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Q&A on EMA pilot offering enhanced support to academic and non-profit developers of Advanced Therapy Medicinal Products (ATMPs)

What is the goal of the academic support pilot project?

The objectives of the pilot are primarily to increase the level of translation of ATMPs developments into late-stage development and ultimately authorised medicines that can make a difference in patients' lives in Europe by:

- Providing enhanced support to selected applicants from the academic sector[1] during the development of promising advanced therapies to ensure these products will meet regulatory standards in terms of quality, safety and efficacy, and also if needed during the planning and submission of marketing authorisation applications.
- Identifying and addressing potential gaps in the existing regulatory tools and guidance available to applicants, with a particular focus on challenges experienced by developers from the academic sector.

Importantly no new regulatory tool will be introduced as part of this pilot, and the main support will consist of in kind regulatory and scientific support in relation to existing regulatory processes and tools.

Who can apply?

The pilot is open to applicants from the academic sector[1] who are developing ATMPs in the European Union[2], have generated at least some proof-of-principle data and might benefit from Agency's support; as part of the pilot, they would therefore be committed to collaborate with the Agency during development, e.g. seek and follow scientific advice.

The Agency will confirm academic status based on the information in the application form but additional information may be requested during the selection process.

What are the benefits of taking part in the pilot?

Financial: fee incentives[3] will be applied to the five academic developers.

[1] Defined in the Framework of collaboration between the EMA and academia (EMA/125511/2017)
[2] For further information, see below under the question: 'What are the selection criteria?'
[3] See Decision of the Executive Director on fee incentives for scientific advice, marketing authorisation applications and pre-authorisation inspections in the ATMP support pilot for academia

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In-kind: EMA will:

- appoint a dedicated EMA contact point and EMA support team to provide regulatory and scientific support;
- organise regular teleconferences with the product developers as appropriate to check progress and identify potential needs for additional support and complement interaction mechanisms under existing tools, such as PRIME;
- optimise the use of all existing tools such as pre-submission meetings, to support further the developers during regulatory interactions for e.g. scientific advice, PRIME, marketing authorisation;
- discuss the development plan with pre-planned time and action points for regular assessment of level of maturity, co-decisions and stopping points (when/reasons to cease the additional support to academic developer);
- provide the support as available to SME developers[4].
- Provide the support for proactively identifying questions for which scientific advice should be sought, maintaining regular interactions with dedicated contact points (see next section) and developing an integrated regulatory life cycle knowledge base ("regulatory history") for the product.

What are the selection criteria?

The key principles for the selection of the development programs to be enrolled in the pilot are the following:

1. The product is an ATMP; In case of doubt, the developer may be asked to submit first a request for ATMP classification[5].
2. The applicant is from the academic sector, including: public / not-for-profit hospitals or research organisations and hospitals, Higher Education Institutions (HEI), Public-Private Partnerships / Consortia, International Research Organisations.
3. The applicant is established in the European Union. Consortia with EU and non-EU participants can apply, provided that the principal investigator is located in the EU and EU patients are enrolled in clinical trials.
4. The product in development must target an indication in an area where there is an unmet medical need, because this product may offer a major therapeutic advantage over existing treatments, or benefit patients with no or few treatment options.
5. The product is of promising activity and has the potential to address to a significant extent an unmet medical need as supported by preliminary clinical evidence in patients (i.e. clinical response and safety data in patients in the targeted indication generated in exploratory clinical studies) or compelling non-clinical data in a relevant model coupled with data from first in man studies indicating adequate exposure for the desired pharmacotherapeutic effects and tolerability. It would also be expected that the mechanism of action has been investigated in non-clinical (in vitro/in vivo) studies. In addition, a sound justification on the potential for the product to address an unmet medical need will need to be provided. Taken into account the

[4] See here for more information: Support to SMEs | European Medicines Agency (europa.eu)
[5] See here for more information: Advanced therapy classification | European Medicines Agency (europa.eu)

limited number of products (total of 5 applicants will be selected) and the timeframe associated with the pilot, the selection panel will prioritize candidates for which the Agency's support could be more impactful. Note: Such data would ideally be generated in clinical trial, but additional clinical evidence, e.g. from compassionate use programmes or hospital exemption use could be presented.

6. The quality development of the product is of sufficient maturity to ensure the program will likely benefit from additional support in advancing further towards later stage of clinical development and/or towards a marketing authorisation application in the European Union rather than solely under hospital exemption. The maturity of the quality development will be judged upon the available information on the pharmaceutical process, and reference to (planned) manufacture of the product in line with Good Manufacturing Practices.
7. The applicant has freedom to operate (e.g. via intellectual property rights of the product under development) and full access to the data related to the development and manufacture of the product, e.g. control of critical starting materials (e.g. the viral vector to manufacture genetically modified cells). The sponsor must be free from operating agreements with any pharmaceutical company.
8. The applicant is willing to collaborate with the Agency and follow the recommendations given through the pilot's enhanced support; The applicant must also be adequately resourced and have a sound understanding of the regulatory framework for the development of an ATMP towards the conduct of clinical trials and/or future marketing authorisation applications and be able to interact effectively with regulatory authorities. Ideally a person with experience in the different sectors of product development, including regulatory affairs is available to provide support to the applicant, either internally or via a consultant.

Does the product selected for the pilot also need PRIME eligibility?

PRIME eligibility is not a prerequisite nor provides automatic eligibility to the pilot, however products selected for the pilot by the Agency may also have been or be granted PRIME due to selection criteria overlap (e.g. addressing an unmet need, promising data; see PRIME - Priority medicines (europa.eu) for details).

For ATMPs already granted PRIME eligibility that apply for the ATMP pilot, EMA will, as part of the selection process, consider the need for the additional support and benefits of the pilot (see above) as an add-on to the developmental support already offered via the PRIME scheme.

How and when to apply?

Potential applicants are asked to complete the ATMP PILOT APPLICATION FORM (available on the EMA website as of January 2023) and submit it via ATMPpilot@ema.europa.eu An initial pre-selection will then be done based on the information provided at time of application and successful candidates will then be invited to a meeting organised by the EMA ITF team where they will have the opportunity to interact with EMA and provide further information on their project which will then allow the Agency to proceed with the final selection. Of note, once applicants are invited to a meeting, most interactions will be managed via IRIS and therefore applicants will need to register their organisation in the online platform IRIS and request a research product identifier (RPI) if not available yet. More information is available at https://iris.ema.europa.eu/RPI but further assistance can also be provided if needed.

The goal is to select up to 5 by end of 2024. Applicants should apply by end of April 2024 as the next pre-selection of candidates is scheduled to take place in June 2024 and the final selection in November 2024.

Is there a link between the academic pilot and hospital exemption (HE)?

There is no direct link between (HE) and the academic pilot. The aim of the pilot is to increase translation of products reaching patients in the EEA via the conduct of clinical trials and ultimately regulatory approval via an enhanced collaboration with academic developers; this is regardless of whether these products may have been made available under an HE in one or several countries in the EU.

Taking also into account that HE is implemented differently in the EU member states, HE is not a prerequisite for an academic ATMP to be selected, but it is not an impediment either.

What happens once entering the pilot? Who is involved?

The applicant will be provided with a dedicated point of contact in the relevant therapeutic area office from the Agency and depending on the stage of development and nature of the program, relevant EMA staff members will be appointed to provide support throughout participation to the pilot. These may include for example quality, risk management, therapeutic area, scientific advice and paediatric specialist, but we may also reach out to colleagues from the Data Analytics and Methods (TDA) or Regulatory Science and Innovation (TRS) Task force where appropriate.

Then during the pilot, the applicant may be invited for preparatory teleconferences to help planning for regulatory submissions/interactions, and debriefs before and/or after regulatory interactions to collect feedback on the support and identify potential gaps.

What are the anticipated milestones for the pilot?

The Agency plans to select up to 5 candidates by the end of 2024; 3 candidates were already selected by end of 2023.
The progress will be closely monitored, and initial results of the pilot are expected to be available in 2025. Upon completion, a report will be published and a workshop with relevant stakeholders may be organised to discuss the learnings.