USP Welcome and Opening Remarks

Fouad Atouf, Ph.D.
Vice President–Science,
Global Biologics, USP

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Introduction and Objectives for Today's Biologics Stakeholder Forum

Edward Chess, Ph.D.
USP Biologics
Stakeholder Forum
Planning Committee Chair

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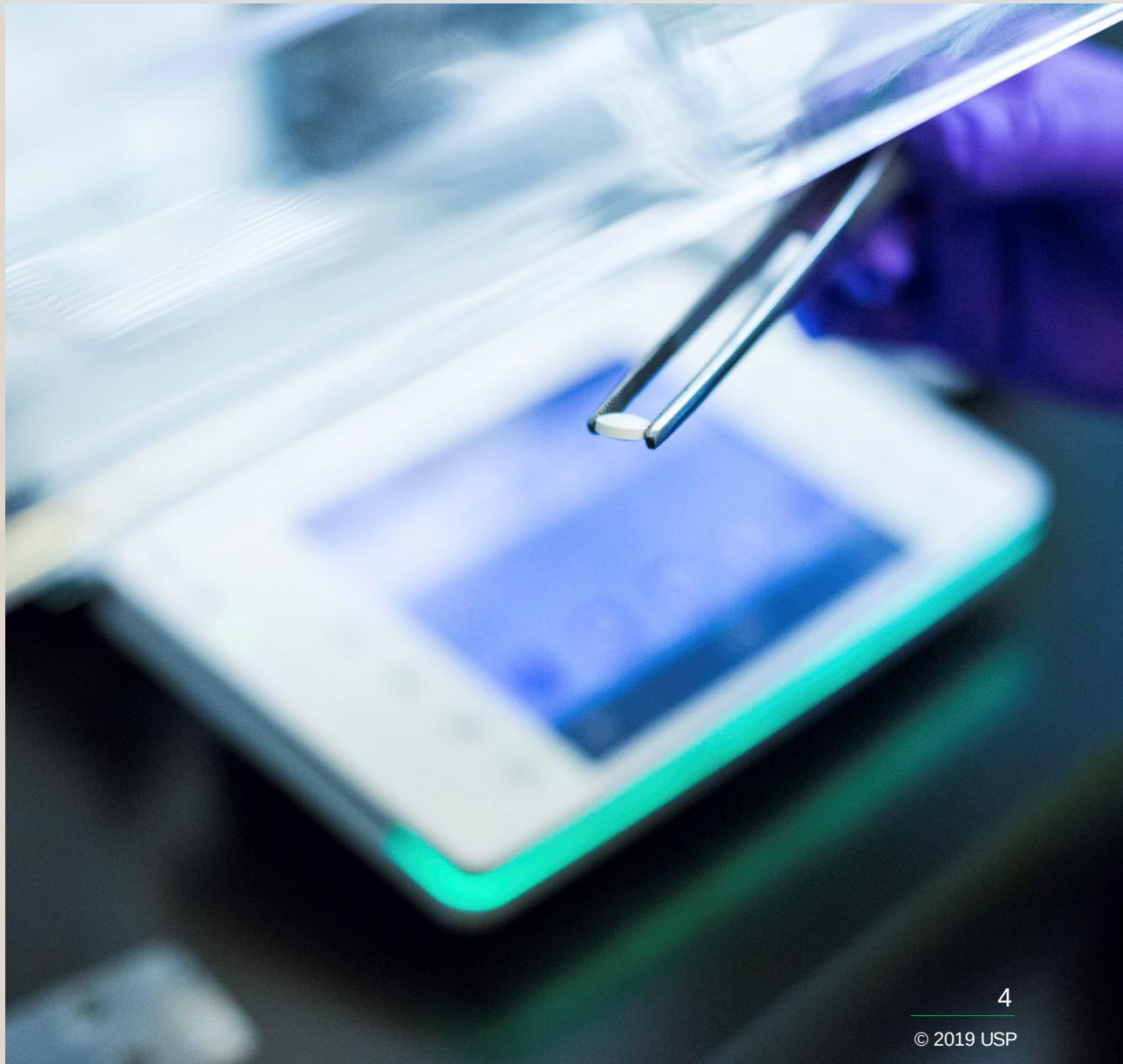
Biologics Stakeholder Forum (SF) charter

The goals of the Biologics Stakeholder Forum are:

1. To learn the needs of biologics stakeholders that may intersect with USP's core capabilities.
2. To share new information with the stakeholders and obtain feedback.
3. To recruit volunteers to support USP's Council of Experts.
4. To identify subject matter experts who can provide guidance or resources to support the execution of USP's biologics strategy.

2024 Biologics SF planning committee

- Ed Chess, Chair (Consultant, USA)
- Linda Narhi (Consultant, USA) Vice-Chair
- KS Lim (Metagenomi, USA) Vice-Chair
- Gael Debauve (UCB, Belgium)
- Mike Havert (CBMG, USA)
- Ben Clarke (USP)
- Bruno De Carvalho (USP)



Multi Attribute Methods for Biologics

Topics & Speakers

- **Development and Application of a Multi-Attribute Method (MAM)**
  - Jette Wypych, Ph.D., Amgen
- **Enhancing Biotherapeutic Process and Product Knowledge with the Multi-Attribute Method (MAM)**
  - Andrew Dawdy, Ph.D., Pfizer
- **Quality Considerations for the Multi-Attribute method (MAM)**
  - Sarah Rogstad, Ph.D., FDA
- **USP Standard to Support Multi-Attribute Methods (MAM) and Mass Spectrometry**
  - Diane McCarthy, Ph.D., USP

First Biologics SF, Jan 10, 2020

Discussion and Outcomes

- Breakout sessions
  - Best practices
  - Opportunities for standards
- Recommendations
  - There is a need for best practices for MAM and attendees supported developing a general chapter on best practices for MAM
  - Physical standards will be useful, with particular interest in matched sets of intact and pre-digested mAbs
- Outcome
  - MAM Expert Panel has published a general chapter (<1060>) on best practices
  - USP conducted method assessment using 3 USP mAbs and initiated development of matched pre-digested mAbs
  - Cooperative agreement with FDA under BsUFA Pilot Research Program on comparison of MAM vs Conventional Methods*

*Acknowledgement of Federal Support:: This project is supported by the Food and Drug Administration (FDA) of the U.S. Department of Health and Human Services (HHS) as part of a financial assistance award FAIN# U01FD007762 totaling $1,530,721 with 100 percent funded by FDA/HHS. The contents are those of the author(s) and do not necessarily represent the official views of, nor an endorsement, by FDA/HHS or the U.S. Government.

Second Biologics SF, Aug 10 & 12, 2021

Innovative Analytical and Digital Solutions to Advance Biomanufacturing and Product Quality

Topics & Speakers

Day 1 Analytical Day

- **CHO Cell Process Monitoring of Quality Attributes Using Raman Spectroscopy at Manufacturing Scale**
  - Caitlin O'Mahony Hartnett, Ph.D., Scientist, Janssen Sciences Ireland
- **In-line Viral Inactivation: Monitoring Key Process Parameters to Ensure Effectiveness**
  - Michael Phillips, Ph.D., Director-Bioprocess Development, MilliporeSigma
- **Enabling Real Time Release Testing (RTRT) with Multi-Attribute Methods**
  - Galahad Deperalta, Ph.D., Senior Scientist and Innovation Group Leader, Analytical Development and Quality Control, Genentech

Innovative Analytical and Digital Solutions to Advance Biomanufacturing and Product Quality

Topics & Speakers

Day 2 Digital Day

- **How can in silico Models be Accepted in Lieu of Lab Experiments and Manufacturing Runs?**
  - Marcus Fiadeiro, Associate Director, Purification Development, Sanofi
    Siddhartha Jain, Ph.D., Director, MSAT Digital, Sanofi

- **The Potential of Hybrid Process Modeling and Digital Twins to Master the Goals of Industry 4.0 in Bioprocessing**
  - Michael Sokolov, Ph.D., COO and Co-Founder of DataHow AG

- **Advanced Technology for Manufacturing Process Control**
  - Riley Myers, Ph.D., Emerging Technology Team/OPQ and Lead Biologist, Office of Biotechnology Products, CDER, US FDA

Second Biologics SF, Aug 10 & 12, 2021

Discussion and Outcomes

- Breakout sessions
  - Best practices??
  - Opportunities for standards??
- Recommendations
  - USP to focus on analytical solutions because digital solutions are not yet mature enough for standardization
  - Host a roundtable on PAT for biomanufacturing
- Outcome
  - USP Roundtable on Implementation of At-Line and In-Line Analytical Tools for Biomanufacturing Process Development and Monitoring, Feb 24, 2022
  - Expert Panel initiated to draft a General Chapter on At-line/ In-line Analytics

Third Biologics SF, Oct 26, 2022

Collaborating to Solve CMC Challenges and Support Efficient Development of Lentiviral-Mediated CAR T Cell Therapies

Topics & Speakers

- **USP Standards to Support the Development of Lentiviral-mediated CAR T Cell Therapies**
  - Ben Clarke, Ph.D., Senior Scientist II, Global Biologics, USP
- **Regulatory Considerations for the Development of Potency Assays During CAR T Cell Development**
  - Andrew Timmons, Ph.D., Biologist, U.S. Food and Drug Agency (FDA)
- **Approaches to Potency Testing for Chimeric Antigen Receptor T Cells**
  - Shree Joshi, Senior Scientist, BioTD, Analytical Development, Janssen Pharmaceutical
- **Implementation of QbD Principles in Potency Assay Development and Overcoming Challenges on the Road to Commercialization**
  - Kim Nguyen, Ph.D., Head of Product Attribute Sciences, Kite Pharma

Third Biologics SF, Oct 26, 2022

Discussion and Outcomes

- Breakout sessions
  - Q&A for Speakers
  - Biological activity and potency
  - Safety (replication-competent virus, integration, etc.)
- Recommendations
  - USP should focus on broadly applicable physical standards for lentiviral titration, process impurity quantification, and raw material qualification.
  - Develop best practice recommendations for industry
- Outcome
  - Lentiviral Vector Expert Panel was formed to draft a best practice chapter.
  - Standards in development to support assessment of copy number, site integration and lentivirus titration

USP Biologics Stakeholder Forum

NOTICE TO PARTICIPANTS:

- During the main meeting virtual attendees will be muted. Please ask questions or make comments at any time by using the **CHAT f**eature.
- On-line questions will be collated for the Q&A that is a part of the breakout discussions.
- Questions may be initially posed for one speaker, but other speakers are also free to join in and provide answers
- Any unanswered questions will be answered after the event.
- Today's event will be recorded strictly for note taking purposes and the recording destroyed afterwards.
- You will receive a satisfaction survey after today's event. Please send us your input!

Questions VIA the CHAT Feature:

- Click on the CHAT icon at the bottom of the screen.
- All questions/comments should be sent to "everyone".

Questions during the breakouts:

- You will be able to unmute to ask questions during the breakouts or you can continue to use the CHAT function as before. We also encourage you to turn on your video for a more interactive experience.
- Please click on the REACTIONS button at the bottom of your screen and select "raise hand" to let the moderator know you would like to speak. Please wait to unmute your microphone until the moderator calls on you.

Fourth Biologics SF, Feb 22, 2024

Innovative Analytical Approaches to Cell and Gene Therapy

NGS and Other Methods

8:00 a.m. – 8:30 a.m.	**Registration and Check-in**
8:30 a.m. – 8:35 a.m.	**USP Welcome and Opening Remarks** Speaker: Fouad Atouf, Ph.D., Vice President, Global Biologics, USP
Session 1:	**Innovations to Overcome Limitations in an Evolving Regulatory Landscape**
8:35 a.m. – 8:50 a.m.	**Introduction and Objectives for Today's Biologics Stakeholder Forum** Speaker: Edward Chess, Ph.D., USP Biologics Stakeholder Forum Planning Committee, Chair
8:50 a.m. – 9:05 a.m.	**USP Standards to Support Gene Therapy** Speaker: Ben Clarke, Senior Scientist II, Global Biologics, USP
9:05 a.m. – 9:30 a.m.	**Minimizing the Impact of Stability Studies on Gene Therapy Batch Yield** Speaker: Gaël Debauve, Head of Gene Therapeutics, CMC Analytics, UCB Pharma S.A.

Fourth Biologics SF, Feb 22, 2024

Innovative Analytical Approaches to Cell and Gene Therapy

NGS and Other Methods

09:30 a.m. – 09:55 a.m.	**EDQM/Ph. Eur. Activities in the Field of NGS/HTS** Speaker: Gwenael Cirefice, Scientific Officer, EDQM, European Pharmacopoiea
09:55 a.m. – 10:10 a.m.	**Break**
10:10 a.m. – 10:35 a.m.	**FDA Perspectives on NGS in Adventitious Virus Testing** Speaker: Arifa Khan, Senior Investigator, Center for Biologics Evaluation and Research, US FDA
10:35 a.m. – 11:20 a.m.	**Moderated Breakout Discussion** Kok-Seong (KS) Lim, USP Biologics Stakeholder Forum Planning Committee, Vice-Chair
11:20 a.m. – 12:20 p.m.	**Lunch and networking break for in-person attendees**

Fourth Biologics SF, Feb 22, 2024

Innovative Analytical Approaches to Cell and Gene Therapy

NGS and Other Methods

Session 2:	**NGS: improvement, replacement, or new standard approach?**
12:20 p.m. – 12:25 p.m.	**Introduction and Objectives for Session 2** Edward Chess, USP Biologics Stakeholder Forum Planning Committee, Chair
12:25 p.m. – 12:50 p.m.	**NGS Transcriptome Analysis in Cell Banking** Speaker: Colette Cote, Chief Scientific and Portfolio Oficer, US General Manager, Pathoquest
12:50 p.m. – 01:15 p.m.	**Off-Target Analysis: Identification, Verification and Compliant Testing** Speaker: Aaron ZC, CTO, GeneGoCell
01:15 p.m. – 01:30 p.m.	**Break**

Fourth Biologics SF, Feb 22, 2024

Innovative Analytical Approaches to Cell and Gene Therapy

NGS and Other Methods

01:30 p.m. – 01:55 p.m.	**Recommendations for the Validation of rAAV Identity by Next Generation Sequencing** Speaker: Jarrod Dean, Associate Director, Sanofi, Genomic Medicine Unit BioAnalytics, NGS CMC
01:55 p.m. – 02:50 p.m.	**Moderated Breakout Discussion** Michael Jesudoss, Senior Scientist, NGS, BioMarin Pharmaceutical Inc
02:50 p.m. – 03:00 p.m.	**Next Steps and Closing Remarks** Edward Chess, USP Biologics Stakeholder Forum Planning Committee, Chair
03:00 p.m.	**Adjourn**

Your moderators

- Kok Seong Lim, Ph.D., Metagenomi
- Michael Jesudoss, Ph.D., BioMarin Pharmaceuticals Inc.

Thank You

Empowering a healthy tomorrow