IRDiRC
INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

Orphan Drug Development Guidebook

Building Block E133

This document defines the content of the Building Block created for each identified tool, incentives, initiative or practice introduced by public bodies or used by developers to expedite drug development in Rare Diseases (RDs).

ITEM	DESCRIPTION
Building Block (BB) Title	Advanced Therapy Medicinal Products (ATMPs) Classification
References	https://www.ema.europa.eu/en/human-regulatory/marketing-authorisation/advanced-therapies/advanced-therapy-classification
Description	Companies can consult the European Medicines Agency (EMA) to determine whether a medicine they are developing is an advanced therapy medicinal product (ATMP). This is an opportunity for ATMP developers to receive confirmation that a medicine, which is based on genes, cells or tissues, meets the scientific criteria for defining an ATMP. The procedure can help developers to clarify the applicable regulatory framework since the beginning of the product development. The use of this procedure is essential for ATMPs developers especially with borderline products. EMA's Committee for Advanced Therapies (CAT) delivers scientific recommendations on ATMP classification after consultation with the European Commission (EC) within 60 days after receipt of the request.
Category	Regulatory Building Block
Geographical scope	European Union

Availability	Applicants developing ATMPs for rare and non-rare diseases.
Scope of use	The purpose of this procedure is to allow applicants to clarify, in case of doubt, the classification whether a given product based on genes, cells or tissues meets the scientific criteria which define ATMPs, in order to address, as early as possible, questions of borderline with other areas such as medical devices, which may arise as science develops. While the recommendation on classification provided by the Agency is voluntary and not binding, the procedure can help developers to clarify the applicable regulatory framework. It also provides clarity on the development path and scientific-regulatory guidance to be followed. The ATMP classification may sometimes also be a useful tool for applicants to initiate a tailored dialogue on the product development with regulators. Indeed, the ATMP classification, along with other tools (e.g. ITF briefing meetings – E101), should be seen as a first opportunity to engage with regulators. Once the candidate ATMP classification has been clarified and confirmed, the dialogue can continue with the use of other regulatory procedures such as scientific advice/protocol assistance (E103) and ATMP certification (E114) (only for small and medium enterprises). The ATMP classification may also help developers to gain access to all relevant services and incentives offered by the EMA.
Stakeholders	• Any ATMP developer • Committee for Advanced Therapies (CAT) – EMA [and European Commission (EC) for consultation]
Enablers / Requirements	Providing that the product is based on genes, cells or tissues, the applicant should provide information on the product (e.g. on active substance, finished product, mechanism of action and proposed use) and on the status of the development of the product (including element of the manufacturing, quality aspects and outline of the non-clinical and clinical development) relevant for the ATMP classification. Applicants should also substantiate their positions on the classification of their product on the light of legal definitions in force.
Output	The CAT shall deliver to the Applicant its 'scientific recommendation on ATMP classification' after consultation with the European Commission (EC). The EMA shall also publish summaries of this recommendation, after deletion of all information of commercial confidential nature. This summary will consist of the following information: product description, therapeutic area, outcome of the scientific recommendation, date.

Best time to apply and time window	The ATMP classification can be applied for at any stage of the product development, even when non-clinical and clinical data are not available.
Expert tips	- It should be noted that scientific recommendations given by the CAT are always related to a defined product. It is thus not possible to classify scientific 'concepts' in the absence of a clear description of the product. - If additional scientific information becomes available after the original ATMP classification that may impact the classification of the product, the applicant can submit a follow-up request. This should follow the same procedure as the original submission. - The claim can be based either on data and/or on current scientific knowledge, but it has to be sufficiently substantiated in each case. Otherwise, the CAT may only conclude that a product is an ATMP, but not yet if it is, for example, a tissue engineered product or a somatic cell therapy medicinal product. - The summary outcome ATMP classifications assessed so far by the CAT is available on the EMA website. Since 2011, summary reports of all ATMP classifications are published. PROs: – Although clinical trials are under the responsibility of the National Competent Authorities, it is important to stress that the classification recommendation made by the CAT may help when submitting a clinical trial dossier, as the applicant and the concerned competent authorities will be made aware of a European classification position which can clarify and facilitate identification of the most relevant criteria and procedure to be applied.