Practical Application of Phase-Appropriate GMP & Quality Principles to Clinical Development of ATMPs

Overview

There has been a surge in clinical development of Advanced Therapy Medicinal Products (ATMPs), also known as Cellular and Gene Therapy (CGT) Products in the USA. Many start up companies, hospitals, universities, and now even large biopharmaceutical companies, are planning or have already entered into manufacturing these genetically engineered virus and human cell products for clinical studies. While the ground rules for good manufacturing practices (GMPs) and quality of recombinant protein and monoclonal antibody manufacturing and control are well established, for ATMPs these are still under development.

ATMP manufacturing and control presents unique GMP and Quality challenges, such as the heightened concern about the safety and consistency of the starting material, the safety and quality of the raw materials added during processing, the need to protect against adventitious agent contamination during the entire manufacturing cycle, the limitations of the analytical methods available to characterize these virus and cell products, and the reality that the administered clinical medicinal product is a complex living organism.

Patients in these clinical development programs need to be protected by common sense GMPs and Quality Principles. The available regulatory authority guidance documents (both EMA and FDA) for ATMPs will be thoroughly examined. In addition, the core principles presented in PDA's 2016 Technical Report 56 'Application of Phase-Appropriate Quality System and cGMP to the Development of Therapeutic Protein Bulk Drug Substance (API)' will be adapted to ATMPs.

Who Should Attend:

This course is designed for those involved or interested in the manufacturing, quality and GMP compliance of ATMPs during clinical stages of development, including Senior Managers, Directors and Managers/Supervisors, QA/QC, Regulatory, Manufacturing and Process Development personnel.

Learning Objectives:

Upon completion of this course, the attendee will be familiar with:

- Explain the importance and underlying GMP and Quality principles for manufacturing, control and compliance of ATMPs during clinical stages of development
- Appreciate the GMP and Quality similarities and differences between protein medicinal products and ATMPs
- Appropriately apply phase-appropriate GMPs and Quality Principles to the clinical development of ATMPs

John Geigert, *PhD, RAC, President, BioPharmaceutical Quality Solutions*

John Geigert has been a consultant for the past 15 years, specializing in CMC regulatory strategy for the biopharmaceutical industry. He has held senior leadership positions in industry as Vice President of Quality for both IDEC Pharmaceuticals Corporation and Immunex Corporation. He has been a major participant in regulatory approvals for six biopharmaceutical products now commercially available in the U.S. and in Europe, and has over 40 years of experience in the biologic and biopharmaceutical industry. John obtained his B.S. in Chemistry from Washington State University, and his Ph.D. degree in Organic/Analytical Chemistry from Colorado State University. He has served as a member of the PDA the Board of Directors, and is currently the chair of the PDA Biopharmaceutical Advisory Board. He is US Regulatory Affairs Certified (RAC) by the Regulatory Affairs Certification Board (RACB).

Thursday, 6 June 2019 — 9:00 – 17:30

9:00 **Welcome & Introduction**

9:10 **Understanding the Basics**
- Painting the terminology landscape: ATMP, HCT/P, GMP, Quality System, etc.
- Introduction to the risk-based, phase-appropriate GMP and Quality approach

10:30 **Coffee Break**

11:00 **Major Differences, and the Regulatory Consequences**
- Viruses/cells are not biologic proteins
- GMP and Quality consequences of the differences between gene / cell-based medicines and protein-based medicines

12:30 **Lunch Break**

13:30 **Regulatory Authority (EMA/FDA) Expectations**
- Regulatory authority risk-based considerations to ATMP GMPs and Quality
- Regulatory authority guidances for a phase-appropriate approach to ATMP GMPs and Quality

15:00 **Coffee Break**

15:30 **Industry Practice in Applying Phase-Appropriate GMPs and Quality to ATMPs**
- Adapting PDA Technical Report 56 as a model for phase-appropriate ATMP GMPs and Quality
- Lesson learned from industry

17:30 **End of Training Course**