April 9-11, 2019
Hilton Washington DC/Rockville Hotel and Executive Meeting Center
1750 Rockville Pike, Rockville, MD 20852 USA

To Register, see: www.signmeup.com/126943.
Visit the PQRI website for more details: http://pqri.org/4th-fda-pqri-conference/

Draft Program as of March 15, 2019 – Presentation Titles and Speakers subject to change

USE THIS COLOR GUIDE AS A REFERENCE:

TRACK #1: BIOPHARMACEUTICS: NOVEL APPROACHES TO IMPROVE TREATMENT OUTCOME AND PATIENT SAFETY
TRACK #2: DEVELOPMENT: EMERGING TECHNOLOGIES AND PATIENT CENTRICITY IN EARLY DRUG DEVELOPMENT
TRACK #3: MANUFACTURING: NOVEL MANUFACTURING TECHNOLOGIES AND CHALLENGES FOR THE PRODUCTION OF PATIENT-CENTRIC DRUG PRODUCTS
# Conference At-A-Glance

**Draft Program as of March 15, 2019**

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## Day 1 – Tuesday, April 9, 2019

### 7:30 – 8:30 AM Registration

### 8:30 – 10:15 AM Plenary Session

- **8:30 – 8:45 AM** Welcome - Mehran Yazdanian, Ph.D., Teva Pharmaceuticals
- **8:45 – 9:15 AM** Keynote - Patrizia Cavazzoni, MD, Deputy Director for Operations, CDER, US FDA (Invited)
- **9:15 – 9:45 AM** Innovating to Accelerate the Delivery of Transformative Therapies to Patients – Stephanie Krogmeier, Ph.D., Vertex Pharmaceuticals, Inc.

### 10:15 -10:45 AM Coffee Break

### 10:45 AM – 12:30 PM Session 1: Complex Generics – Challenges and Opportunities

**Moderator:** Wenlei Jiang, FDA

- **Considerations for Biologics and Non-biological Complex Drugs**
  - Daan Crommelin, Utrecht University
- **Early Drug Development, Regulatory Perspective**
  - Ramesh Sood, FDA
- **Manufacturing and Validation Challenges**
  - Palani Palaniappan, Sarepta

### 11:15 AM – 12:15 PM Session 1: Complex Generics – Regulatory Perspective

**Moderator:** Jeff Jiang, FDA

- **An Overview of Complex Drug Substances and Complex Formulations – A Quality Perspective**
  - Katherine Tyner, FDA
- **Accelerating Drug Product Development Using Small Scale, Data Intensive, Iterative Design Approaches**
  - Gregory Troup, Merck & Co., Inc.
- **CMC Considerations for Cell and Gene Therapies**
  - Michael Havert, Bluebird Bio (Invited)

### 12:15 – 1:15 PM Session 1: Complex Generics – Challenges and Opportunities with Patient-Centric Dosage Form Design: Industry Perspectives

**Moderator:** Matthew Burke, GlaxoSmithKline

- **Challenges and Opportunities with Patient-Centric Dosage Form Design: Industry Perspectives**
  - Matthew Burke, GlaxoSmithKline

### 12:30 – 1:30 PM Lunch

### 12:30 – 1:30 PM Panel Discussion (above speakers)

## Track #1 Novel Approaches to Improve Treatment Outcome and Patient Safety

### 10:45 AM – 12:30 PM

**SESSION 1: COMPLEX GENERICS – CHALLENGES AND OPPORTUNITIES**

**Moderator:** Wenlei Jiang, FDA

- Considerations for Biologics and Non-biological Complex Drugs
  - Daan Crommelin, Utrecht University

### 11:15 AM - 12:15 PM

- An Overview of Complex Drug Substances and Complex Formulations – A Quality Perspective
  - Katherine Tyner, FDA

### 12:15 – 1:15 PM

- Overview of Complex Generics – Regulatory Perspective
  - Jeff Jiang, FDA

### 12:30 – 1:30 PM

Panel Discussion (above speakers)

## Track #2 Emerging Technologies and Patient Centricity in Early Drug Development

### 10:45 AM – 12:30 PM

**SESSION 1: EARLY DRUG DEVELOPMENT: A VISION FOR THE FUTURE**

**Moderator:** Geoffrey Wu, FDA

- Early Drug Development, Regulatory Perspective
  - Ramesh Sood, FDA

### 11:15 AM - 12:15 PM

- Accelerating Drug Product Development Using Small Scale, Data Intensive, Iterative Design Approaches
  - Gregory Troup, Merck & Co., Inc.

### 12:15 – 1:15 PM

- Challenges and Opportunities with Patient-Centric Dosage Form Design: Industry Perspectives
  - Matthew Burke, GlaxoSmithKline

### 12:30 – 1:30 PM

Panel Discussion (above speakers)

## Track #3 Novel Manufacturing Technologies and Challenges for the Production of Patient-Centric Drug Products

### 10:45 AM – 12:30 PM

**SESSION 1: NOVEL MANUFACTURING TECHNOLOGIES AND CHALLENGES FOR CELL AND GENE THERAPIES**

**Moderator:** Michael Skidmore, Pharmaceutical Quality Consulting, Inc.

- Manufacturing and Validation Challenges
  - Palani Palaniappan, Sarepta

### 11:15 AM - 12:15 PM

- CMC Considerations for Cell and Gene Therapies
  - Michael Havert, Bluebird Bio (Invited)

### 12:15 – 1:15 PM

- Regulatory Expectations for Cell and Gene Therapies
  - Ramjay Vatsan, FDA

### 12:30 – 1:30 PM

Panel Discussion (above speakers)

### 12:30 – 1:30 PM

Panel Discussion (above speakers)
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<th>Time</th>
<th>Track 1: Novel Approaches to Improve Treatment Outcome and Patient Safety</th>
<th>Track 2: Emerging Technologies and Patient Centricity in Early Drug Development</th>
<th>Track 3: Novel Manufacturing Technologies and Challenges for the Production of Patient-Centric Drug Products</th>
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<tr>
<td>2:00 – 2:30 PM</td>
<td>Challenges and Considerations in the Development and Validation of In Vitro Drug Release Testing for Intravaginal Rings ○ Karl Malcolm, Queen’s University Belfast</td>
<td>Discovering and Developing Non-Traditional Drug Modality Molecules with Optimal Pharmaceutical Properties ○ Mike Hageman, University of Kansas</td>
<td>Use of Computational Modeling in Specification Setting and Establishing Control Strategy – Regulatory Perspective ○ Thomas O’Connor, FDA</td>
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<tr>
<td>2:30 – 3:00 PM</td>
<td>FDA Perspective on Non-oral Delivery Biopharmaceuticals Aspects ○ Wenlei Jiang, FDA</td>
<td>Designing for Delivery: The Use of Mathematical Modeling ○ Ronald Iacocca, Eli Lilly and Company</td>
<td>PAT for Model Based Design, Optimization, Monitoring and Control of Continuous Manufacturing ○ Thomas De Beer, Ghent University, Belgium</td>
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<td>3:00 – 3:15 PM</td>
<td>Panel Discussion (above speakers)</td>
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<td>3:15 – 3:45 PM</td>
<td>Coffee Break</td>
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<td>4:15 – 4:45 PM</td>
<td>In Vitro Release and Q3 Measurements for Semisolid Drug Products ○ Flavian Rădulescu, Carol Davila University of Medicine and Pharmacy</td>
<td>Emerging Drug-Device Combinations: A Digitally Enhanced Patient Experience ○ Kristina Lauritsen, FDA</td>
<td>Continuous Manufacturing – Small Molecule Drug Substance or Drug Product ○ Paul Collins, Eli Lilly and Company</td>
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<tr>
<td>5:15 – 5:30 PM</td>
<td>Panel Discussion (above speakers)</td>
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<tr>
<td>5:30 – 7:00 PM</td>
<td>Reception</td>
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### DAY 2 – WEDNESDAY, APRIL 10, 2019

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<td>Continental Breakfast</td>
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<td>8:00 – 10:00 AM</td>
<td>Plenary Session</td>
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<td>• Topic Summaries from Day 1 (40 minutes per Track; 10 minutes per Topic)</td>
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<td>10:00 AM – 10:30 AM</td>
<td>Coffee Break –</td>
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<tr>
<td>10:30 AM– 12:15 PM</td>
<td>Track #1 Novel Approaches to Improve Treatment Outcome and Patient Safety</td>
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<tr>
<td>10:30 AM– 12:15 PM</td>
<td>Session 4: Predictive Approaches to Gain Insight into the Clinical Performance of Inhaled Medicines</td>
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<tr>
<td>11:00 AM – 12:00 PM</td>
<td>Track #2 Emerging Technologies and Patient Centricity in Early Drug Development</td>
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<tr>
<td>11:00 AM – 12:00 PM</td>
<td>Session 4: Development Considerations for Evolving Non-Traditional Drug Modalities</td>
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<tr>
<td>12:00 – 12:15 PM</td>
<td>Track #3 Novel Manufacturing Technologies and Challenges for the Production of Patient-Centric Drug Products</td>
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<tr>
<td>12:00 – 12:15 PM</td>
<td>Session 4: Regulatory Submission Lifecycle Management</td>
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<tr>
<td>12:15 – 1:15 PM</td>
<td>Lunch</td>
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<td>Time</td>
<td>Track 1: Novel Approaches to Improve Treatment Outcome and Patient Safety</td>
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| 1:15 – 3:00 PM | **SESSION 5: Enabling Patient-Focused Quality Standards via Modeling and Simulation for Oral Products**  
Moderator: Sandra Suarez Sharp, FDA | **SESSION 5: New Visualization and Analysis Techniques in Drug Development**  
Moderator: Bob Meyer, Merck & Co., Inc. | **SESSION 5: Challenges with Drug Device Combination Products**  
Post Approval  
Moderator: Susan Neadle, Johnson & Johnson |
| 1:15 – 1:45 PM | • PBPK-based and Traditional IVIVC as Complementary Tools to Quality by Design in the Biopharmaceutics Space  
  o David Good, Bristol-Myers Squibb | • What do Petroleomics, Jet Fuel and Pharmaceuticals Have in Common? Visualization and Characterization of Complex Mixtures of Extractables/Leachables and Other Pharmacologically Relevant Compounds using High Resolution 2-D and 3-D Mass Mapping  
  o Douglas Kiehl, Eli Lilly and Company | • Types and Handling of Product Complaints for Combination Products  
  o John Towns, Eli Lilly and Company |
| 1:45 – 2:15 PM | • The US Food and Drug Administration Perspective on Physiologically-Based Absorption Modeling in Biopharmaceutics  
  o Yang Zhao, FDA | • Advanced Analytical Techniques for Characterizing Amorphous Solid Dispersions  
  o Eric Munson, Purdue University | • Regulatory Perspective on Applying Human Factors Engineering Considerations to Post Approval Changes  
  o QuynhNu Nguyen, FDA |
| 2:15 – 2:45 PM | • Mechanistic Absorption Modeling and Clinically Relevant Specifications for Enabling Formulations Technologies  
  o Christophe Tistaert, Janssen Research & Development | • Beyond the Big Crunch of Excel: The Big Bang of Digital Visualizations  
  o Marcus Adams, Merck & Co., Inc. | • Challenges based on Differences in Global Regulatory Filing Requirements  
  o Doug Mead, Janssen |
| 2:45 – 3:00 PM | Panel Discussion (above speakers) | Panel Discussion (above speakers) | Panel Discussion (above speakers) |
| 3:00 – 3:30 PM | Coffee Break | | |
| 3:30 – 5:15 PM | **SESSION 6: Oral Biopharmaceutics: Challenges, Opportunities, and Advancements**  
Moderator: Andreas Abend, Merck & Co., Inc. | **SESSION 6: Emerging Technologies for Improving Patient Adherence**  
Moderator: Dave Schoneker, Colorcon | **SESSION 6: CMC Innovation in the 21st Century – Global Regulatory Perspectives**  
Moderator: Nina Cauchon, Amgen |
| 3:30 – 4:00 PM | • Advancing the Dissolution Toolbox in Drug Development: Novel Bio-predictive Dissolution Methodologies for Oral Products  
  o Greg Amidon, University of Michigan | • Challenges in the Opioid Epidemic Crisis  
  o Douglas Throckmorton, FDA | • Regulatory Framework and Industrial Initiatives – A European Perspective  
  o Sven Stegemann, Graz University of Technology |
| 4:00 – 4:30 PM | • Use of 3D-printed Tablets as a Biopharmaceutics Investigation Tool  
  o Adam Procopio, Merck & Co., Inc. | • The Expanding Universe of Patient Adherence Solutions: Long-acting Implantables, Micro-Chip, Smart Packaging, Apps, and Social Robotics  
  o Stephanie Barrett, Merck & Co., Inc. | • Pharmaceuticals and Medical Devices Agency (PMDA) Perspective  
  o Yoshihiro Matsuda, PMDA |
| 4:30 – 5:00 PM | • Advancing Biopharmaceutics Knowledge and Toolkit to Improve the Quality of Pediatrics Medicines  
  o Gilbert Burkart, FDA | • New Formulation Technologies for Patient Adherence: Solid Oral Dosage Forms  
  o Ali Rajabi-Siahboomi, Colorcon | • FDA Perspective  
  o Celia Cruz, FDA |
| 5:00 – 5:15 PM | Panel Discussion (above speakers) | Panel Discussion (above speakers) | Panel Discussion (above speakers) |
DAY 3 – THURSDAY, APRIL 11, 2019

7:30 – 8:00 AM Continental Breakfast

8:00 – 9:30 AM Topic Summaries (30 minutes per Track; 10 minutes per Topic)
- 8:00 – 8:30 AM  Track #1 Summary
- 8:30 – 9:00 AM  Track #2 Summary
- 9:00 – 9:30 AM  Track #3 Summary

9:30 – 10:00 AM Coffee Break

10:00 – 11:30 AM Introducing FDA’S New Initiative: KASA (Knowledge-aided Assessment and Structured Application)
A creative regulatory approach for modernizing the quality assessment and enhancing submission format
Moderator: Lawrence Yu
Presenters: Susan Rosencrance, Andre Raw, Derek Smith, and Larisa Wu
Panelists: Susan Rosencrance, Sharmista Chatterjee, Mahesh Ramanadham, Paul Seo, Mary Ann Slack, Ramesh Sood, Geoffrey Wu

11:30 AM – 12:00 PM Poster Award Announcement and Presentations

12:00 PM Closing Remarks