2024

Looking back on Year 1

Advanced Therapy Medicinal Products (ATMPs) are innovative gene, cell, and tissue-based medicines designed to treat or cure diseases with limited or no current treatment options. Despite efforts by the European Medicines Agency and EU initiatives, regulatory and legal frameworks remain focused on standard drug production, while the evolving nature of ATMPs requires adaptable and dynamic approaches.

The JOIN4ATMP project brings together international experts and stakeholders to drive a reform of EU policies and accelerate ATMP development. With 14 core partners involved in ATMP development, production, application and regulation – alongside patient representatives - JOIN4ATMP leverages real-world experiences to rigorously map hurdles, chart solutions, and deliver actionable recommendations in collaboration with regulatory, economics and policy-making advisors.

JOIN4ATMP, launched in January 2024, has made significant achievements over the past year. This progress reflects the collaborative efforts of an enthusiastic consortium that works in a coordinated way throughout 7 different Work Packages (WP) to navigate challenges and determine the best paths forward. We are excited to provide an overview of our main milestones to date and share our plans for the year ahead!

Governance and Organizational Structure

JOIN4ATMP has implemented a comprehensive governance and communication framework, including a Steering Committee, a Communication Committee, and a Scientific Advisory Board. Key developments include monthly coordination meetings, strategic communication efforts, and expert guidance on vital topics such as ATMP database development and GMP technology

In the first months of JOIN4ATMP, the governance and organizational framework were established to ensure efficient coordination and decision-making.

JOIN4ATMP Steering Committee

The primary governing body is the **JOIN4ATMP Steering Committee**, which gathers every month Work Package (WP) leaders and members of the patients' organization EURORDIS and the EU-consortia T2Evolve and RESTORE. These monthly meetings align activities across WPs, monitor milestones, address challenges, and propose solutions. Discussions have focused on real-world data collection, stakeholder questionnaires, the development of an ATMP database, and specific challenges requiring decision-making solutions.

JOIN4ATMP Communication Committee

The **JOIN4ATMP Communication Committee** plays a critical role in shaping the project's communication strategy. It ensures the quality of communication outputs, engages the broader community (>500 followers on JOIN4ATMP LinkedIn page) and promotes the project through dedicated activities. The first communication activities involved the design of the JOIN4ATMP website, an introductory article on ATMPs, the display of the consortium's geographical diversity, and partner presentation videos on social media platforms.

The Scientific Advisory Board (SAB)

The Scientific Advisory Board (SAB), selected by the Steering Committee, provides expert guidance across various fields, including GMP technology, clinical research, regulatory affairs, policy makers, academia, ethics, health economics, and patient advocacy. This board, consisting of 9 international experts, held its first meeting in September 2024. During this meeting, key topics such as ATMP database development, GMP technology, and patient perspectives were discussed, highlighting significant areas for consortium focus. These relevant aspects will be addressed and rediscussed during the second SAB meeting in Spring 2025.

Spreading JOIN4ATMP's word: Communication and Dissemination actions

In 2024, JOIN4ATMP partners actively engaged in key conferences and events to present its activities and foster collaborations with stakeholders. The project's annual meeting in Milan emphasized long-term sustainability and stakeholder engagement, while a joint session during the World Orphan Drug Congress discussed development pathways for ATMPs in Europe.

Conferences and Joint Event

The project has been represented by the JOIN4ATMP Coordinator and core partners at **World EPA Congress (Amsterdam), NOTA Congress (Turin), 6th European CAR T-cell Meeting (Valencia), ESCGT (Rome), EUCOPE's Cell and Gene Therapy Working Group**. These events represented key opportunities to foster synergies with advisors and stakeholders in academia, pharma, industry, healthcare pricing and access, and create future collaboration. In the frame of the **ESCGT conference** in Rome, a **joint JOIN4ATMP/T2EVOLVE** roundtable was held, showcasing two real case examples of parent-child platform for CAR-T cell therapies targeting solid tumors, and platform approaches for ex-vivo gene therapies for lysosomal storage disorders. In addition, **a special JOIN4ATMP session** was held during **the World Orphan Drug Congress (WODC)** to discuss the threats and opportunities during the new development pathways for ATMPs. These talks ultimately emphasized JOIN4ATMP's capacity to answer to these needs and gather relevant actors around ATMP development.

JOIN4ATMP Annual Meeting/EUHA Symposium

The **1st JOIN4ATMP Annual Meeting** was held in **Milan in November 2024**. During this event, partners shared updates on the project's progress and key achievements. Key highlights of the day were the workshop sessions, where partners worked in small groups and in an interactive way to explore the major challenges and align tasks across various work packages. Particular emphasis was given to the long-term sustainability of the project, with a focus on strengthening stakeholder engagement for lasting impact.

During the second day of the meeting, partners attended the **EUHA Symposium**: ATMP frameworks from policy and regulatory perspectives were explored, with JOIN4ATMP's coordinator presenting the project as a proactive solution to challenges in ATMP access, particularly from an academic manufacturing angle.

Case study presentation on platform technology

An abstract has been selected to be presented to a restricted audience of the **4th QIG Lessons and Learn Focus Group (LLFG)** meeting of EMA, with representatives from academia and industry. The purpose of this meeting was to discuss examples of platform technologies and case studies, providing an opportunity to showcase the JOIN4ATMP platform approach for developing gene therapy products. This was done in collaboration with T2Evolve, with the objective of engaging with regulators.

Tools and Systems Development

JOIN4ATMP developed a health economic analysis tool and a comprehensive ATMP clinical trial database, focusing on sustainable pricing frameworks and mapping current ATMP landscapes. These tools, tested with real-world scenarios, aim to advance ATMP translation and address regulatory hurdles by analyzing preclinical and clinical trial data. Key developments include monthly coordination meetings, strategic communication efforts, and expert guidance on vital topics such as ATMP database development and GMP technology.

ATMP economic model tool

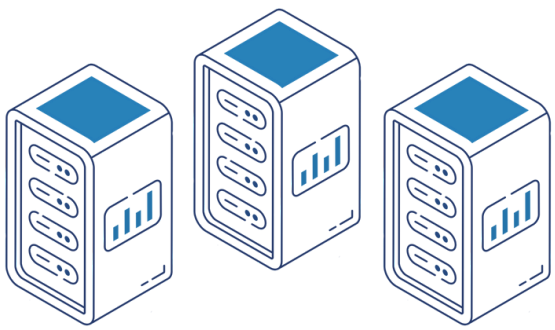
JOIN4ATMP aims at creating a business model ensuring a sustainable and fair pricing framework for academic or not-for-profit license holders, ultimately benefiting national health systems and advancing ATMP translation. This approach involved the development of a health economic analysis tool and its testing by using digital simulations based on real-world case scenarios.

A beta version of the **ATMP health economic analysis tool has been successfully developed**. This first version of the model includes comprehensive parameters such as target and general population estimates, comparator analysis, R&D costs and timelines, pricing, success probabilities, royalty obligations, and discount rates.

ATMP clinical trial database designed

Mapping the current ATMP landscape will help to predict the clinical translation of future ATMP developments. By analysing ATMP preclinical results currently used in clinical applications, partners are able to evaluate the correlations between preclinical safety and efficacy studies, and their predictability or reproducibility in patients.

For this purpose, a preliminary **ATMP database has been developed**. ATMP related entries were extracted from the European Union Drug Regulating Authorities Clinical Trials Database (EudraCT), that contains all the interventional clinical trials on medicinal products, by designing programmatic queries in Python language. This in-depth search query returned **727 ATMP clinical trials** in Phase I or Phase II, subsequently filtered for medicinal product and/or disease category. ATMP-related clinical trials are being manually curated by extracting and categorizing data from the EudraCT database and reviewing PubMed literature, while a browser-based WebApp (Dashboard) is being developed to visualize the manually curated database. The dashboard will offer a broad ATMP overview, enabling the JOIN4ATMP consortium members to search for the ATMP clinical trials of their interest. Moreover, members can filter the database based on factors such as trial phase, type, region, and disease. Hurdles preventing the translation will be mapped, and potential solution for easing ATMP developments will be charted.



Real-World Impact and Data-Driven Progress

Use-cases identified

JOIN4ATMP aims at addressing regulatory and policy roadblocks currently preventing rapid ATMP clinical translation. This approach involves collecting real-life experiences of academic and SME developers in EU countries to map regulatory variations from the applications for ATMP clinical trials to marketing authorization. By mapping real-world use cases, the study gathered experiences from early clinical trial development to regulation and market approval for different ATMP, including differences between EU states and ATMP market schemes.

The analysis identified **7 real-world use cases** (PragmaTIL as a an example in the box on the right) covering diverse areas such as oncology and rare diseases. Four of these use cases have been finalized, two are under the final review process and one is under development. These cases cover the full development pipeline, from basic research to market approval, involving multiple JOIN4ATMP work packages. The analysis provided a detailed mapping of regulatory hurdles and offered a comprehensive overview of current solutions in the ATMP field within the EU, thus contributing to a better understanding of the challenges and opportunities for streamlining ATMP development.

Stakeholder communities and questionnaire development

Real-world insight and interview experiences will support the creation of dedicated recommendation guidelines. As a first step, the JOIN4ATMP consortium collectively identified **over 300 stakeholders belonging to 15 different target groups**, ranging from the preclinical to the clinical field, academia, manufacturing, economic, ethics, and legal framework, including patients' representatives. In parallel, outstanding questions have been identified and selected as a joint effort across JOIN4ATMP Work Packages. **A dedicated questionnaire compiling about 130 questions addressed to different stakeholder groups has been developed** and implemented in the RedCap system. A first internal test round reviewed the survey before distributing it to selected stakeholders. To ensure the legal framework of the survey, an ethics application was submitted and successfully approved. Additionally, a data management plan was prepared and submitted. Survey answers will then be analysed and the outcome will be the basis for interviews with the experts.

PragmaTIL

PragmaTIL pioneers decentralized, multi-center clinical trials for Tumor-Infiltrating Lymphocyte Adoptive Cell Therapy (TIL-ACT) in melanoma, lung, and cervical cancer. For the first time in Europe, multiple academic centers will independently produce TILs under a unified protocol, setting a new standard for academic trials. PragmaTIL aims to develop TIL-ACT with reduced toxicity while maintaining efficacy. Its decentralized model enables centers to manage TIL production and treatment under a shared protocol, addressing logistical and regulatory challenges. JOIN4ATMP leverages PragmaTIL to assess regulatory approval, identify obstacles, and evaluate implemented solutions.

"The first year of activity has been very revealing of the broad scope of JOIN4ATMP and the highly collaborative dimension that the consortium and stakeholders embody. With significant progress across several work packages and strong representation at external events, it is inspiring to witness initial plans gradually evolving towards lasting European outcomes."

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

RESTORE

T2EVOLVE

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