff and Vale University Health Board (CVUHB) and Betsi Cadwaladr University Health Board (BCUHB) (23 patients each; 17.5%).

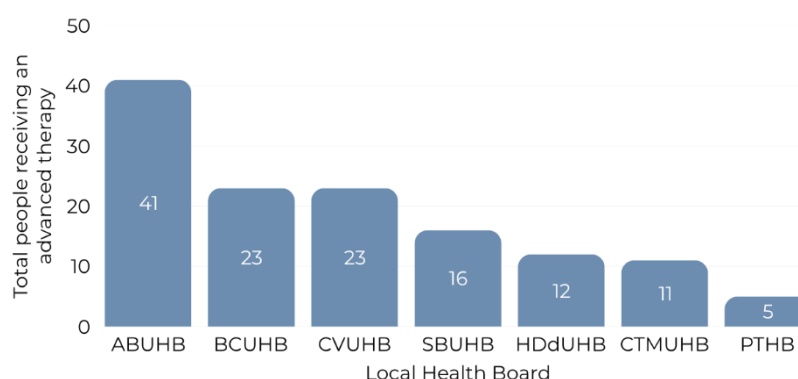


Figure 9 – Number of People Treated with Advanced Therapies by Local Health Board

To account for differences in population size between health boards, treatment rates were calculated using average resident populations² (Table 2). Across Wales, 131 patients received an advanced therapy during the reporting period, equivalent to a treatment rate of 4.20 patients per 100,000 population. Rates varied between health boards, ranging from 2.21 patients per 100,000 population in Cwm Taf Morgannwg University Health Board (CTMUHB) to 6.96 patients per 100,000 population in ABUHB.

Although variation in treatment rates was observed between health boards, patients from all regions of Wales accessed advanced therapies during the reporting period. Given the small patient numbers and the rarity of many eligible conditions, differences in treatment rates should be interpreted with caution. The analyses include only patients who received an advanced therapy and do not capture all individuals who were referred, assessed or considered for treatment. As a result, it does not yet provide a complete picture of variation in access across Wales. Further work is planned to better understand referral patterns and equity of access.

² Population data from Digital Health and Care Wales – Average Health Board Populations between 2019-2023

Health Board	Average Population	Patient count	Rate per 100,000
Aneurin Bevan University Health Board	589,354	41	6.96
Cardiff and Vale University Health Board	443,383	23	5.19
Swansea Bay University Health Board	383,021	16	4.18
Powys Teaching Health Board	133,118	5	3.76
Betsi Cadwaladr University Health Board	688,242	23	3.34
Hywel Dda University Health Board	383,117	12	3.13
Cwm Taf Morgannwg University Health Board	498,751	11	2.21
All Wales	3,118,985	131	4.20

Table 2 – Number and Rate of People Treated with Advanced Therapies by Local Health Board

Next Steps

This report provides an overview of the use of advanced therapies in Wales and establishes a baseline for future monitoring. As the number of available therapies continues to increase, further work is required to better understand equity of access across Wales.

Future analyses should move beyond treated patients to explore diagnostic, referral and treatment pathways, helping to understand the variation between regions and identify potential barriers to access, including factors that may influence referral, treatment uptake and eligibility for advanced therapies. In parallel, a work package focused on real-world outcomes following advanced therapies is progressing well. However, the small patient numbers associated with many advanced therapies currently limit the scope for external reporting. As patient cohorts grow and appropriately aggregated analyses become feasible, outcomes data may be incorporated into future editions of this report. Early learning has also highlighted opportunities to strengthen monitoring through prospective data collection at source, particularly in relation to treatment and outcomes prior to receipt of an advanced therapy.

Alongside this, further work is planned to understand current delivery models for advanced therapies, assess opportunities for local adoption or repatriation of services where clinically appropriate and sustainable, and support strategic planning for future service provision in Wales. This work will be undertaken in collaboration with Advanced Therapies Wales to ensure planning reflects the evolving advanced therapy landscape and anticipated future demand.

This report is intended to be published annually, with data updated for each financial year. Regular reporting will support ongoing monitoring of activity, access and service development, while providing an evidence base to inform commissioning and strategic planning for advanced therapies in Wales.

Appendix 1

What are Advanced Therapies?

For the purpose of this report, Advanced Therapies refer to Advanced Therapy Medicinal Products (ATMPs) that use genes, cells or tissues to target the root cause of disease. The Human Medicines Regulations 2012³ defines three types of ATMP: gene therapies, somatic cell therapies and tissue engineered products. These categories reflect whether a treatment works by introducing modified genetic material, using living cells, or repairing and regenerating tissues. In practice, some therapies combine these approaches. For clarity and consistency within this report, advanced therapies are grouped using the following simplified definitions:

- **ex vivo gene therapies**
 - cell-based therapies that involve removing cells from the body and genetically modifying them in a laboratory before returning them to the patient, enabling them to repair, replace or adjust how genes function to help treat or prevent disease.
- **in vivo gene therapies**
 - non-cell-based therapies that involve delivering genetic material directly into the body to repair, replace or adjust how genes function, helping to treat or prevent disease.
- **somatic cell therapies**
 - contains cells or tissues that have been modified or used differently from their original purpose, so they can treat, prevent or diagnose disease through their biological activity.
- **tissue engineered products**
 - contains cells or tissues that have been modified or used differently from their original purpose, so they can regenerate, repair or replace damaged or diseased tissue in the body.

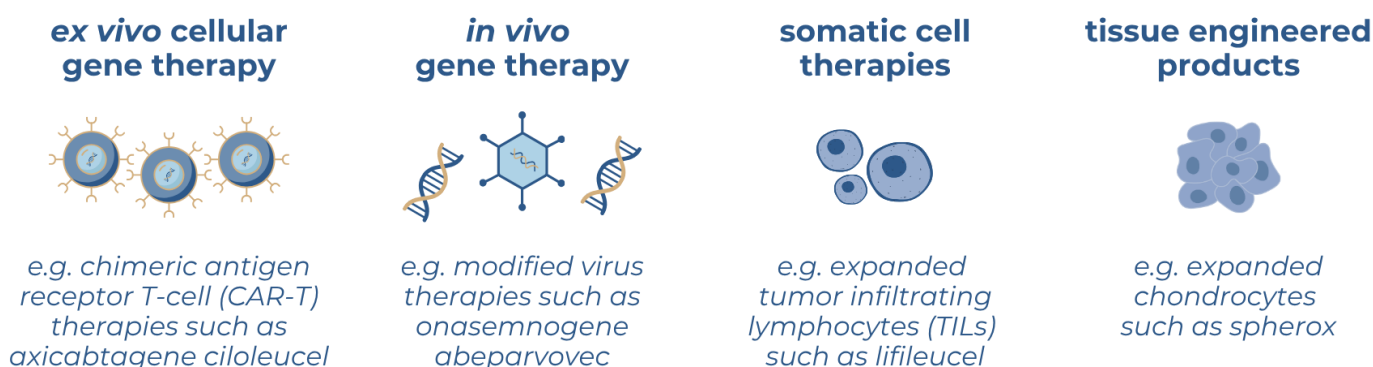


Figure S1 – Types of Advanced Therapy

³ <https://www.legislation.gov.uk/uksi/2012/1916/regulation/2A>

Appendix 2

Current NICE Recommended Advanced Therapies (April 2026)

Financial Year	Generic Name	Brand Name	Speciality	Indication	NICE ID
25/26	Obecabtagene autoleucl	Aucatzyl	Haemato-oncology	Acute Lymphoblastic Leukaemia (26+)	TA1116
24/25	Lisocabtagene maraleucl	Breyanzi	Haemato-oncology	Large B-Cell Lymphoma (2 nd line)	TA1048
24/25	Exagamlogene autotemcel	Casgev	Haematology	Sickle Cell Disease (12+)	TA1044
24/25	Exagamlogene autotemcel	Casgev	Haematology	Beta Thalassaemia (12+)	TA1003
24/25	Etranacogene dezaparvovec	Hemgenix	Haematology	Haemophilia B	TA989
23/24	Axicabtagene ciloleucl	Yescarta	Haemato-oncology	Large B-Cell Lymphoma (2 nd line)	TA895
23/24	Brexucabtagene autoleucl	Tecartus	Haemato-oncology	Acute Lymphoblastic Leukaemia (26+)	TA893
23/24	Eladocogene exuparvovec	Upstaza	Neurology	AADC Deficiency	HST26
23/24	Onasemnogene abeparvovec	Zolgensma	Neurology	Spinal Muscular Atrophy	HST24
21/22	Atidarsagene ciloleucl	Libmeldy	Neurology	Metachromatic Leukodystrophy	HST18
21/22	Onasemnogene abeparvovec	Zolgensma	Neurology	Spinal Muscular Atrophy	HST15
20/21	Brexucabtagene autoleucl	Tecartus	Haemato-oncology	Mantle Cell Lymphoma	TA677
19/20	Voretigene neparvovec	Luxturna	Ophthalmology	Retinal Dystrophy RPE65	HST11
18/19	Axicabtagene ciloleucl	Yescarta	Haemato-oncology	Large B-Cell Lymphoma (3 rd line)	TA559/TA872
18/19	Tisagenlecleucl	Kymriah	Haemato-oncology	Acute Lymphoblastic Leukaemia (25-)	TA554/TA975
17/18	Spherox	Spherox	Orthopaedics	Cartilage Defects of the Knee	TA508
17/18	Strimvelis	Strimvelis	Immunodeficiency	ADA-SCID	HST7
17/18	Holoclal	Holoclal	Ophthalmology	Eye Burns	TA467
16/17	Talimogene laherparepvec	Imlygic	Oncology	Metastatic Melanoma	TA410

Figure S2 – Table of NICE-Recommended Advanced Therapies (April 2026)