Training Course Agenda

PDA EU00190 CMC Regulatory Compliance Strategy for ATMPs and CGTPs (Advanced Therapy Medicinal Products - Cellular/Gene Therapy Products)

DAY 1 – Monday 23 September 2024	
9:00	**Welcome and Introduction** • Collecting Participants Expectations
9:10	**Overview of the ATMP/CGTP Landscape (90 min)** • Discussion of the increasing diversity of these market-approved advanced therapy medicinal products • The critical role that Genetic/Process/Analytical Development groups, Manufacturing, QA/QC and Regulatory Affairs have in assuring that CMC regulatory compliance protects patients
10:30	***Coffee Break***
11:00	**CMC Regulatory Compliance Differences (90 min)** • Overview of the FDA/EMA regulatory systems in place for ATMPs/CGTPs (IND → BLA; IMPD → MAA) • Why these products are not regulated like chemical drugs (or in some cases, even monoclonal antibodies)
12:30	***Lunch Break***
13:30	**Risk-Managed CMC Regulatory Compliance Strategy for ATMPs/CGTPs (90 min)** • Why a risk-managed approach to CMC regulatory compliance is absolutely necessary for these products • Introduction to the 'Minimum CMC Regulatory Compliance Continuum' risk-based approach
15:00	***Coffee Break***
15:30	**Recommended Risk-Based Approach for ATMPs/CGTPs (90 min)** • How three interactive risk-based components protect patients – CMC Regulatory, cGMPS, Quality System • Application of Quality by Design (QbD) – QTPP, CQA, CPP – and Quality Risk Management (QRM)
17:00	***End of Training Course Day 1***

DAY 2 – Tuesday 24 September 2024	
9:00	**<u>Applied</u> Risk-Managed CMC Regulatory Compliance Strategy Across the Manufacturing Process (90 min)** • ATMP/CGTP raw materials and components – risk reduction procedures • ATMP/CGTP starting materials (e.g., DNA plasmids, viral vectors, patient cells, stem cells) • ATMP/CGTP upstream production processes (e.g., AAV viral vector by transient plasmid transfection, patient cell transduction by LV viral vector, mRNA non-viral vector by IVT)
10:30	***Coffee Break***
11:00	**<u>Applied</u> Risk-Managed CMC Regulatory Compliance Strategy Across the Manufacturing Process (90 min)** • ATMP/CGTP downstream purification processes (e.g., AAV viral vectors, mRNA non-viral vectors) • ATMP/CGTP formulation and drug product manufacturing processes (e.g., AAV viral vectors, mRNA/LNP non-viral vectors, genetically modified patient cells) • Administered ATMP/CGTP drug products
12:30	***Lunch Break***
13:30	**Challenges in Demonstrating Product Comparability After Manufacturing Changes for ATMPs/CGTPs (90 min)** • Regulatory authority guidance in planning for ATMP/CGTP product comparability studies • Three risk-based considerations to reduce residual uncertainty of ATMP/CGTP product comparability • Industry experience in implementing product comparability after manufacturing process changes
15:00	***Coffee Break***
15:30	**Critical Manufacturing/Quality-Focused Meetings with Regulatory Authorities for ATMPs/CGTPs (90 min)** • CMC regulatory compliance deficiencies can seriously delay clinical development advancement • Meeting opportunities with regulatory authorities for ATMPs/CGTPs • Intensity of the regulatory authority review process for to obtain market approval for ATMPs/CGTPs
16:30	**Q&A**
17:00	***End of Training Course***