JOIN4ATMP

2025

Looking back on Year 2

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2025

Looking back on Year 2

Advanced Therapy Medicinal Products (ATMPs) are innovative gene-, cell-, and tissue-based medicines that are transforming the treatment landscape for rare diseases, oncology and other complex conditions with limited or no effective therapeutic options. While regulatory initiatives at European level continue to evolve, ATMP development and patients access still face significant and persistent clinical, regulatory and economic barriers.

JOIN4ATMP was launched in January 2024 to address these challenges through a coordinated, European-level action. The project brings together a highly engaged consortium of 14 partners and a broad network of stakeholders with complementary expertise spanning ATMP clinical development, manufacturing, regulatory science, health economics, policy and patient representation.

By connecting real-world experience with structured analysis and actionable solutions, JOIN4ATMP aims to ensure that innovation in ATMPs translates into tangible and sustainable benefits for patients and healthcare systems across Europe.

In year 2, JOIN4ATMP consolidated its work around its four interlinked objectives:

1. Mapping and categorising key hurdles in ATMP development
2. Charting solutions and best practices from real-world experiences
3. Designing actionable recommendations
4. Laying the foundations for a sustainable European framework beyond the project's lifetime

With this second results-oriented overview, the JOIN4ATMP consortium is pleased to present the main achievements obtained so far, while at the same time embarking on the final and most impactful phase of the project. As JOIN4ATMP entered its last year, the focus remains on ensuring that the knowledge, tools and frameworks developed will endure long-time and contribute to tangible impact on ATMP development and patient access across Europe.

The JOIN4ATMP consortium at the annual progress meeting of 2025 in Barcelona, Spain

Generating a pan-European evidence base on ATMP development

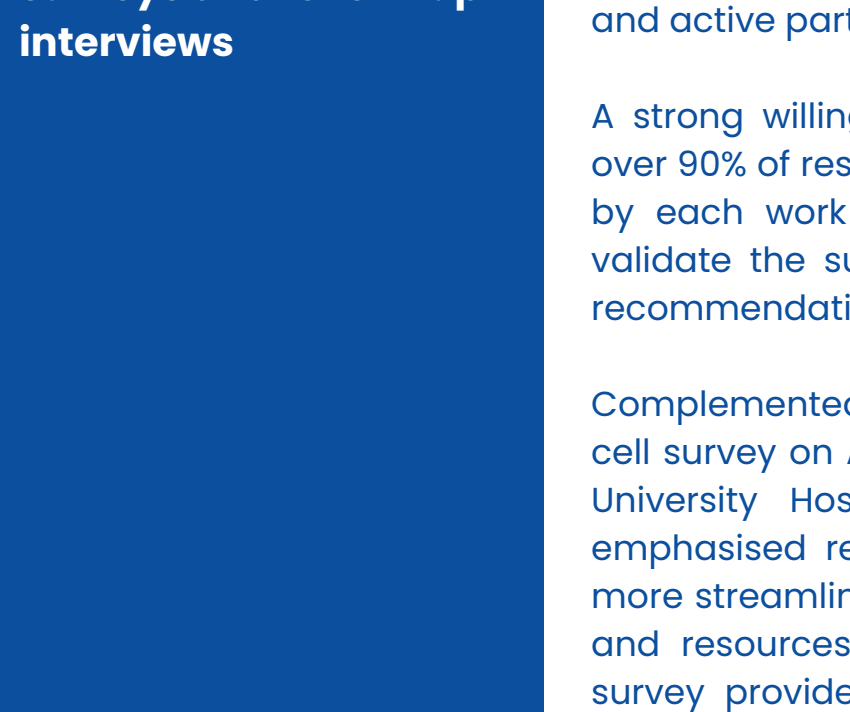
ATMP stakeholder surveys and follow-up interviews

In 2025, JOIN4ATMP completed a large stakeholder survey capturing real-world experiences across the ATMP development lifecycle and highlighting regulatory, clinical, manufacturing, economic and societal challenges. The survey gathered input from a highly experienced ATMP community of 260 stakeholders across more than 20 European countries, reflecting a strong representation from academic and hospital-based ATMP developers with interest in rare diseases. The survey revealed that nearly 80% of respondents have over five years of ATMP experience, with almost 50% currently involved in ATMP development, indicating a highly knowledgeable and active participant base.

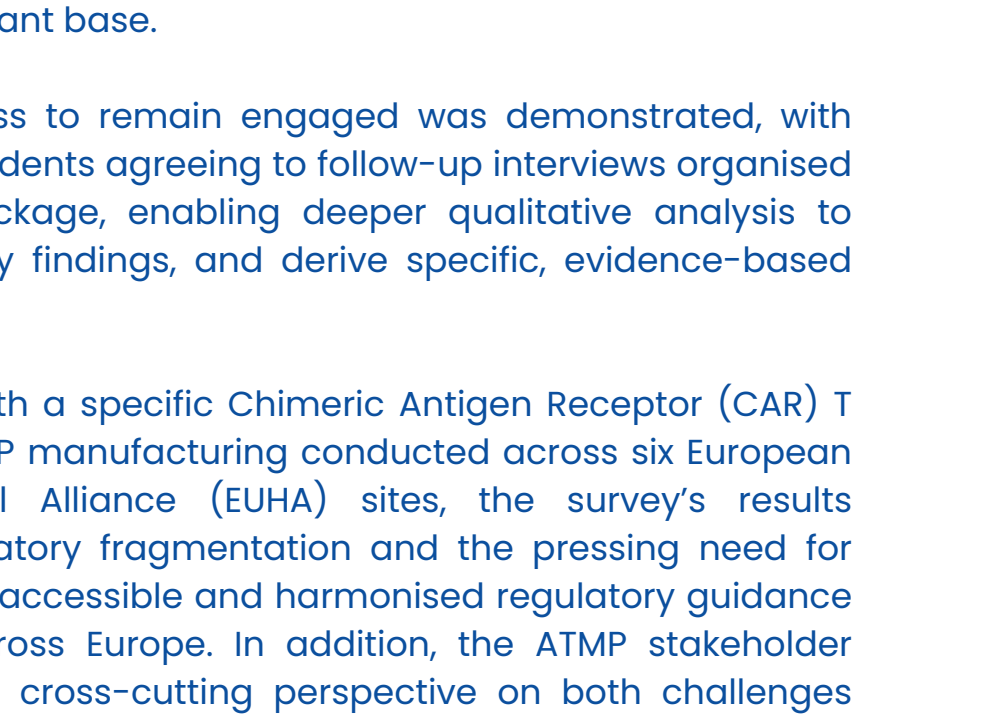
A strong willingness to remain engaged was demonstrated, with over 90% of respondents agreeing to follow-up interviews organised by each work package, enabling deeper qualitative analysis to validate the survey findings, and derive specific, evidence-based recommendations.

Complemented with a specific Chimeric Antigen Receptor (CAR) T cell survey on ATMP manufacturing conducted across six European University Hospital Alliance (EUHA) sites, the survey's results emphasised regulatory fragmentation and the pressing need for more streamlined, accessible and harmonised regulatory guidance and resources across Europe. In addition, the ATMP stakeholder survey provided a cross-cutting perspective on both challenges and potential solutions on preclinical testing, patients' engagement and public awareness of ATMPs, underlining the need for clearer communication on ATMP mechanisms, benefits and access pathways.

<u>Representation of Countries:</u>



<u>Representation of Institutions:</u>



Survey demographics & institutional representation

Learning from real-world use cases and real-world data

Systematic analysis of real-world use cases, identifying gaps

These use cases cover the full development pipeline from basic research to market approval, providing comprehensive insights into the ATMP development landscape.

Six real-world ATMP use cases

JOIN4ATMP systematically analysed six real-world ATMP use cases spanning from rare diseases to oncology, including gene therapies and advanced cell therapies. Insights from individual cases were synthesised through cross-country SWOT analyses, comparing national regulatory, clinical and marketing evaluation as well as policy environments for ATMP development. The systematic analysis of these cases created a shared empirical basis for identifying both successful practices and recurring bottlenecks across Europe. Additional four use cases focused on Good Manufacturing Practices (GMP) that were selected and analysed, providing valuable perspectives on practical challenges and expectations related to the adoption of innovative technologies in manufacturing settings. These use cases cover the full development pipeline from basic research to market approval, providing comprehensive insights into the ATMP development landscape.

Gaps in real-world data and real-world evidence integration

JOIN4ATMP also identified significant gaps in how real-world data and real-world evidence are integrated into health technology assessment and reimbursement decisions. These analyses provided rare, evidence-based insight into why clinically successful ATMPs may still fail to reach patients.

Mapping regulatory knowledge gaps

In parallel, JOIN4ATMP mapped regulatory knowledge gaps experienced by academic and non-commercial ATMP developers. A comprehensive review of regulatory advice mechanisms across national authorities revealed uneven accessibility and limited tailoring to non-commercial stakeholders. Patient and public perspectives were also assessed through a systematic review of recent literature, highlighting persistent concerns related to awareness, ethical considerations, trust and equitable access.

Together, these findings underscored the importance of early dialogue, transparency and societal engagement as integral components of sustainable ATMP development.

Digital and analytical tools for ATMP development

One of the central achievements of year 2 was the further development of digital and analytical tools supporting ATMP development analysis.

Implementation of web-based database

During year 1, JOIN4ATMP designed a curated database covering 678 ATMP clinical trials conducted in Europe linking preclinical development, clinical trial characteristics and regulatory information. The database includes comprehensive parameters such as trial phase, mode of action, lead institution and disease indication, providing a robust foundation for evidence-based decision making.

During year 2, this database was implemented through a Streamlit web-based application, with a user-friendly interface enabling structured filtering by ATMP type, disease indication, development stage and geographic origin. Currently under testing by consortium partners, the web-based application is planned to enable editing and entry functionality to support real-time updates and expansion.

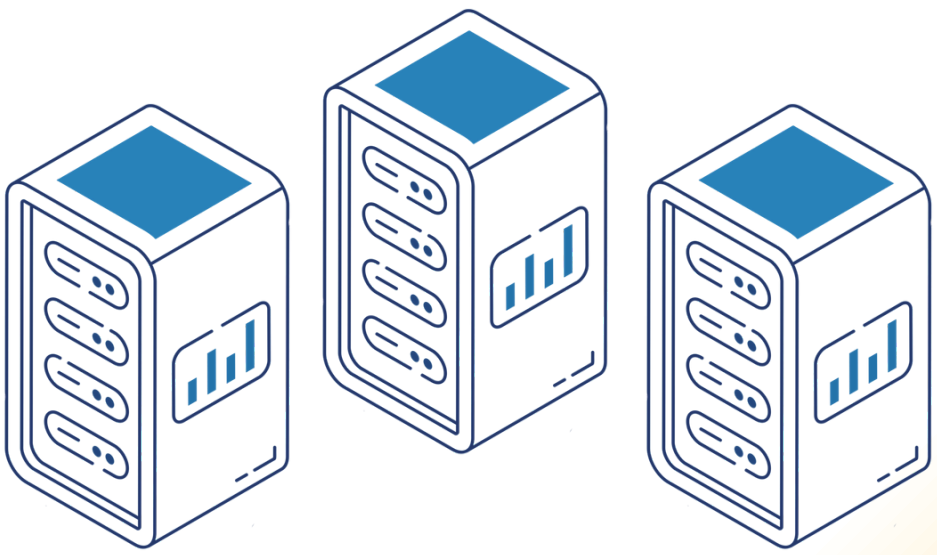
This digital infrastructure represents a shift from static reporting to a living, analytical and sustainable resource.

Publically accessible European map of ATMP GMP facilities

It provides structured information on manufacturing sites, capabilities and regulatory status across EU Member States. This mapping exercise made visible the geographic distribution of GMP capacity and highlighted disparities in access and infrastructure. At the end of year 2, this map covered GMP facilities of nine EU member states. The map was actively disseminated through JOIN4ATMP communication channels, including LinkedIn, to get user feedback from the JOIN4ATMP community, increasing its visibility and practical value for developers, regulators and policymakers.

ATMP economic evaluation tool

To support sustainable ATMP development, JOIN4ATMP introduced a practical ATMP economic evaluation tool based on a risk-adjusted Net Present Value model. The tool enables developers to assess development costs, timelines, risks and potential value early in the lifecycle. In June 2025, the model was successfully tested live with academic stakeholders during the GeneNovate Workshop in Berlin, demonstrating its usability and relevance beyond industry settings.



Policy-relevant recommendations and regulatory impact

Feedback on the EU Biotech Act

As a joint consortium activity, JOIN4ATMP took part in a public consultation and submitted its feedback on the EU Biotech Act suggesting specific measures addressing criticalities related to the ATMP fields from an academic and non-profit perspective, especially for rare diseases. Notably, the European Commission acknowledged for adoption some suggestions proposed, such as the elimination of 50 days for the evaluation of ATMP clinical trial applications and the definition of "Centres of Excellence for Advanced Therapies".

Contribution to European Medical Agency guidelines

JOIN4ATMP experts also contributed to the European Medical Agency (EMA) guidelines on "quality, non-clinical and clinical requirements for investigational advanced therapy medicinal products in clinical trials", ensuring that academic and non-commercial development perspectives were reflected.

This active engagement with regulatory bodies demonstrates JOIN4ATMP's role in addressing European ATMP policy and regulatory frameworks.

Shared lessons across projects

In 2024, two selected use cases on GMP technology were discussed in dedicated multi-stakeholder settings, including a joint T2evolve/JOIN4ATMP roundtable session at ESGCT 2024 and 4th QIG Lessons and Learn Focus Group (LLFG) from EMA, respectively. Shared lessons, focusing on regulatory strategies and including JOIN4ATMP contributions, have been translated in 2025 from T2Evolve and EMA into impactful reports, positioning JOIN4ATMP as a reference initiative within the broader European ATMP policy landscape.

In 2025, JOIN4ATMP started to translate its evidence base into policy-relevant input and recommendations at European level.

Recommendations for the European Commission

Building on evidence from real-world use cases, stakeholder engagement and comparative analyses, JOIN4ATMP drafted a set of interim recommendations to be shared with the European Commission. The recommendations are being developed through a collaborative process involving all work packages and will address key bottlenecks identified through the project's comprehensive evidence-gathering activities. They aim to improve the overall framework for ATMP development and access, covering regulatory and manufacturing conditions, evidence generation and use, clinical development pathways, patient engagement and long-term economic sustainability, to support a more coherent and predictable European ATMP ecosystem.

The road towards sustainability - Setting the framework for cross-border cooperation

JOIN4ATMP laid the foundations for sustainable cross-border cooperation in ATMP development by designing a set of complementary and mutually reinforcing frameworks.

A **legal framework** enables cross-border ATMP clinical trials, approved by different institutional legal departments, representing an operational success in facilitating EU-wide trials. In parallel, JOIN4ATMP advanced the conceptualisation of an **EU-wide GMP coordination hub**, developing a structured blueprint that defines core coordination services, governance arrangements, funding and sustainability considerations. This work constitutes a concrete step toward long-term European coordination of ATMP manufacturing beyond the project's lifetime, with the aim of reducing duplication, fragmentation and inefficiencies. Finally, JOIN4ATMP designed the conceptual framework for a **European ATMP expert platform**, informed by mapped needs across developers, regulators, clinicians, HTA bodies and patient organisations. Envisioned as a long-term knowledge-sharing and coordination mechanism, this platform is intended to support structured information exchange, regulatory preparedness and collaboration across the European ATMP ecosystem.

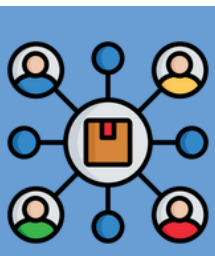
Together, these frameworks address regulatory, operational and knowledge-sharing barriers that currently limit efficient European collaboration and the progression of ATMPs from early development to patient access. They are designed to be complementary and mutually reinforcing, creating a comprehensive ecosystem for long-term ATMP development in Europe.

Community engagement and public outreach

JOIN4ATMP results were shared through joint roundtables, including a T2Evolve roundtable at the 7th European CAR-T Cell Meeting, and through sustained collaboration with related European initiatives such as EUCOPE, TRANSFORM, ReSHAPE, ATMP Sweden, ERDERA and PRECISEU.

The project's LinkedIn community grew beyond 800 followers, reflecting sustained engagement and increasing visibility of JOIN4ATMP outputs. A comprehensive literature review including 276 publications of clinical trials from 2022 to 2024 across CAR-T therapies, gene therapies and somatic cell therapies provides a methodological and statistical perspective on current ATMP clinical development and has been accepted by the "Molecular Therapy Methods & Clinical Development" journal.

Patient engagement remained a core focus of JOIN4ATMP's outreach activities. In 2025, a dedicated webinar series registering over 130 participants and involving multiple JOIN4TAMP partners was organised to address ATMP development challenges in rare diseases and CAR-T therapies for rare cancers. These activities, integrating patient perspectives with regulatory and clinical insights, were closely aligned with JOIN4ATMP's broader work on public awareness, ensuring that societal and patient perspectives are integrated into ATMP innovation.



Since the project started, JOIN4ATMP recorded more than 50 outreach activities, including conferences, workshops, roundtables and webinars, reaching diverse audiences across academia, industry, policy and patient communities.

“Through JOIN4ATMP, we have been able to systematically capture real-world challenges and opportunities in ATMP development. Our priority now is to ensure that the evidence, tools and frameworks developed are taken up beyond the project and make a lasting difference for patients across Europe.”

Annette Künkele-Langer
JOIN4ATMP coordinator

The JOIN4ATMP consortium

CHARITE	Charité – Universitätsmedizin Berlin
UNISR	Università Vita Salute San Raffaele
AUH	Aarhus University Hospital
APHP	Assistance Publique – Hôpitaux de Paris
ErasmusMC	Erasmus Universitair Medisch Centrum Rotterdam
EURORDIS	European Organisation for Rare Diseases Association
EURICE	European Research and Project Office GmbH
FHOFER	Fraunhofer Gesellschaft zur Förderung der Angewandten Forschung e.V
FTELE	Fondazione Telethon ETS
UZL	Katholieke Universiteit Leuven
KUH	Region Stockholm - Karolinska University Hospital
MUV	University Hospital AKH Wien & MedUni Wien
UKW	University Hospital Würzburg
VHIO	Vall d’Hebron Institute of Oncology
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