PHARMALEX
CONFIDENCE BEYOND COMPLIANCE

ATMP GUIDELINES

Agency Name	ATMP Regulatory Pathway	Links to ATMP Guidelines
European Medicines Agency (EMA)	**Part 4** of the **Annex I** to **Directive 2001/83/EC** and **The Regulation on Advanced Therapy Medicinal Products 1394/2007/EC**	• **EMA/149995/2008** (Guideline on Safety and Efficacy Follow-up and Risk Management of Advanced Therapy Medicinal Products) • **EMA/CAT/80183/2014** (Quality, Preclinical and Clinical Aspects of Gene Therapy Medicinal Products) • **CHMP/GTWP/671639/2008** (Quality, Non Clinical and Clinical Aspects of Medicinal Products containing Genetically Modified Cells) • **EMA/CHMP/410869/2006** (Guideline on Human Cell-based Medicinal Products) • **EMA/CHMP/BWP/271475/2006 Rev.1** (Potency Testing of Cell-based Immunotherapy Medicinal Products for the Treatment of Cancer) • **CAT/CPWP/686637/2011** (Risk-based Approach According to Annex I, Part IV or Directive 2001/83/EC Applied to Advanced Therapy Medicinal Products) • **CHMP/CPWP/83508/09** (Xenogeneic Cell based Medicinal Products) • **CPMP/BWB/2458/03** (Development and Manufacture of Lentiviral Vectors) • **CHMP/GTWP/125459** (Non-clinical Studies Required Before First Clinical Use of Gene Therapy Medicinal Products) • **EMEA/273974/05** (Non-clinical Testing for Inadvertent Germline Transmission of Gene Transfer Vectors) • **CHMP/GTWP/60436/07** (Follow-up of Patients Administered with Gene Therapy Medicinal Products) • **CHMP/GTWP/125491/06** (Scientific Requirements for the Environmental Risk Assessment of Gene-therapy Medicinal Products)

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Food and Drug Administration (FDA)	Same as any investigational biological product (as described in **21 CFR 312.23**)	• Chemistry, Manufacturing and Control (CMC) Information for Human Gene Therapy Investigational New Drug Applications (INDs); Draft Guidance for Industry • Long Term Follow-up After Administration of Human Gene Therapy Products; Draft Guidance for Industry • Testing of Retroviral Vector-Based Human Gene Therapy Products for Replication Competent Retrovirus During Product Manufacture and Patient Follow-up; Draft Guidance • Regulatory Considerations for Human Cells, Tissues, and Cellular and Tissue-based Products: Minimal Manipulation and Homologous Use; Guidance for Industry and Food and Drug Administration Staff • Recommendations for Microbial Vectors Used for Gene Therapy; Guidance for Industry • Draft Guidance for Industry: Assay Development for Immunogenicity Testing of Therapeutic Proteins • Considerations for the Design of Early-Phase Clinical Trials of Cellular and Gene Therapy Products; Guidance for Industry • Guidance for Industry: Preclinical Assessment of Investigational Cellular and Gene Therapy Products • Guidance for Industry: Potency Tests for Cellular and Gene Therapy Products • Eligibility Determination for Donors of Human Cells, Tissues, and Cellular Tissue-based Products; Guidance for Industry • Guidance for Industry: Gene Therapy Trials – Observing Subjects for Delayed Adverse Events • Guidance for Industry: Supplementary Guidance on Testing for Replication Competent Retrovirus in Retroviral Vector Based Gene Therapy Products and During Follow-up of Patients in Clinical Trials Using Retroviral Vectors • Guidance for Industry: Guidance for Human Somatic Cell Therapy and Gene Therapy