

Royal College of Pharmacy Response to the MHRA Consultation on the Regulation of Gene Therapy Medicinal Products

Scope of response: This response focuses on pharmacy, medicines safety, clinical governance, pharmacovigilance, workforce readiness, clinical trials, NHS implementation and patient access considerations. It is not intended to provide legal advice on the drafting of medicines legislation.

Executive Summary

The Royal College of Pharmacy (RCPharm) welcomes the MHRA consultation on updating the definition of Gene Therapy Medicinal Products (GTMPs) within the Human Medicines Regulations 2012. RCPharm recognises that advances in synthetic biology, nucleic acid technologies, genome editing, engineered cellular therapies and personalised therapeutics have outpaced the scientific assumptions underpinning the current GTMP definition [1].

RCPharm supports a proportionate modernisation of the GTMP framework. A more technology-neutral and mechanism-informed definition has the potential to improve regulatory clarity, reduce classification uncertainty and support the safe development of innovative therapies. However, definitional reform should be accompanied by clear operational guidance, robust pharmacovigilance, pharmacy workforce preparation, medicines governance safeguards and explicit consideration of equitable NHS implementation [1,2,3].

Advanced Therapy Medicinal Products (ATMPs) are increasingly moving from specialist research environments into regulated clinical delivery. The UK hosted 193 ongoing ATMP clinical trials in 2025, compared with 187 in 2024, including 42 Phase III studies, and maintained more than 56,000 m² of dedicated GMP manufacturing space for advanced therapies [4,5]. These figures highlight the need for regulatory definitions that are scientifically current and for implementation frameworks that are clinically, operationally and workforce sustainable.

Pharmacy professionals are central to the safe implementation of GTMPs across both licensed clinical use and clinical trials, including through medicines governance, investigational medicinal product management, aseptic services, procurement, cold-chain assurance, chain-of-identity and chain-of-custody systems, pharmacovigilance, medicines optimisation, patient counselling and long-term monitoring [2,3]. Pharmacists also provide clinical leadership in multidisciplinary teams and contribute to service design, implementation and the development of sustainable models of care for advanced therapies. RCPharm, therefore, recommends that the revised GTMP framework be accompanied by continued engagement with pharmacy leadership across the UK and that pharmacy expertise is represented in the development of supporting guidance and implementation frameworks. This will help ensure that medicines governance, workforce requirements and operational considerations are fully addressed as advanced therapies move into routine clinical practice.

RCPharm also supports international alignment of definitions and nomenclature where this improves predictability for developers, regulators, clinical trial sponsors, healthcare providers and patients. Where UK-specific wording is scientifically justified, it should be clearly explained in guidance to avoid unintended divergence and to support cross-border research, clinical trials, manufacturing, supply and regulatory decision-making.

Overall RCPharm Position

RCPharm supports modernising the GTMP definition to reflect contemporary science, manufacturing and clinical practice. The current definition, which is closely linked to biological origin, recombinant nucleic acids and direct therapeutic effect from the transferred sequence, no longer fully captures the range of modern advanced therapy technologies [1].

The College considers that clearer and more scientifically current GTMP definitions could benefit patients by supporting more consistent regulatory pathways, improving access to innovative treatments, enabling more personalised and precision-medicine approaches, and facilitating better long-term treatment outcomes where therapies are shown to be safe, effective and clinically appropriate. This is particularly relevant for patients with rare diseases, inherited conditions, cancer and other areas of high unmet clinical need [6,7].

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At the same time, RCPharm emphasises that improved regulatory classification alone will not guarantee improved patient outcomes. Successful implementation will depend on wider system readiness, including resilient supply chains, manufacturing capacity, cold-chain logistics, specialist aseptic services, long-term pharmacovigilance, equitable commissioning pathways and a pharmacy workforce with the knowledge, skills and capacity to support safe delivery across different care settings [4,5,8,9].

RCPharm supports scientifically justified UK flexibility, but recommends that revised definitions and supporting guidance remain aligned with international regulatory terminology wherever possible. This will help reduce avoidable uncertainty for industry, academic developers, clinical trial sponsors, regulators and healthcare providers involved in cross-border development, trials, manufacture and supply. Consistent nomenclature will also be important to avoid confusion between related concepts such as precision medicine, individualised therapies and personalised therapeutic approaches.

RCPharm therefore supports the proposed amendments, in principle, subject to clear implementation guidance, proportionality, robust medicines governance, strengthened pharmacovigilance, workforce preparation, supply-chain resilience and explicit consideration of NHS operational readiness and patient access. Implementation frameworks should be developed in partnership with healthcare professionals, including pharmacy leaders, to support safe, effective and sustainable adoption across the health system.

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Question Set 1: Non-Biological Starting Materials

Question 1

[LIKERT] Do you think that medicinal products produced from non-biological starting material should also be classified as gene therapy medicinal products (GTMPs), if they meet other proposed criteria of GTMPs as outlined in Appendix 2?

Recommended Likert answer: Agree.

RCPharm agrees that medicinal products produced from non-biological starting materials should be capable of classification as GTMPs where they meet the proposed functional and mechanistic criteria. A distinction based solely on whether a starting material is biological or synthetic is increasingly difficult to justify where the resulting product has equivalent biological activity, therapeutic intent and risk-management implications [1].

From a pharmacy and healthcare-system perspective, synthetic and biologically derived GTMPs may both require specialist governance, cold-chain assurance, aseptic handling, traceability, long-term monitoring and enhanced pharmacovigilance [2,3]. Classification should therefore be informed by the function and risk profile of the medicine, rather than manufacturing origin alone. However, MHRA guidance should clearly describe the boundaries of this approach to avoid inappropriate classification of products whose mechanisms, risks and clinical governance requirements are materially different from GTMPs.

Question Set 2: Transcription and Translation

Question 2

[LIKERT] Do you think that medicinal products containing recombinant or synthetic nucleic acids should be classified as gene therapy medicinal products, only where said nucleic acids undergo transcription or translation?

Recommended Likert answer: Agree.

RCPharm agrees that transcription or translation provides a useful criterion for distinguishing recombinant or synthetic nucleic acid products that should fall within the GTMP framework from other nucleic acid-based medicines. This distinction may help avoid unintended reclassification of products such as antisense oligonucleotides, siRNA or microRNA-based products that do not mediate their effect through transcription or translation [1].

The College recommends that MHRA guidance should acknowledge that the scientific boundary between gene regulation, RNA therapeutics, genome editing and programmable medicines may evolve. The framework should therefore be capable of future interpretation without requiring frequent legislative revision.

Question Set 3: Sequence-Specific Genome Editing and Epigenetic Mechanisms

Question 3

[LIKERT] Do you think that medicinal products should be classified as gene therapy medicinal products when their mechanism of action involves deliberate, sequence specific genome editing, regardless of the composition of the active substance?

Recommended Likert answer: Agree.

RCPharm agrees that medicinal products whose mechanism of action involves deliberate, sequence-specific genome editing should be classified as GTMPs, irrespective of the composition of the active substance.

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Future genome editing platforms may involve recombinant nucleic acids, synthetic nucleic acids, protein-based systems, transient editors, hybrid delivery systems or technologies not yet widely used clinically [1,8]. A definition tied too narrowly to a specific delivery platform would risk becoming rapidly outdated.

Question 4

[LIKERT] Do you think that medicinal products that modulate gene expression through epigenetic (or epitranscriptomic) mechanisms without altering a nucleic acid sequence should be classified as gene therapy medicinal products?

Recommended Likert answer: Disagree.

RCPharm agrees with the MHRA's distinction between deliberate sequence-specific genome editing and epigenetic or epitranscriptomic modulation that does not alter nucleic acid sequence. Medicines that affect gene expression without editing the genome should not automatically be classified as GTMPs solely on that basis [1]. This distinction is important to preserve proportionality and avoid unintended capture of conventional or non-ATMP medicines.

Question Set 4: Direct Therapeutic Effect

Question 5

[LIKERT] Do you think that classification of a product as a gene therapy medicinal product should include medicinal products for which the transferred genetic material is not directly responsible for the therapeutic, prophylactic, or diagnostic activity of the product?

Recommended Likert answer: Agree.

RCPharm agrees that GTMP classification should not depend solely on whether the transferred genetic material is directly responsible for the primary therapeutic, prophylactic or diagnostic effect. Genetic modification may provide a safety switch, persistence mechanism, manufacturing function, selection marker or control mechanism while still being highly relevant to product governance, safety and monitoring [1].

From a pharmacy governance perspective, such modifications remain relevant because they may affect handling requirements, risk assessment, traceability, pharmacovigilance and patient counselling [2]. The College therefore supports removing the requirement that the therapeutic effect must derive directly from the transferred genetic material, provided the final definition remains sufficiently precise.

Question Set 5: Human Cells, Donor Cells and Intermediate Biological Systems

Question 6

[LIKERT] Do you think that medicinal products which exert their action through targeted genomic edits should be classified as gene therapy medicinal products regardless of whether these edits occur directly within human cells or in other cells administered to the patient?

Recommended Likert answer: Agree.

RCPharm agrees that medicinal products which exert their action through targeted genomic edits should be classified as GTMPs regardless of whether those edits occur directly within human cells or in other cells administered to the patient. The key regulatory consideration should be the functional activity and intended biological effect of the product, not only the cellular location in which the modification occurs [1].

Question 7

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[LIKERT] Do you think that medicinal products which exert their action through the expression of a recombinant or synthetic nucleic acid should be classified as gene therapy medicinal products regardless of if this activity occurs at the level of a human cell or in other cells administered to the patient?

Recommended Likert answer: Agree.

RCPharm agrees that medicinal products which exert their action through expression of recombinant or synthetic nucleic acid should be classified as GTMPs regardless of whether this activity occurs at the level of a human cell or in other cells administered to the patient. Products may exert relevant biological effects through donor cells, engineered cells, microbial systems or other intermediate biological platforms administered to patients [1]. Implementation guidance should provide clear examples to support consistent interpretation by developers, regulators and healthcare providers [2,3].

Question Set 6: International Alignment

Question 8

[LIKERT] Do you think the UK should consider classifying only those medicinal products which exert their effect through “long lasting” transcription or translation of a recombinant or synthetic nucleic acid as gene therapy medicinal products?

Recommended Likert answer: Disagree.

RCPharm recognises the importance of international alignment, particularly with the EU and other major regulatory partners [10]. However, the College considers that a statutory requirement for “long-lasting” transcription or translation may introduce avoidable uncertainty. Duration of expression may vary across products and may not always correlate directly with risk, complexity or healthcare-system burden [1].

RCPharm therefore recommends that duration of transcription or translation should be considered within product-specific regulatory benefit-risk assessment, including quality, non-clinical, clinical, efficacy and safety considerations, rather than being used as a rigid statutory threshold. Supporting guidance should clarify how duration of expression should be interpreted for different product types, without creating unnecessary uncertainty for products already appropriately regulated as ATMPs. Local provider-level assessment should remain proportionate and should recognise that some operational risk assessments, particularly those relating to GMO status, are driven by product-specific characteristics rather than GTMP classification alone.

Question 9

[FREE-TEXT] [OPTIONAL] In the context of the proposed amendments, to what extent should the Human Medicines Regulations 2012 mirror the proposed EU definition, or be tailored to better reflect the UK innovation landscape?

Free-text response:

RCPharm supports alignment with international regulatory approaches where this improves predictability, development efficiency, clinical trial delivery, pharmacovigilance and supply-chain practicality [10]. Given the cross-border nature of advanced therapy development, manufacturing, clinical research and regulatory decision-making, definitions and nomenclature should be harmonised with EU and other major regulatory partners wherever scientifically appropriate.

At the same time, RCPharm recognises that the UK framework may need to use more precise wording where this improves scientific clarity or avoids unintended exclusion of clinically relevant technologies. UK-specific flexibility should therefore be preserved where scientifically justified, but avoidable divergence should be

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minimised to support industry and academic developers, regulators, clinical trial sponsors, healthcare providers and patients working across jurisdictions.

RCPharm recommends that any differences between the UK definition and the terminology used by the EU, USA or other major regulatory partners should be clearly explained in MHRA guidance. This would help reduce the risk of unintended classification consequences, support future mutual recognition or reliance approaches where appropriate, and enable advanced therapies to be developed, trialled, manufactured and supplied with greater consistency across borders.

Question Set 7: Overarching Questions

Question 10

[LIKERT] Do you agree or disagree with the proposed changes as outlined in Appendix 2, to the definitions of gene therapy medicinal products?

Recommended Likert answer: Agree.

RCPharm agrees with the proposed amendments to the GTMP definitions. The reforms are scientifically justified and may improve regulatory clarity for synthetic nucleic acid products, genome-editing systems and genetically modified cellular therapies [1].

RCPharm's support is subject to clear guidance that minimises avoidable divergence from international terminology and reduces the risk of unintended classification consequences for developers, regulators, clinical trial sponsors and healthcare providers.

Question 11

[FREE-TEXT] [OPTIONAL] What implications, benefits, or risks do you foresee the proposed changes to the definitions of gene therapy medicinal products?

Free-text response:

RCPharm considers that the proposed changes may improve regulatory clarity, better align legislation with modern science, support innovation and enable more consistent risk-based oversight of comparable products. However, these benefits will depend on detailed implementation guidance, operational readiness and continued attention to pharmacy governance, aseptic services, pharmacovigilance and workforce capability [2,3]. The College also notes that products close to the boundary between GTMP and non-GTMP classification may require specific guidance to support consistent interpretation.

A further risk is that definitional changes, if not carefully aligned with international terminology and supported by practical examples, could create uncertainty for cross-border development, clinical trials, marketing authorisation planning and NHS provider readiness. RCPharm therefore recommends that MHRA guidance includes practical examples, boundary cases and clear communication for developers, clinical trial sponsors, NHS providers and pharmacy teams.

Question 12

[FREE-TEXT] [OPTIONAL] Are there specific categories of products which, in your view, should be explicitly excluded from the revised definitions of a gene therapy medicinal product, and what scientific or regulatory rationale supports their exclusion?

Free-text response:

RCPharm recommends clear guidance for products that modulate gene expression without altering nucleic acid sequence, including antisense oligonucleotides, siRNA, microRNA-based products and epigenetic

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modulators, unless they otherwise meet GTMP criteria [1]. The College also supports maintaining the exclusion for vaccines against infectious diseases.

Question 13

[FREE-TEXT] [OPTIONAL] Please provide examples of products that were previously not classified as gene therapy medicinal products (GTMP) but would now fall under the revised criteria (as proposed in Appendix 2) of a GTMP?

Free-text response:

Potential examples of product types that may fall within the revised criteria include synthetic mRNA therapeutics for non-infectious disease indications, synthetic DNA constructs intended for transcription or translation, non-recombinant sequence-specific genome editors, and genetically modified cellular products where the modification provides a safety, persistence or control function rather than the primary therapeutic effect [1]. RCPharm recommends that MHRA guidance presents these as illustrative examples and clarifies boundary cases, recognising that final classification will depend on product-specific characteristics and regulatory assessment.

Question Set 8: Impact of Changes

Question 14

[LIKERT] [OPTIONAL] With reference to the protected characteristics covered by the Public Sector Equality Duty set out in section 149 of the Equality Act 2010, or by section 75 of the Northern Ireland Act 1998, we have assessed that the proposals set out in Appendix 2 do not risk impacting people differently with reference to their protected characteristics, or in regard to the Rural Needs Act (Northern Ireland) 2016. Do you agree or disagree with this statement?

Recommended Likert answer: Agree.

RCPharm agrees that the proposed definitional changes are unlikely in themselves to create direct differential impacts by protected characteristics. However, the implementation of GTMPs may have equity implications because some target conditions disproportionately affect specific populations, including sickle cell disease and beta-thalassaemia [6,7]. Access should therefore be monitored by geography, ethnicity, socioeconomic status and specialist centre availability where appropriate.

Question 15

[FREE-TEXT] [OPTIONAL] We assume the main organisations impacted will be developers. We would welcome your view on if you agree or disagree with this, and how many developers you would expect to be affected by these changes. Please share any evidence to support your view.

Free-text response:

RCPharm agrees that developers, including industry and academic developers, are likely to be directly affected by the proposed changes. However, the College recommends that MHRA also recognises downstream implications for NHS providers, pharmacy services, aseptic units, clinical trial teams, specialist commissioners, pharmacovigilance teams and patient-facing services [2,3].

Clear communication will be needed to ensure NHS providers and pharmacy teams are aware of the revised definitions and understand any implications for local governance, clinical trial delivery, documentation, service readiness and patient-facing information. This is particularly important where products are already in development, in clinical trials, or approaching marketing authorisation, and where cross-border development or supply may be affected by differences in regulatory terminology.

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Question 16

[FREE-TEXT] [OPTIONAL] We assume that there will be no additional costs to business arising from these proposals. Do you agree or disagree? If you consider that there will be costs, please describe these and share any evidence to support your view.

Free-text response:

RCPharm agrees that the legal definitional change itself may not necessarily impose substantial direct costs in all cases. However, transitional costs may arise where the revised definition affects products already in development, in clinical trials, or under preparation for marketing authorisation. These may include costs associated with reviewing classification status, updating clinical trial protocols, revising marketing authorisation documentation, communicating changes to trial sites and NHS providers, and updating local governance assessments or documentation [2,3].

RCPharm notes that some operational requirements, such as storage, handling, logistics and local risk assessment, may be driven by product-specific factors, including whether a product contains a genetically modified organism, rather than GTMP classification alone. Staff awareness and practical guidance will therefore be important to prevent unnecessary, inconsistent or overly cautious local interpretation of the revised definition.

Question 17

[FREE-TEXT] [OPTIONAL] We expect that there will be a direct benefit for developers, and that the ultimate benefit of these changes will be improved health outcomes for patients. Do you agree or disagree, and please share any evidence to support your view, including on what these improved health outcomes might be.

Free-text response:

RCPharm agrees that the reforms may benefit developers and may ultimately support improved patient access to innovative therapies. However, improved health outcomes will depend on timely commissioning, clinical pathway readiness, specialist workforce capacity, equitable access and robust long-term evidence generation [3,4,5,6,7].

These benefits will be strengthened if the revised framework is communicated clearly to NHS providers and remains sufficiently aligned with international regulatory terminology to support efficient development, clinical trials, manufacture, supply and patient access.

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Pharmacy-Specific Implementation Considerations

RCPharm emphasises that pharmacy professionals, including pharmacists and pharmacy technicians, will be essential to the safe implementation of GTMPs across clinical care, clinical trials, medicines governance, technical services, pharmacovigilance and patient-facing pathways. Pharmacy professionals also provide leadership within multidisciplinary teams and contribute to service design, implementation planning and the development of sustainable models of care for advanced therapies. GTMPs are medicines, and pharmacy teams provide critical governance, technical, clinical and operational expertise across the product lifecycle [2,3].

In specialist and tertiary-care settings, pharmacy teams may contribute to medicines governance, aseptic preparation, product receipt, cold-chain assurance, chain-of-identity and chain-of-custody processes, clinical verification, multidisciplinary treatment planning, adverse-event monitoring and long-term follow-up. In clinical trials and research settings, pharmacy teams currently provide essential support for investigational medicinal product management, protocol compliance, accountability, documentation, temperature monitoring, pharmacovigilance and communication between sponsors, investigators and clinical teams [4,5]. RCPharm recommends that pharmacists be more fully integrated into trial design, protocol development, risk assessment and strategic decision-making. Their expertise in medicines governance, patient safety, supply chain logistics, and long-term therapy monitoring enables them to contribute not only operationally but also strategically to trial planning, regulatory compliance, and optimisation of patient pathways. Expanding pharmacist involvement beyond dispensing and routine trial support could enhance trial efficiency, safety, and alignment with real-world clinical care, ensuring that advanced therapies are implemented effectively and safely across care settings.

In commissioning, medicines optimisation and integrated care settings, pharmacists, pharmacy technicians and wider pharmacy teams may support pathway design, formulary governance, treatment eligibility processes, outcomes monitoring and real-world evidence generation. In community, primary care and other patient-facing settings, pharmacy professionals may support patient education, medicines reconciliation, monitoring for concomitant medicine risks, adherence to follow-up requirements, identification of adverse effects and signposting back to specialist teams. In industry, academic development, manufacturing, regulatory and supply-chain settings, pharmacy expertise may also support quality assurance, qualified-person oversight, pharmacovigilance systems, product information and safe implementation planning.

RCPharm recognises that these implementation issues extend beyond the legal definition of GTMPs and are not solely the responsibility of MHRA. Workforce readiness and system preparedness will require coordinated engagement with relevant organisations, including the

RCPharm recommends that existing institutional readiness guidance and GTMP guidance developed by the Pan UK ATMP Pharmacy Working Group and published through the NHS Specialist Pharmacy Service should be acknowledged and signposted where relevant [2]. These resources are important for supporting consistent local implementation, governance and pharmacy preparedness.

RCPharm also notes that horizon scanning for future GTMP service demand is challenging because of the high attrition rate of ATMPs during development. In the short to medium term, many GTMPs are likely to remain focused on rare diseases, cancer and other specialist indications, with delivery concentrated in existing specialist or tertiary centres. Although ATMPs for broader indications are in clinical trials, it remains difficult to predict which products will progress successfully through regulatory approval, commissioning and implementation pathways.

The UK advanced therapies sector is nevertheless growing rapidly. CGT Catapult reported 193 ongoing UK ATMP clinical trials in 2025, while wider workforce foresighting evidence indicates that the UK cell and gene therapy workforce grew from approximately 3,033 people in 2019 to 6,232 people in 2023, with an additional 3,929 people expected by 2028 [4,8]. This growth reinforces the need for proportionate workforce planning,

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clinical trials pharmacy capacity, specialist pharmacy services, genomic medicine literacy, pharmacovigilance capability and pharmacy technician development alongside the revised GTMP framework.

Future Review: AI, Synthetic Biology and Adaptive Regulation

RCPharm recommends that MHRA consider a defined future review point for the revised GTMP framework, given the rapid convergence of artificial intelligence, synthetic biology, automated design platforms and programmable therapeutics. AI-enabled molecular design, high-throughput genome-editing optimisation, synthetic construct generation and increasingly automated manufacturing may accelerate the development of therapies that challenge existing regulatory taxonomies [8].

RCPharm does not suggest that AI-enabled development should itself determine GTMP classification. Rather, the College recommends that future regulatory review should assess whether emerging AI-designed or highly automated synthetic biology products continue to be appropriately captured by the revised definitions, guidance and risk-based assessment pathways.

Any future review should also consider whether UK definitions remain interoperable with EU, US and other major regulatory approaches, particularly where AI-enabled design, automated manufacturing or synthetic biology platforms are used in cross-border development, clinical trials and supply chains.

RCPharm Recommendations

RCPharm recommends that MHRA proceed with modernising the GTMP definition while maintaining proportionality, scientific clarity and international alignment where appropriate. The College recommends that MHRA publish detailed implementation guidance with practical examples of included and excluded product types, preserve clear distinctions between genome editing, gene-expression modulation and non-ATMP nucleic acid therapeutics, and support clear communication to developers, regulators, clinical trial sponsors, NHS providers and pharmacy teams. Guidance should also minimise avoidable divergence in definitions and nomenclature from EU, US and other major regulatory approaches, while preserving scientifically justified UK flexibility.

RCPharm further recommends coordinated national workforce development for pharmacists, pharmacy technicians and wider pharmacy teams involved in ATMP and GTMP services. This should include clinical trials pharmacy, aseptic services, advanced therapy governance, pharmacovigilance, genomic medicine literacy, patient-facing medicines optimisation, and industry, academic, regulatory and supply-chain roles. RCPharm recognises that this will require engagement beyond MHRA, including with the General Pharmaceutical Council, NHS England workforce and education functions, the Genomic Medicine Service, devolved administrations, the Pan UK ATMP Pharmacy Working Group and the NHS Specialist Pharmacy Service. Existing institutional readiness and GTMP guidance from specialist pharmacy groups should be acknowledged and signposted where relevant.

Post-authorisation surveillance and real-world evidence systems should be strengthened, particularly for long-term safety, durability and equitable access. MHRA should continue international regulatory dialogue to minimise avoidable divergence while preserving scientifically justified UK flexibility. The College also recommends a future review point to assess whether the revised GTMP framework remains suitable as AI-enabled design, synthetic biology and programmable therapeutic platforms mature.

Conclusion

RCPharm supports the MHRA's proposed modernisation of the GTMP framework. The proposed amendments are scientifically justified and may help ensure that UK medicines regulation remains aligned with advances in synthetic biology, genome editing and advanced therapeutic platforms [1].

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However, successful reform will depend on more than legal definitional change. It will require clear guidance, robust governance, specialist pharmacy capability, long-term pharmacovigilance, real-world evidence generation, workforce development and equitable implementation [2,3,9].

RCPharm would welcome continued engagement with MHRA, NHS England, NICE, devolved administrations, the General Pharmaceutical Council, NHS England workforce and education functions, the Genomic Medicine Service, the Pan UK ATMP Pharmacy Working Group, the NHS Specialist Pharmacy Service, specialist clinical networks, academia, industry and patient organisations to support safe, proportionate and patient-centred implementation of the revised GTMP framework. RCPharm also encourages MHRA to maintain close international dialogue so that revised UK definitions support innovation, clinical trials, manufacturing and supply across borders, while avoiding unintended divergence in terminology or classification.

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References

1. Medicines and Healthcare products Regulatory Agency (MHRA). Consultation on the Regulation of Gene Therapy Medicinal Products. Published 11 May 2026. Available from: <https://www.gov.uk/government/consultations/consultation-on-the-regulation-of-gene-therapy-medicinal-products/consultation-on-the-regulation-of-gene-therapy-medicinal-products>
2. NHS Specialist Pharmacy Service. Advanced Therapy Medicinal Products (ATMPs). Available from: <https://www.sps.nhs.uk/home/guidance/advanced-therapy-medicinal-products/>
3. NHS England. Advanced Therapy Medicinal Products Programme. Available from: <https://www.england.nhs.uk/commissioning/spec-services/advanced-therapy-medicinal-products/>
4. Cell and Gene Therapy Catapult. UK 2025 Advanced Therapy Medicinal Product Clinical Trials Database. Available from: <https://ct.catapult.org.uk/resources/clinical-trials-database>
5. Cell and Gene Therapy Catapult. New Data Shows Continued Growth in Advanced Therapy Clinical Trials in the UK for 2025. Published 26 January 2026. Available from: <https://ct.catapult.org.uk/news/new-data-shows-continued-growth-in-advanced-therapy-clinical-trials-in-the-uk-for-2025-with-the-number-of-early-stage-trials-remaining-high>
6. National Institute for Health and Care Excellence (NICE). Exagamglogene Autotemcel for Treating Transfusion-Dependent Beta-Thalassaemia in People 12 Years and Over. Technology Appraisal Guidance TA1003. Published 11 September 2024. Available from: <https://www.nice.org.uk/guidance/ta1003>
7. National Institute for Health and Care Excellence (NICE). Exagamglogene Autotemcel for Treating Severe Sickle Cell Disease in People 12 Years and Over. Technology Appraisal Guidance TA1044. Published 26 February 2025. Available from: <https://www.nice.org.uk/guidance/ta1044>
8. Cell and Gene Therapy Catapult. Digitalisation and Automation for Cell and Gene Therapy: Workforce Foresighting Report. 2025. Available from: <https://advancedtherapiesapprenticeships.co.uk/wp-content/uploads/2025/06/Cell-and-Gene-Therapy-Catapult-Workforce-Foresighting-Report-FINAL-1-1.pdf>
9. NHS England. Pharmacy Genomics Workforce, Education and Training Strategic Framework. Available from: <https://www.england.nhs.uk/long-read/pharmacy-genomics-workforce-education-and-training-strategic-framework/>
10. European Medicines Agency (EMA). Advanced Therapy Medicinal Products: Overview. Available from: <https://www.ema.europa.eu/en/human-regulatory-overview/advanced-therapy-medicinal-products-overview>
11. Association of the British Pharmaceutical Industry (ABPI). Advanced Therapy Medicinal Products (ATMPs). Available from: <https://www.abpi.org.uk/value-and-access/advanced-therapy-medicinal-products-atmps/>