USP-APEC Center of Excellence for Advanced Therapies

Virtual Training Workshop on "Development and Validation of Bioassays for Advanced Therapies"

19, 20 January 2022 (Americas) / 20, 21 January 2022 (Asia)

Draft Program – *Updated 9 November 2021*

Background

For many years and still today, USP staff and external expert volunteers have collaborated to develop standards applicable to medicines, excipients and other common raw materials found in small molecule and biologic therapeutics. Almost two decades ago, USP began building its work on cell, gene and tissue engineered products. As a result, USP published a series of general chapters, documentary and physical Reference Standards, with support from Expert Committees and Panels. To support the standards setting activities and engage with stakeholders in this space, USP organized and delivered courses, and workshops on cells, tissues and gene therapies to bring together experts to debate and determine solutions to common problems. Recognizing that some of these concerns and the best approaches to mitigate their risks were not fully understood, USP staff have also provided dozens of talks around the world in leading advanced therapy conferences, as well as developing and delivering training webinars to build capability among developers and regulators.

Following the successful training in March 2021 on considerations for raw materials used to manufacture advanced therapy products, the students indicated that they wished to have future trainings focused on development and validation of methods which are commonly used to analyze and control critical quality attributes of these products. Since biological assays that measure activity or potency (known as bioassays) for these critical therapies can be one of the most challenging methods to develop and validate, USP in collaboration with the Regulatory Harmonization Steering Committee and Advanced Therapy Priority Work Area champions have built the attached virtual training program for regulators. USP has a rich history in development of standards to support bioassay measurements, development, validation, and analysis. Internal and external bioassay experts will deliver the training content.

Upon completion of this 6 hour training program, attendees will be able to:

1. Define the word "bioassay" and describe common bioassay formats for different types of advanced therapies
2. Describe standards that exist for bioassays and how to assess if the standard was suitably followed or variations sufficiently documented

3. Summarize why bioassays must be fit for purpose (e.g., for potency assignment, stability assessments, etc.)
4. Discuss how both science and risk play a role in determining the choice of a bioassay and why multiple formats may be beneficial during clinical development
5. Explain common assay parameters that are assessed and documented during bioassay development and validation
6. Describe common statistical approaches that support bioassay analysis
7. Discuss common bioassay lifecycle management challenges

The training will include pre-reads and pre-assessments for attendees, opportunities to ask questions of the instructors, and a post-assessment to ensure that the training was effective.

Draft Agenda

Day 1: – 3 hours

- **Introduction: Welcome and Objectives for the Program** **10'**
  - Fouad Atouf, Ph.D., Vice President, Global Biologics, USP

- **Welcome from Moderator for Day 1** **5'**
  - Judith Arcidiacono, M.S., US FDA, Center for Biologics Evaluation and Research, Office of Tissues and Advanced Therapies

- **What is a bioassay and how do we demonstrate it is fit for purpose?** 45'
  - Linda Engle, Ph.D., Biogen Principal Scientist, CMC Lead for Gene Therapy and Biosimilar Portfolio

- **Regulatory Perspectives on Best Practices and Guidances that Support Bioassays for Characterization and Release of Cell and Gene Therapy Products** 45'
  - Ramjay Vatsan, Ph.D., Team Lead, Gene Therapy Branch, Division of Cellular and Gene Therapies, Office of Tissues and Advanced Therapies, CBER, US FDA
  - *To include standards for regenerative medicine in support of harmonization- CBER perspectives*

- **Standards and Statistical Tools in Support of Bioassay Development, Validation, and Analysis** 60'
  - Steve Walfish, M.S., USP Senior Principal Scientist and lead for USP Statistics Expert Committee

- **Moderated Question and Answer Session with Audience and Speakers**

Day 2:

- **Welcome to Day 2 and Recap of Day 1** 15'
  - Maura Kibbey, Ph.D., Principal Scientific Fellow, USP Global Biologics; *Moderator for Day 2*

- **Regulatory Perspectives on Best Practices and Guidances that Support Bioassays for Characterization and Release of Cell and Gene Therapy Products** 45'
  - Yoji Sato, Ph.D., Head of the Division of Cell-based Therapeutic Products, Japan National Institute of Health Sciences, Japan
    - Overview of ICH Q2(R1)
    - Specific evaluation targets for quality, safety and effectiveness of cell and gene products
    - Introduction of test methods specific to cell therapy products including examples illustrating their validation

- **Industry Case Studies: Oncolytic Viruses and Gene Therapies** 45'
  - Chae-Ok Yun, Ph.D., CEO and CTO of GeneMedicine Co., Ltd. and Professor, Dept. of Bioengineering at Hanyang University, Seoul, Korea
    - Introduction to development of oncolytic viruses and their GMP manufacturing parameters
    - Development and validation of bioassays to assess virus potency

- **Industry Case Studies Illustrating Successful Bioassays** 60'
  - Max Tejada, Ph.D., Kite Pharma Executive Director

- **Closing Session Moderated by Dr. Kibbey**
  - Questions for the Speakers
  - Summarize the learnings
  - Identify future training opportunities and next steps