The Royal College of Pathologists
Pathology: the science behind the cure

Enhancing outcomes for patients and donors: cell, apheresis and gene therapies

James Griffin reviews current progress in cellular and gene therapies and the need for equitable access.

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Over the past decade, cell, apheresis and gene therapies have progressed from experimental concepts to established components of routine clinical care. What once appeared to be an 'overnight success' has been built on decades of scientific discovery, clinical translation and system-level innovation. For patients with otherwise refractory disease – and for the donors whose generosity underpins some of these therapies – the impact has been transformative.

From basic science to living medicines

The conceptual foundations of advanced therapies lie in our expanding understanding of genetics, immunology and cell biology. DNA is no longer viewed solely as a static blueprint, but as a modifiable, dynamic system that can be edited, regulated or replaced. Parallel advances in cellular immunology have revealed how tightly regulated interactions between immune cells and malignant or infected targets determine surveillance, dormancy and disease progression.

Cancer immunology provides a striking illustration. Malignancy develops not simply because tumours grow, but because they evade immune surveillance. Cellular therapies, particularly immune effector cell therapies based on T lymphocytes, seek to reassert the immune response against malignant cells. Chimeric antigen receptor T-cell (CAR-T) therapy exemplifies this approach: autologous (and, in trials, allogeneic) T cells are collected via apheresis, genetically modified ex vivo and reinfused to create a living, expanding therapy capable of recognising and destroying malignant cells.

The result is a paradigm shift – from drugs that act *on* the body to therapies that act *as* the body.

Clinical impact: from last resort to earlier intervention

CAR-T therapy has achieved remarkable clinical results in relapsed and refractory haematological malignancies, including acute lymphoblastic leukaemia, diffuse large B-cell lymphoma and multiple myeloma. Durable remissions, previously unachievable with conventional therapies, are now a reality for a meaningful proportion of patients.

Importantly, UK outcomes have been broadly consistent with international experience, demonstrating that high-quality delivery is achievable within the NHS. However, 'success' should not be judged purely on efficacy metrics. Access, timeliness and equity are equally critical outcome measures.

The direction of travel is clear: earlier intervention, broader indications and combination strategies. Earlier use may reduce cumulative toxicity and improve long-term outcomes, but it also increases demand for specialist services. As advanced therapies move upstream in treatment pathways, pressure on apheresis units, manufacturing capacity and multidisciplinary teams will intensify.

This evolution necessitates robust real-world data collection to inform commissioning, pathway design and long-term follow-up. Meeting this challenge requires coordinated national planning rather than isolated centre-level innovation.

The logistics challenge: from vein to vein

Few treatments expose the complexity of modern healthcare logistics more clearly than CAR-T therapy. The pathway from patient identification to infusion involves:

- careful patient selection and timing
- therapeutic apheresis
- cryopreservation and transport of starting material
- highly regulated manufacturing, and frequently the transportation of material across international borders
- product release, transport back to the treating centre, and clinical administration.

Each step introduces potential delay and risk. Reducing vein-to-vein time is not simply an operational ambition, it is a clinical necessity for patients with aggressive disease.

Encouragingly, advances in manufacturing efficiency, supply chain coordination and digital tracking have already shortened turnaround times. Emerging models – including point-of-care manufacturing and decentralised production – promise further gains, though these bring their own regulatory and quality challenges.

Apheresis: the essential but under-recognised enabler

Apheresis sits at the heart of many advanced therapies, yet its importance is often underappreciated. High-quality cell collection determines downstream manufacturing success, product consistency and, ultimately, patient outcomes.

From the donor or patient perspective, apheresis must be safe, tolerable and efficient. From a system perspective, it must be scalable, resilient and supported by a highly skilled workforce. As immune effector cell therapies expand beyond oncology into autoimmune disease, transplantation and regenerative medicine, demand for apheresis capacity will continue to rise. Investment in staff training, equipment and service resilience is therefore not optional; it is foundational to the success of the advanced therapies ecosystem.

Equity of access: a moral and clinical imperative

One of the most sobering findings from real-world CAR-T data has been the persistence of inequality. Despite equivalent efficacy when delivered, patients from more deprived socioeconomic backgrounds are less likely to receive CAR-T and more likely to experience poorer overall survival. The reasons are complex, encompassing referral patterns, comorbidity burden, geography and health literacy.

Addressing these disparities requires action at multiple levels:

- **pathway design** to ensure early and equitable referral
- **capacity planning** to avoid postcode-dependent access
- **data transparency** to identify where inequities persist
- **policy alignment** so commissioning keeps pace with evidence.

Equity cannot be an afterthought. If advanced therapies are to fulfil their promise, they must be accessible to all patients who stand to benefit.

Beyond autologous CAR-T: gene editing and allogeneic platforms

Gene editing technologies, particularly CRISPR-based systems, have accelerated the field even further. The ability to precisely edit genes allows not only correction of inherited defects but also the engineering of immune cells with enhanced function, persistence or safety profiles.

Applications include reducing T-cell exhaustion, eliminating endogenous receptors and enabling allogeneic 'off-the-shelf' products. These approaches promise faster access and improved scalability, but introduce additional complexity in governance, long-term safety monitoring and manufacturing control.

In parallel, in-vivo gene delivery approaches represent a potential further paradigm shift, though these remain early in clinical translation. Clinical successes in gene-edited therapies for haemoglobinopathies have demonstrated the power of this approach. In-vivo CAR-T approaches – where gene delivery occurs directly within the patient, rather than ex vivo – represent another potential paradigm shift, though safety, targeting and control remain active areas of research.

Safety, standards and trust

The complexity and novelty of advanced therapies place significant demands on regulatory and quality frameworks. Their complexity, variability and long-term risks demand rigorous oversight to maintain patient trust. FACT-JACIE standards have been central in establishing consistent, high-quality practice across cellular therapy programmes, spanning laboratories, clinical services and governance structures to encompass clinical governance, staff competence and continuous quality improvement. In the UK, activity for collection is licenced by the Human Tissue Authority and, once cells have become drugs, by the Medicines and Healthcare products Regulatory Authority. This ensures vigorous requirements are met in relation to the safety of these treatments.

Embedding robust quality systems does not inhibit innovation; it enables safe innovation at scale.

The donor perspective

Donors remain essential to the success of many advanced therapies. Whether donating stem cells or lymphocytes, their safety, experience and trust underpin service sustainability. As demand grows, donor pathways must remain efficient, transparent and well supported.

Enhancing donor outcomes includes minimising procedural risk, providing clear communication and recognising donor contribution. Donor wellbeing is not separate from patient outcomes; it is integral to them.

As demand for cellular starting material grows, maintaining donor trust will be as important as technological advancement.

Conclusion: from insight to impact

Cellular and gene therapies are redefining what is possible in modern medicine. Their success, however, depends on far more than scientific ingenuity. It requires coordinated infrastructure, rigorous standards, attention to equity and an unwavering focus on both patients and donors.

The field is likely to see continued expansion of indications, increasing use of gene-edited products and sustained pressure on apheresis and manufacturing capacity. Real-world data, workforce development and service integration will be key determinants of success.

The UK is well positioned with strong national institutions, an integrated health system and a collaborative clinical community. By aligning innovation with operational excellence and social responsibility, we can ensure that the promise of advanced therapies translates into meaningful, lasting benefit for all.

Meet the author

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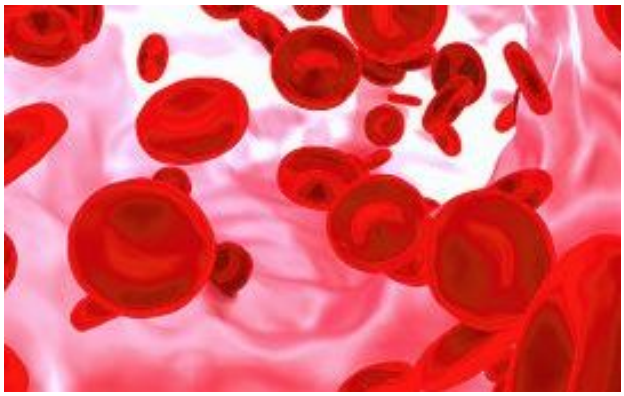

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