



FPM Response to MHRA consultation on the regulation of Gene therapy medicinal products

In May 2026, the Medicines and Healthcare products Regulatory Agency opened a consultation on the regulation of Gene therapy medicinal products.

Advanced Therapy Medicinal Products (ATMPs) are innovative human medicines designed to treat serious conditions such as cancer and genetic disorders, offering potential improvements in outcomes and quality of life, especially where unmet needs exist. They are classified as gene therapy, somatic cell therapy, or tissue-engineered products, with gene therapy regulations taking precedence when overlapping. ATMPs are complex, involving variable biological materials, intricate structures, and novel manufacturing processes, making standardisation and scale-up difficult.

Regulators apply a risk-based approach to determine data requirements for approval, allowing flexibility provided that robust scientific justification demonstrates safety, quality, efficacy, and a favourable benefit-risk balance.

Any such deviation from standard data requirements is contingent on relevant scientific justifications being provided, typically based on product characteristics, demonstration of a positive benefit-risk balance, and consideration of potential implications of such deviations on product quality, safety, and efficacy.

FPM submitted its response, below, on 17 June 2026.

Questions

1. 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?

- **Strongly agree**
- Agree
- Neither agree or disagree
- Disagree
- Strongly disagree



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2. 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?

- Strongly agree
- Agree
- Neither agree or disagree
- Disagree
- Strongly disagree

3. 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?

- Strongly agree
- Agree
- Neither agree or disagree
- Disagree
- Strongly disagree

4. 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?

- Strongly agree
- Agree
- Neither agree or disagree
- Disagree
- Strongly disagree

5. 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?

- Strongly agree



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- Agree
- **Neither agree or disagree**
- Disagree
- Strongly disagree

6. 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?

- **Strongly agree**
- Agree
- Neither agree or disagree
- Disagree
- Strongly disagree

7. 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?

- **Strongly agree**
- Agree
- Neither agree or disagree
- Disagree
- Strongly disagree

8. 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?

* In addition to medicinal products which have targeted genomic edits

- Strongly agree
- Agree
- **Neither agree or disagree**
- Disagree
- Strongly disagree



9. 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? (optional)

While harmonisation allows for supply chain resilience and regulatory harmonisation, the flexibility of not having harmonisation allows for better patient needs and reduces administration.

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

- Strongly agree
- Agree
- Neither agree or disagree
- Disagree
- Strongly disagree

11. What implications, benefits or risks do you foresee the proposed changes to the definitions of gene therapy medicinal products? (optional)

The definitions require further explanation and clarity. There is also the potential that they will create increased regulatory burden and potentially reduce innovation.

The proposed changes raises risks in terms of the regulatory burden and potential legal challenges and innovation constraints. In addition, there will need to be a legacy period for adoption.

12. 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? (optional)

Vaccines - cells that are not viable and not manipulated cells.



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13. 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? (optional)

Vaccines and nonviable cells.

14. 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? (optional)

- **Strongly agree**

- Agree
- Neither agree or disagree
- Disagree
- Strongly disagree

15. 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. (optional)

Disagree with this premise as many orphan developers are academics and charities. So the effects will be wide ranging.

16. 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. (optional)

Disagree with this premise as the developer compliance will come at a cost and the CMC could subsequently increase.

17. 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



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agree or disagree, and please share any evidence to support your view, including on what these improved health outcomes might be. (optional)

A risk from this is that there may be a delay in access and reduced innovation.