Horizon scanning of platform technologies for the development of ATMPs

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Introduction

- ATMPs are now a clinical reality, but there are still **systemic barriers** challenging further development and implementation: high development and **manufacturing costs**, **small patient populations** and **limited batch sizes**, especially in **rare diseases**.
- **Platform technologies** have emerged as a candidate approach to address these issues and are already used for monoclonal antibodies and vaccines. They support a **standardised, knowledge-based process** that may reduce development time and cost.
- No global consensus exists on the criteria that define a platform technology for medicinal products, and **regulatory requirements differ across regions**. The situation is even more complex for ATMPs, as their **inherent diversity** and **product-specific attributes** further complicate the development and application of appropriate regulatory frameworks .
- In the present work, a **horizon scanning** methodology was adapted to support the regulatory assessment of emerging **platform technologies applied to ATMP development**.

Objectives

1. Map the state of **platform technology definitions** across major global regulatory agencies.
2. Characterise the platform approaches under development in **scientific**, **patent**, **clinical trial** and **grey literature** sources.
3. Identify **gaps** and **opportunities** in applying platform technologies to ATMP development.

Aligned with EMA Regulatory Science Research Need **RSN-2025-NT3.2**.

Methods

Regulatory definitions
EMA, FDA, NMPA, PMDA, MHRA, Health Canada, TGA, CDSCO, ANVISA, ICH, WHO, ICMRA, IABS/CEPI

→ **Structured search**
PubMed, Espacenet and ClinicalTrials.gov, 2016 to 18 July 2026; PubMed trend searches and keyword network (VOSviewer); grey literature (qualitative)

→ **787 records retrieved**
Screened against the regulatory definitions

→ **200 records included**
63 scientific articles, 115 patents and 22 clinical trials

→ **Classification**
ATMP type, development domain (CMC, nonclinical, clinical), product type and region of origin

Regulatory definitions of platform technology: summary

Framework	Core criteria
EU Reform of the EU general pharmaceutical legislation (Article 5(1) of Draft Directive)	*"(34) 'platform technology' means a technology or collection of technologies that has the potential to be incorporated in, or used by, more than one medicinal product, that is comprehensive, well-characterised, reproducible and standardised, that is used for the development, the manufacturing process or quality control of medicinal products, that relies on prior knowledge and that is established in accordance with scientific principles for which there is reasonable scientific certainty that they will remain unchanged across medicinal products"*
US FD&C Act 506K	*"a well-understood and reproducible technology, which can include a nucleic acid sequence, molecular structure, mechanism of action, delivery method, vector, or a combination of any such technologies that the Secretary determines to be appropriate, that the sponsor demonstrates (1) is incorporated in or used by a drug and is essential to the structure or function of such drug; (2) can be adapted for, incorporated into, or used by, more than one drug sharing common structural elements; and (3) facilitates the manufacture or development of more than one drug through a standardized production or manufacturing process or processes"*
China NMPA guideline 2024	*"one or a series of technologies used by the manufacturer to develop and/or produce similar products, and the previous products using this technology have been marketed or have been under clinical study. If, in the subsequent product development, such elements as product technology skeleton (may including nucleic acid carrier, virus carrier, protein skeleton, etc.), manufacturing processes, critical quality attributes and GMP implementation are basically unchanged, it can be regarded as a platform"*

Results

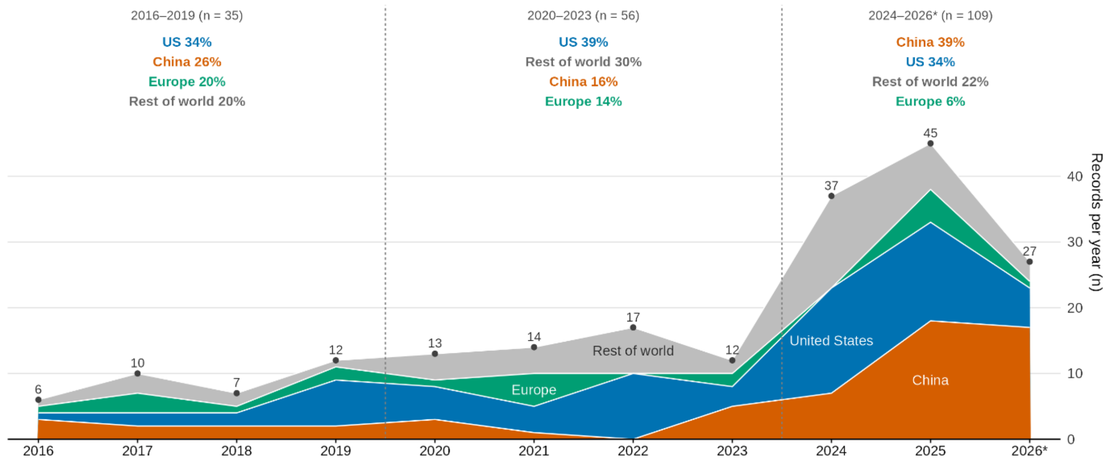

Figure 1. Platform technology records per year, by region of origin
54.5% of records date from 2024–2026 (peak 2025, n = 45). China leads in 2024–2026 (38.5%); Europe falls from 20.0% to 5.5%. *2026 to 18 July.

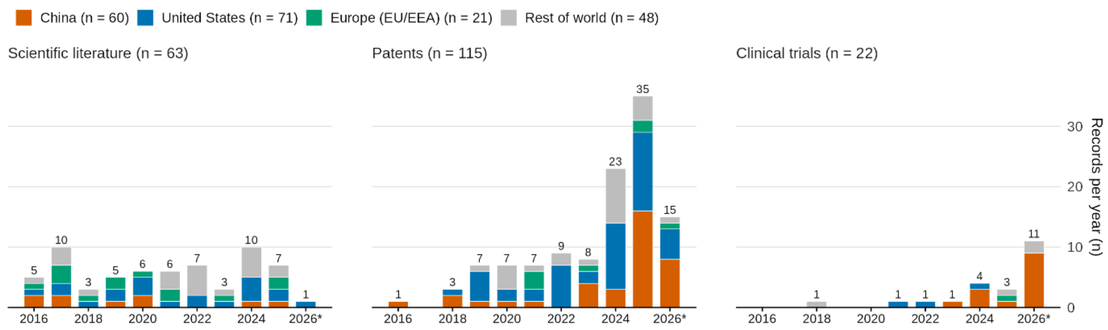

Figure 2. Records per year, by source
Growth is driven by patents and clinical trials; article output stays flat. China holds 63.6% of trial records (14/22).

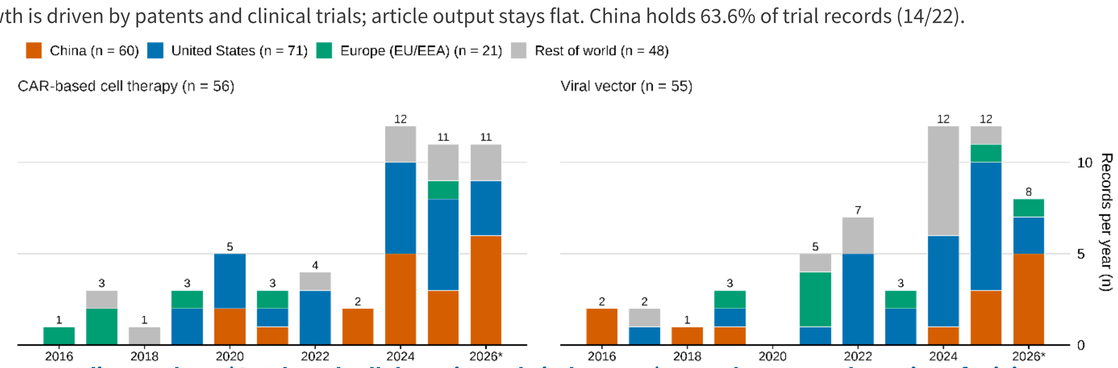

Figure 3. Leading products (CAR-based cell therapies and Viral vectors): records per year, by region of origin
CAR-based cell therapy: China's share rises from 22.7% (2016–23) to 41.2% (2024–26); Europe's falls from 22.7% to 2.9%.
Viral vectors: the US leads in both periods (43.5% in 2016–23; 43.8% in 2024–26), while China rises from 17.4% (4/23) to 28.1% (9/32) and Europe falls from 21.7% to 6.3%.

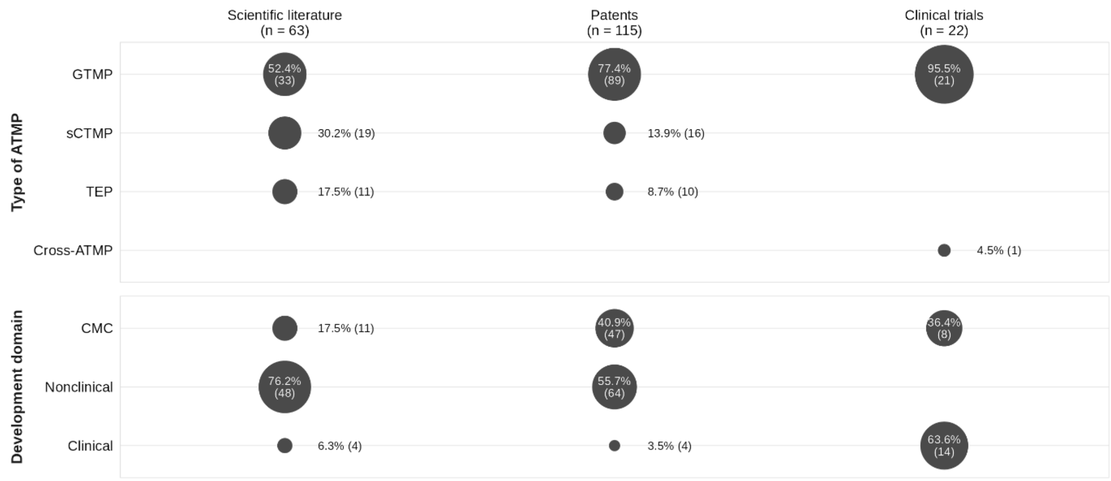

Figure 4. Type of ATMP and development domain, by source
GTMPs rise from 52.4% of articles to 77.4% of patents and 95.5% of trials; CMC is 40.9% of patents vs 17.5% of articles; trials are mainly clinical (63.6%).

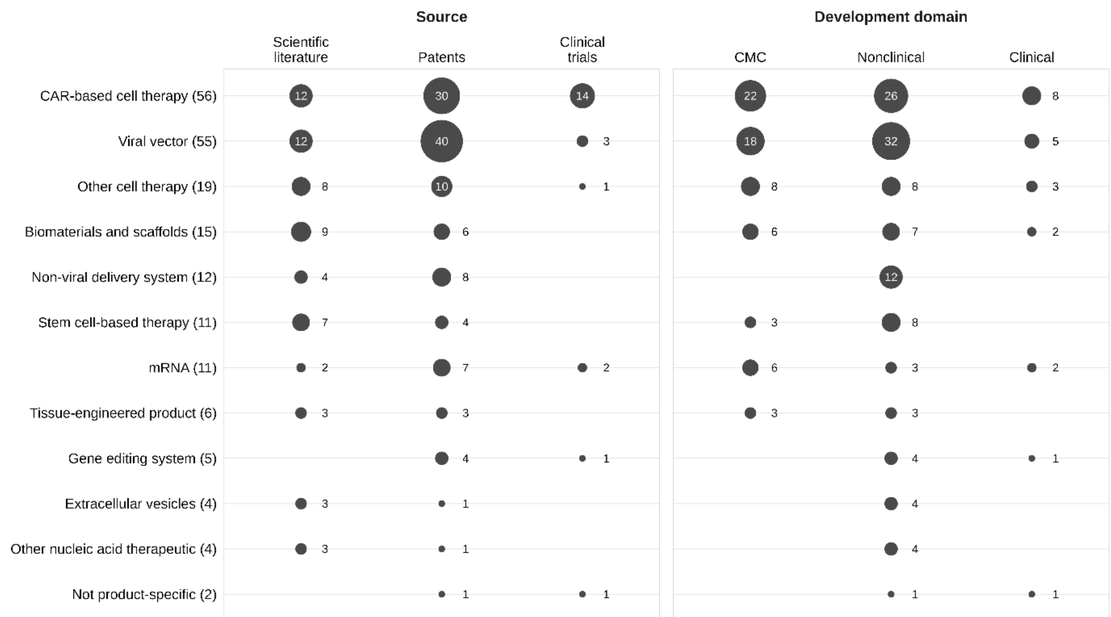

Figure 5. Product type, by source and development domain
CAR-based cell therapy (56) and viral vectors (55) make up 55.5% of records; 72.7% of viral vector records are patents and 63.6% of trials are CAR-based.

Key findings. The US and China account for 65.5% of records: the US leads patents and China leads clinical trials, with an increasing share of China since 2024 (38.5% of records in 2024–2026).
Most platforms are directed to GTMPs (71.5%) and support nonclinical development (56.0%).

Drafted PTMF for a viral vector platform: CTD Module 3.2.S

CTD section	Potential reduction / omission in subsequent applications
3.2.S.1 General information	Cross-reference to data on the vector backbone, serotype, promoters, enhancer elements if remain fixed components. New information limited to the new gene of interest.
3.2.S.2 Manufacture	Cross-reference to the platform file for unchanged steps; control of starting materials not specific to the new insert, unchanged in-process controls and process validation.
3.2.S.3 Characterisation	Cross-reference to data on the vector backbone, serotype, promoters, enhancer elements characterisation studies if remain fixed components Prior knowledge could be leveraged also for known product-related and process-related impurities characterisation.
3.2.S.4 Control of drug substance	Cross-reference to the platform file for unchanged test methods and validations. Prior knowledge on batch data could be used for specifications establishment and justification.
3.2.S.5 Reference standards	Cross-reference to the platform file for common reference standards or reference materials used for testing.
3.2.S.6 Container closure system	No reduction/omission applicable.
3.2.S.7 Stability	Prior knowledge on stability data could be used to support shelf-life establishment and extrapolation.
3.2.A Appendices	Cross-reference to the platform file for consolidated information on facilities and equipment. New information limited to product-specific adventitious agents safety evaluation or novel excipients used if applicable.

Reduction proposed in 7 of 8 sections. Drafted from the EMA vaccine PTMF framework. Fixed components (vector backbone, serotype, promoters, enhancer elements) are cross-referenced; new information is essentially limited to the therapeutic transgene and product-specific items.

Conclusions

- Platform technologies for ATMPs are not only an emerging category in publications and patents, but already a **tangible regulatory reality reflected in clinical trials** and recent guidelines.
- **Viral vector** and **CAR-based cell therapy** platforms are the most mature;
- Patenting and clinical testing have **shifted towards China since 2024**, while Europe's share has declined.
- Persistent regulatory differences across regions support the **need for harmonised definitions**, qualification criteria and **dossier-reduction tools**.
- Consistent regulatory frameworks are crucial to optimise scientific and commercial impact across all ATMP modalities and, ultimately, to **facilitate patient access**.

Limitations. 200 records only (22 trials) and descriptive analysis; 2026 is a partial year (cut-off 18 July); grey literature is qualitative and bibliometrics reflect reporting trends.

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