HTA376

Challenge of ATMP Reimbursement and Patient Access in a Changing UK Landscape

Ben Barron-Millar, PhD; Malcolm Boyd, MSc; Ruth Howells, MSc.
Kintiga, United Kingdom.

Objectives

- Advanced Therapy Medicinal Products (ATMPs), including gene therapies, somatic cell therapies and tissue-engineered products, represent a rapidly expanding class of innovative treatments with the potential to deliver substantial and, in some cases, durable clinical benefit for patients with rare diseases, cancers and other high unmet need conditions.
- The UK is well positioned to support ATMP adoption through its integrated healthcare system, established genomics infrastructure, specialist clinical networks, and internationally recognised regulatory and HTA evaluation organisations.
- Despite these strengths, translating ATMP innovation into routine patient access remains challenging, reflecting the need to align evidence generation, reimbursement decision-making, commercial considerations and healthcare system implementation. England has implemented flexible appraisal pathways and managed access mechanisms, to enable access while mitigating considerable uncertainty.
- This study describes data identified by a detailed HTA review for NICE reimbursement outcomes for ATMPs in England along with evidence submitted, key drivers of uncertainty and decision-making.

Methods

Identification of ATMP NICE appraisals:

- A retrospective HTA trend analysis of ATMP NICE reimbursement outcomes was conducted up to September 2026.
- Marketing authorisation information in UK was sourced from the MHRA register.

Analysis on national published NICE HTA guidance:

- National HTA assessments and outcomes were analysed by screening databases from NICE publicly available records. This involved both quantitative research and qualitative desk research to examine the submitted evidence and HTA outcomes within the appraisals listed.
- Variables extracted included therapeutic area, indication, MA, NICE appraisal pathway, reimbursement outcome, use of managed access, and reported decision drivers.

Results: ATMP NICE reimbursement outcomes

Table 1: MHRA-approved ATMP drugs market access landscape in UK

Generic / active substance	Brand name	NICE
Autologous corneal epithelial cells	Holoclar®	TA467
Talimogene laherparepvec	Imlygic®	TA410
ADA-transduced autologous CD34+ cells	Strimvelis®	HST7
Autologous chondrocyte spheroids	Spherox®	TA508
Axicabtagene ciloleucel	Yescarta®*	TA895: CDF
		TA872: CDF
Tisagenlecleucel	Kymriah®	TA975: MAA
		CDF • TA933 Terminated
Voretigene neparvovec	Luxturna®	HST11
Onasemnogene abeparvovec	Zolgensma®	HST15: MAA
Brexucabtagene autoleucel	Tecartus®	TA677: CDF
		TA893: CDF
Atidarsagene autotemcel	Libmeldy®	HST18
Lisocabtagene maraleucel	Breyanzi®	TA1048
		TA1159
Eladocagene exuparvovec	Upstaza®	HST26
Etranacogene dezaparvovec	Hemgenix®	TA989: IMF
Exagamglogene autotemcel	Casgevy®	TA1003: IMF
		TA1044: IMF
Obecabtagene autoleucel	Aucatzyl®	TA1116
Beremagene geperpavec	Vyjuvek®	In development
Rebisufligene etisparvovec	UXIII	In development
Ciltacabtagene autoleucel	Carvykti®	Terminated
Tabelecleucel	Ebvallo®	Terminated
Idecabtagene vicleucel	Abecma®	Terminated

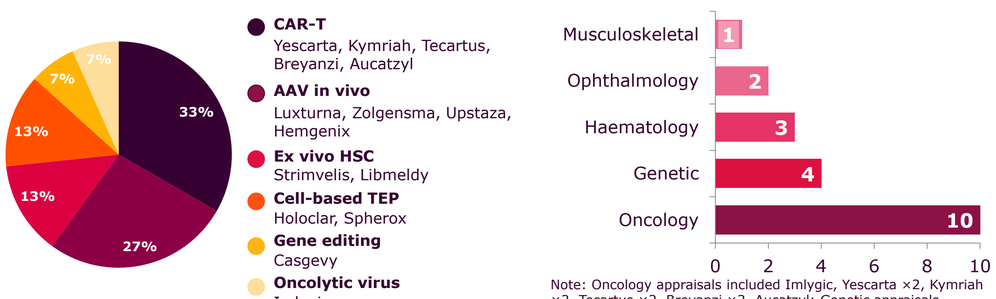
Positive recommendation | Restricted / Optimised indication | Managed access | In development/Terminated

- 15 ATMP therapies are approved for use in the UK by the MHRA and reimbursed by the NHS, two NICE topics are in development, and three were terminated (Table 1).
- 19 reimbursement decisions by NICE including 14 standard technology appraisals and 5 HST appraisals.
- 10 appraisals adopting a managed access period for further data collection with funding from CDF, IMF and other routes.
- 11 of currently reimbursed ATMP drugs have MHRA orphan designation.
- A majority adopted a restricted indication ('optimised') compared to marketing authorisation.

Note: *Yescarta: TA895 consists of CDF-enabled managed access; TA872 recommended routine commissioning after CDF period.
Abbreviations: ATMP, advanced therapy medicinal product; CD34+, cluster of differentiation 34-positive; CDF, Cancer Drugs Fund; HST, highly specialised technology; HTA, health technology assessment; IMF, Innovative Medicines Fund; MHRA, Medicines and Healthcare products Regulatory Agency; NICE, National Institute for Health and Care Excellence; STA, single technology appraisal.

Results: Product and indication profile

Figure 1: ATMP product and indication profile in UK



Abbreviations: AAV, adeno-associated virus; CAR-T, chimeric antigen receptor T-cell; HSC, haematopoietic stem cell; TEP, tissue-engineered product.

Note: Oncology appraisals included Imlygic, Yescarta ×2, Kymriah ×2, Tecartus ×2, Breyanzi ×2, Aucatzyl; Genetic appraisals included Strimvelis, Zolgensma, Libmeldy, Upstaza; Haematology apppraisals included Hemgenix, Casgevy ×2; Ophthalmology appraisals included Holoclar and Luxturna; Musculoskeletal appraisal included Spherox.

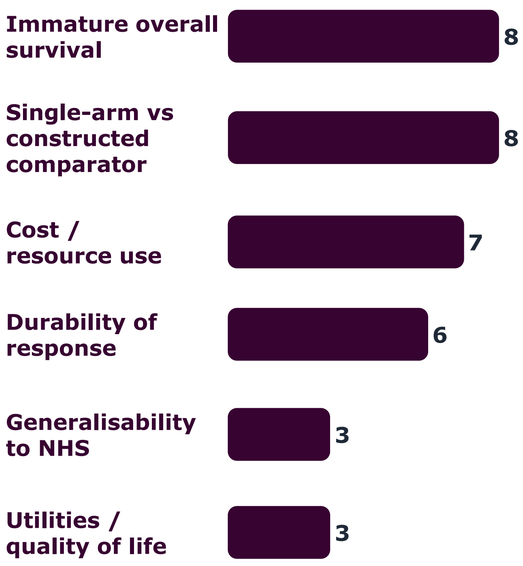
Figure 2: Population restriction and NICE reimbursement outcomes

Restriction	Evidence Implication	Appraisal Outcome
Trial-population alignment TA895 Yescarta 2L, TA1044 Casgevy, TA1048 Breyanzi 2L	Excludes unrepresented or unmodelled patients. Improves internal validity, limits generalisability.	CDF/IMF or routine access only for the evidenced population.
Clinical eligibility criteria HST15 Zolgensma, TA467 Holoclar, HST18 Libmeldy	Targets likely responders. Limits extrapolation to older, bilateral or later-stage patients.	Optimised access with MDT, age, disease-stage or prior-treatment controls.
ICER-driven subgroup exclusion TA1116 Aucatzyl, TA467 Holoclar	Separate subgroup assessment. Weak evidence or unfavourable ICER prevents inclusion.	Narrower positive outcome instead of broader non-recommendation risk.
Comparator/line alignment TA410 Imlygic, TA508 Spherox, TA1159 Breyanzi	Avoids unreliable comparisons or matches an established reimbursed comparator population.	Access only where comparative value can be established.
Managed-access eligibility HST15 Zolgensma, TA1044 Casgevy SCD	Defined population and data collection address model robustness, durability and NHS outcomes.	Conditional access. Zolgensma later moved to routine access. Casgevy remains under review.

Abbreviations: 2L, second-line; CDF, Cancer Drug Fund; HST, highly specialised technology; ICER, incremental cost-effectiveness ratio; IMF, Innovative Medicine Fund; MDT, multidisciplinary team; NICE, National Institute for Health and Care Excellence; SCD, sickle cell disease.

Results: ATMP and managed access insights

Figure 3: Managed access: Driven by uncertainty



- Managed access agreements were funded by CDF (N=6), IMF (N=3) and HST route (N=1).
- N=5 appraisals have exited MAA or reached review stage ahead of resubmission.
- N=5 MAA are still ongoing.

NICE expanded data collection plans:
- SACT database
- Blueteq reports
- Registry data
- Utilisation data.

Figure 4: Addressing uncertainty through managed access – not guaranteed

COMPLETED MANAGED-ACCESS EXITS

Yescarta R/R DLBCL
TA559 → TA872
- OS matured
- SACT validated survival

ROUTINE

Kymriah B-ALL
TA554 → TA975
- OS matured
- MAIC partly uncertain
- IVIG informed

ROUTINE

Zolgensma SMA
HST15 → HST24
- SPR1NT evidence supported presymptomatic use ≤12 months

ROUTINE

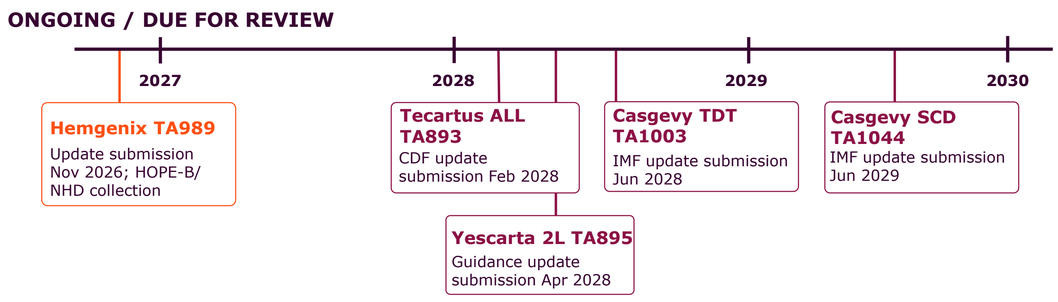
Kymriah DLBCL
TA567 → TA933
- Data collected but not assessed: no submission

TERMINATED

Tecartus MCL
TA677 → REVIEW
- Survival/QoL unresolved
- Age addressed

NEGATIVE/ REMITTED

ONGOING / DUE FOR REVIEW



Note: From 2028 onwards, managed access exit / review and resubmission timelines are indicative and may be subject to change.
Abbreviations: B-ALL, B-cell acute lymphoblastic leukaemia; CDF, Cancer Drug Fund; DLBCL, diffuse large B-cell lymphoma; IMF, innovative medicines fund; IVIG, intravenous immunoglobulin; MAIC, matched-adjusted indirect comparison; MCL, mantle cell lymphoma; NHD, National Haemophilia Database; OS, overall survival; R/R, relapsed or refractory; SACT, systemic anticancer therapies; SCD, severe sickle cell disease; SMA, spinal muscular atrophy; TDT, transfusion-dependent beta-thalassemia.

Conclusions

- ATMP appraisal population restrictions matched reimbursement to the evidence, reducing decision uncertainty without necessarily strengthening evidence for the full licensed population.
- NICE is enabling access to ATMPs despite uncertainty, but only through systematic use of population restriction and MAA, effectively reshaping reimbursement into a conditional, evidence-generating process.
- This reflects a broader shift toward an adaptive HTA model, where earlier access is balanced against risk by targeting high-value patients and deferring evidentiary certainty over time.

Access decisions, built for a changing Europe. Kintiga partners with global pharma and biotech teams to make the European access decisions that matter most – earlier, smarter and with the senior expert insights to defend them.

Abbreviations: ATMP, advanced therapy medicinal product; CDF, Cancer Drug Fund; HST, highly specialised technology; HTA, health technology assessment; IMF, Innovative Medicine Fund; MAA, managed access agreement; MHRA, Medicines and Healthcare products Regulatory Agency; NHS, National Health Service; NICE, National Institute for Health and Care Excellence; SACT, systemic anticancer therapies; STA, standard technology appraisal.

References: MHRA Products (https://products.mhra.gov.uk/); NICE guidance (https://www.nice.org.uk/guidance/published)

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