# Orphan Drug Development Guidebook

## Building Block E114

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 |
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| Building Block (BB) Title | Certification for Advanced Therapy Medicinal Products (ATMPs) |
| References | https://www.ema.europa.eu/en/human-regulatory/research-development/advanced-therapies/advanced-therapy-development/certification-procedures-micro-small-medium-sized-enterprises-smes<br><br>Guideline on the minimum quality and non-clinical data for certification of advanced therapy medicinal products:<br><br>https://www.ema.europa.eu/en/documents/scientific-guideline/guideline-minimum-quality-non-clinical-data-certification-advanced-therapy-medicinal-products_en.pdf |
| Description | The European Medicines Agency's Committee for Advanced Therapies (CAT) provides a certification procedure for advanced therapy medicinal products (ATMPs) under development by micro-, small- and medium-sized enterprises (SMEs) only. This is an opportunity for SMEs to get an assessment of the data they have generated and check that they are on the right track for successful development. |
| Category | Regulatory Building Block |
| Geographical scope | European Union |
| Availability | The certification for ATMP is designed specifically for micro, small and medium enterprises that develop ATMP for rare and non-rare diseases. |
| Scope of use | The certification procedure involves the scientific evaluation of quality data and, when available, non-clinical data that SMEs have generated at any stage of the ATMP |

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| | development process. It aims to identify any potential issues early on, so that these can be addressed prior to the submission of a marketing-authorisation application.<br><br>- The certification aims to facilitate early dialogue between the SMEs and the Regulators and to help SMEs.<br><br>- The certificate could support SMEs who wish to license out their technology or could be used to attract venture capital allowing the SME to further develop their product.<br><br>- Although the certification procedure is an independent evaluation procedure that will not bind the Agency or National Competent Authorities to any future decision about the product, a certificate could facilitate the evaluation of an application for clinical trial authorization provided that these applications are based on the same data. |
| Stakeholders | • SMEs<br><br>• Committee for Advanced Therapies (CAT) – EMA |
| Enablers/ Requirements | To develop an advanced therapy medicinal product (based on genes, tissues or cells). |
| Output | After assessment, the CAT may recommend issuing a certification confirming the extent to which the available data comply with the standards that apply for evaluating a marketing-authorisation application. Following the CAT recommendation, the Agency issues a certification. |
| Best time to apply and time window | The optimum time point to apply for the certification procedure is when the ATMP has reached a level of sufficient development with respect to quality and non-clinical data (please refer to the scientific guideline on the minimum quality and non-clinical data for certification of advanced therapy medicinal products.<br><br>The stage of development will determine the dossier's scientific data content of the certification application: the certification dossier (i.e. data package submitted for certification) is expected to be more comprehensive if a product is in a later stage of development (e.g. already in clinical trial phase). In order to facilitate assessment, the Applicant should provide information about the stage of development and in particular the stage of any study in pre-clinical or clinical setting whether planned, ongoing or completed. |

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| Expert tips | – Certification of quality and non-clinical data is only applicable for ATMPs<br>– Only SMEs can apply for such type of certificate; therefore the Applicant must have already obtained an SME status as per Commission Recommendation 2003/361/EC4 prior to applying for certification procedure<br>– Certification is available for SMEs only, however, Academia/no-profit organizations may apply on exceptional basis<br><br>PROs:<br><br>- Although the certification procedure is an independent evaluation procedure that will not bind the Agency or National Competent Authorities to any future decision about the product, a certificate could facilitate the evaluation of an application for clinical trial authorisation provided that these applications are based on the same data. However, the sponsor is still required to submit the clinical trial authorisation application to the National Competent Authorities according to the Regulation 536/2014. It is not necessary to get a certificate to apply for Clinical trial authorisation.<br><br>- A certificate issued by the EMA is not legally binding with regard to any future regulatory procedure. Any relevant data, even if already certified, should be submitted again for the purpose of any future regulatory procedure. It could, nevertheless, facilitate the evaluation of any future application for clinical trial authorisation or a marketing authorization application (MAA), provided that these applications are based on the same data.<br><br>CONs:<br><br>- The certification procedure cannot be used to review products in their conceptual stage or for 'platform' technologies.<br><br>- The certificate cannot conclude on the benefit/risk profile of the product.<br><br>- The certification procedure does not intend to provide advice for further development of the product, since companies should seek such feedback via the scientific advice procedure. |