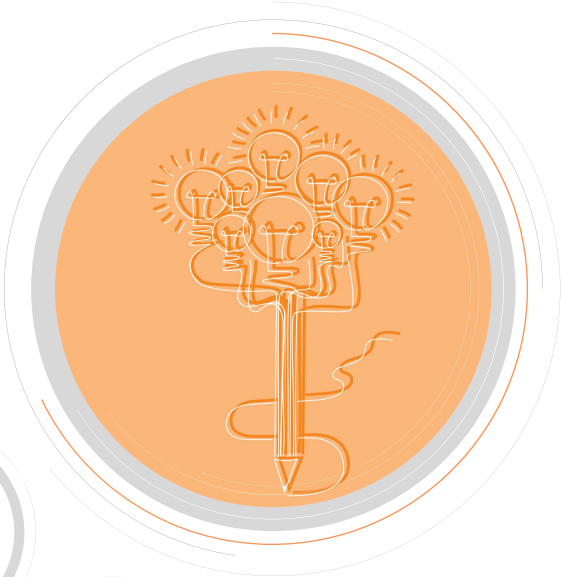
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Support translation of advanced therapy medicinal products (ATMPs) into patient treatments



Underlying actions

EMA's Regulatory Science Strategy to 2025 – Human Stakeholder Workshop

Chaired by Martina Schussler-Lenz, CAT on 18 November 2019
Presented by Ana Hidalgo-Simon, Head of Specialised Scientific Disciplines Department, EMA

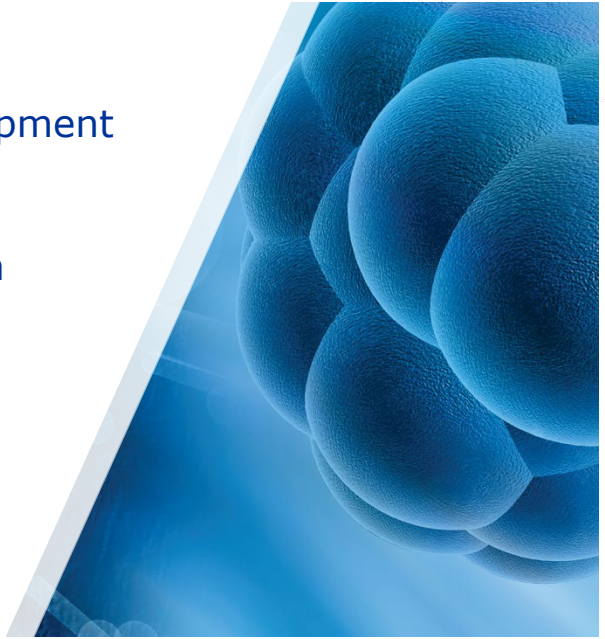
Disclaimer

Comments to the underlying actions represent the views of stakeholders and not the European Medicines Agency.

The fact that these comments from stakeholders are displayed in the presentation does not mean we endorse them or commit to fulfil them in any way.

Support translation of Advanced Therapy Medicinal Products into patient's treatments

- Identify therapies that address unmet medical need
- Provide assistance with early planning, method development and clinical evaluation
- Support evidence generation, pertinent to downstream decision-makers
- Address the challenges of decentralised ATMP delivery locations
- Raise global awareness of ATMPs to maximise knowledge sharing, promote data collection



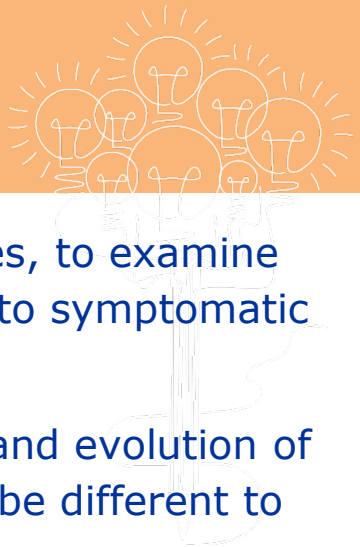
Identify therapies that address unmet medical need: increasing coordination and alignment of EU-wide integrated processes

- Improve cooperation of stakeholders (including European Commission, national agencies and GMO authorities in the Member States) to ensure that the EU is competitive in the ATMP field.
- Address the extension of GMO authorization harmonization across the EU community to additional ATMPs (besides human cells genetically modified by means of retro/lentiviral vectors).
- Leverage existing o provide new platforms for building further continuity between national and EU-level aspects of ATMP development and facilitating increased alignment/convergence between Member States.
- Issue and update guidelines to address principles and not be overly prescriptive.

Provide assistance with early planning, method development and clinical evaluation

- Proactive communication to developers about the usefulness to engage a very early dialogue with the Agency, starting from the classification of their product.
- Efficient continuum of dialogue with stakeholders to allow a more effective way to develop products.
- Dedicated network for ATMPs across regulatory bodies including medicines, medical devices, tissues and cells and environment competent authorities (GMO Bodies).
- EMA to play an important role in fostering increased convergence between Member States and internationally.

Support evidence generation, pertinent to downstream decision-makers

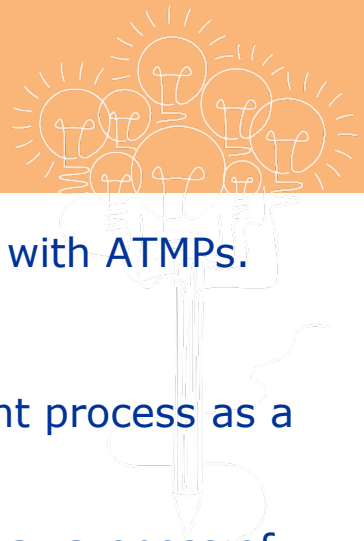


- Promote further multi-stakeholder discussions, in particular HTA bodies, to examine how these products are assessed for efficacy/effectiveness compared to symptomatic treatments.
- Support and help post-marketing activities; ongoing data generation and evolution of methods and clinical situations in a post-approval setting, which may be different to those in clinical trials.

Address the challenges of decentralised ATMP delivery locations

- Address the challenges of ATMP manufacturing (decentralised manufacturing and delivery locations, expedited quality aspect development, conduction and evaluation of comparability exercise…)
- Greater clarity on CMC expectations for submission versus post-marketing commitments (especially for expedited pathways).
- Flexible regulatory framework to allow adoption of more advanced technology once available, leading to continual improvement in the manufacturing processes.

Raise global awareness of ATMPs to maximise knowledge sharing & promote relevant data collection



- Investment into the further familiarisation of healthcare professionals with ATMPs.
- Support cross-sectoral research.
- Take into account the impact on health care setting / patient treatment process as a whole.
- Provide education and training for early stage researchers to increase awareness of regulatory challenges to development and translation.

International cooperation

- To engage with other international regulatory agencies to foster global convergence of requirements for ATMP (including their starting/raw materials, methods and classification) and/or to define new common approaches to assess and approve them.
- Leveraging the best international expertise to achieve such convergence is important to achieve common, science-based evaluation methods and criteria.

Any questions?

Further information

RegulatoryScience2025@ema.europa.eu

Temporary visiting address Spark building • Orlyplein 24 • 1043 DP Amsterdam • The Netherlands
For deliveries refer to www.ema.europa.eu/how-to-find-us
Send us a question via www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000

Follow us on **@EMA_News**

#RegScience2025