DIA

Advanced Therapies: Innovations in CMC Conference

November 8-10 | Virtual

PROGRAM CHAIR

Kirsten Messmer, PhD, RAC
Senior Research Analyst
POLITICO's Agency IQ

PROGRAM COMMITTEE

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Associate Principal Scientist
Merck & Co., Inc.

Judy Chou, PhD
President and CEO
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Research Scientist, Global Regulatory Affairs CMC Biotechnology
Eli Lilly and Company

Cynthia Schnedar, JD
Principal, Regulatory Compliance
Greenleaf Health, Inc.

Overview

Innovations in the development of advanced therapies have yielded new and dynamic solutions for the prevention and treatment of diseases where none existed previously. However, compared to traditional small molecules, advanced therapies present unique CMC challenges that may complicate development. Effective CMC strategies are crucial to the successful development and lifecycle management of these products. Leveraging advances in technology as well as accelerations in development due to the pandemic will enable us to bring even more products to market.

The DIA *Advanced Therapies: Innovations in CMC* conference will explore common CMC challenges posed by the development of these increasingly complex products. The use of multi-stakeholder perspectives and relevant case studies will provide solutions to enable regulatory compliance, shorter review timelines, and support post-approval maintenance.

Highlights

Keynote Address Speakers: Peter Marks, MD, PhD, Director, Center for Biologics Evaluation and Research, FDA

Who Should Attend

Senior-level professionals and those working in the following areas of oligonucleotide science:

- CMC Regulatory Affairs
- CMC Writing
- Quality Assurance/Quality Control
- Regulatory Compliance
- Development and Manufacturing
- CMC Project Management
- Rare, Orphan Diseases
- CMC Lifecycle Management
- Biotechnology

Schedule At-A-Glance

Sessions held in ET

DAY ONE | MONDAY, NOVEMBER 8

10:00-11:00AM	**Opening Remarks and Keynote Address**
11:00-11:15AM	Break/Visit the Virtual Exhibit Hall
11:15AM-12:15PM	**Session 1:** Regulatory Framework and GMP Requirements for ATMPs
12:15-12:30PM	Break/Visit the Virtual Exhibit Hall
12:30-1:45PM	**Session 2:** Bridging the Gap for CMC Assessments Comparability Through Risk-based Control Strategies – Part 1
1:45-2:15PM	Break/Visit the Virtual Exhibit Hall
2:15-3:30PM	**Session 3:** Bridging the Gap for CMC Assessments Comparability Through Risk-based Control Strategies – Part 2

DAY TWO | TUESDAY, NOVEMBER 9

10:00-11:30AM	**Session 4:** Starting and Raw Materials in Cell and Gene Therapy
11:30AM-11:45AM	Break/Visit the Virtual Exhibit Hall
11:45AM-12:45PM	**Session 5a:** Case Studies in Gene Therapy Development
12:45-1:15PM	Break/Visit the Virtual Exhibit Hall
1:15-2:15PM	**Session 5b:** Case Studies on Challenges for Gene Editing Technology
2:15-2:45PM	Break/Visit the Virtual Exhibit Hall
2:45-3:45PM	**Session 5c:** Case Studies on Challenges for Cell-based Therapeutics

DAY THREE | WEDNESDAY, NOVEMBER 10

10:00-11:00AM	**Session 6:** Utilizing Regulatory Agency Emerging Technology Teams During Product Development
11:00-11:15AM	Break/Visit the Virtual Exhibit Hall
11:15AM-12:30PM	**Session 7:** Lessons Learned From Development of Vaccines and Therapeutics in Response to COVID-19
12:30-1:00 PM	Break/Visit the Virtual Exhibit Hall
1:00-2:30PM	**Session 8:** Fireside Chat: Regulatory Approaches to Emerging Technologies and Modalities and Closing Remarks
2:30PM	Conference Adjourns

Learning Objectives

At the conclusion of this conference, participants should be able to:

- Analyze the latest delivery strategies for clinical use of oligonucleotide therapies
- Discuss the non-clinical toxicology profile of oligonucleotide therapies and the latest improvements in predicting effects in humans
- Describe the chemistry, manufacturing, and controls challenges associated with the development of oligonucleotides, including formulation and specification issues
- Explain the latest global regulatory updates in oligonucleotide therapeutic developments
- Evaluate the best mechanism of interactions with patients and partner organizations to address critical needs in the rare disease community

DAY ONE | MONDAY, NOVEMBER 8

10:00-11:00AM

Opening Remarks and Keynote Address

Session Chair
Kirsten Messmer, PhD, RAC, Senior Research Analyst, POLITICO's Agency IQ

Speaker
Peter Marks, MD, PhD, Director, Center for Biologics Evaluation and Research, FDA

11:00-11:15AM

Break/Visit the Virtual Exhibit Hall

11:15AM-12:15PM

Session 1: Regulatory Framework and GMP Requirements for ATMPs

Session Chair
Eliana Clark, Senior Vice President Technical Operations and Quality, Intellia Therapeutics, Inc.

Cell and gene therapy products and other advanced-therapy medicinal products (ATMPs) are different from biologics and small molecules due to their inherent complexity, with many novel manufacturing processes, use of complex starting materials, multiple banking requirements, and complex analytical methods. The regulatory framework and classifications are different between regions, as are GMP requirements for manufacturing of ATMPs. This session will cover case studies in regulatory and manufacturing approaches for gene therapy, gene editing, and autologous and allogeneic cell therapies.

At the conclusion of this session, participants should be able to:

- Understand regulatory CMC requirements for ATMPs, including Drug Substance, Drug Product and Starting Material definitions
- Understand phase appropriate GMP requirements for ATMPs in different regions
- Discuss opportunities for harmonization of CMC requirements for ATMPs

Speakers
Steven Oh, PhD, Deputy Director, Division of Cellular and Gene Therapies, CBER, FDA

Alexandra Beumer Sassi, PharmD, PhD, RAC, Senior Director CMC and Quality, Voisin Consulting Life Sciences

Dana Alexander, Senior Vice President, Technical Operations, Allovir

12:15-12:30PM

Break/Visit the Virtual Exhibit Hall

12:30-1:45PM

Session 2: Bridging the Gap for CMC Assessments Comparability Through Risk-based Control Strategies – Part 1

Session Co-Chairs
Mia Kiistala, Owner and Principal Consultant, Aurora CMC Consulting, Finland

Anthony Bevivino, PhD, Associate Principal Scientist, Merck & Co., Inc.

Cortney Lawrence, PhD, Research Scientist, Global Regulatory Affairs CMC Biotechnology, Eli Lilly and Company

Control strategies are developed based on product data, prior knowledge, and risk assessments, and ensure delivery of safe and efficacious product throughout development and the commercial life of the product. For ATMPs development of risk-based control strategies may be beneficial, where relevant information from clinical and non-clinical domains in addition to CMC information is used to define the boundaries for CQAs. Incorporation of product characterization, bioassay, assay matrixing, non-clinical models, biomarkers, surrogate model and structure activity characterization as early in development as possible can all further elucidate criticality of CQAs for risk-based control strategies. Development of such risk-based control strategies can be used to set specifications for both release and stability, identify critical in-process controls and also improve readiness to support comparability studies evaluating CMC changes. New approaches and case studies supportive of risk-based control strategies are discussed in this session.

At the conclusion of this session, participants should be able to:

- Understand the benefits of risk-based control strategies for ATMPs
- Discuss some examples of new risk-based approaches that strengthen risk-based control strategy
- Understand phase-appropriate implementation of control strategies throughout clinical development

Control Strategy
Paula Salmikangas, PhD, Director of Biopharmaceuticals and ATMPs, NDA Advisory Board, United Kingdom

Comparability
Carmen Warren, PhD, Senior Research Scientist, Product Attribute Sciences, Kite Pharmaceuticals, a Gilead Company

Bioassay
Tessie McNeely, PhD, Principal Scientist, Cell Based Sciences, Merck & Co., Inc.

1:45-2:15PM — **Break/Visit the Virtual Exhibit Hall**

2:15-3:30PM — **Session 3:** Bridging the Gap for CMC Assessments Comparability Through Risk-based Control Strategies – Part 2

Session Co-Chairs
Mia Kiistala, Owner and Principal Consultant, Aurora CMC Consulting, Finland

Anthony Bevivino, PhD, Associate Principal Scientist, Merck & Co., Inc.

Cortney Lawrence, PhD, Consultant Scientist, Global Regulatory Affairs CMC Biotechnology, Eli Lilly and Company

Continue the conversation on this topic with three additional experts.

CMC Strategy
Linda Engle, PhD, Principal Scientist, Technical Development/Associate Director, CMC Gene Therapy, Biogen

Lessons Learned from Comparability
Reena Patel, PhD, Associate Director, Analytical Development – Cell & Gene Therapy, Vaccines, Janssen Pharmaceuticals

Rapid Microbiological Methods
Bernice Westrek-Esselink, Associate Director, Operations, COE Microbiology, MSD Netherlands

DAY TWO | TUESDAY, NOVEMBER 9

10:00-11:30AM — **Session 4:** Starting and Raw Materials in Cell and Gene Therapy

Session Chair
Sofia Håkansson Buch, Vice President for Stem Cell CMC & Manufacturing, Novo Nordisk A/S, Denmark

Starting- and raw materials are an important part of the cell and gene therapy products and can be highly complex in nature. This session will focus on gene edited cells as starting material, approaches to selection and qualification of raw materials and differences in regulations from different countries.

At the conclusion of this session, participants should be able to:

- Have an increased understanding of cells as starting material that are gene edited
- Have increased understanding of approaches to raw materials
- Have a general overview regulatory landscape for raw materials

Overview of Regulatory Landscape for Raw Materials
Benjamin Fryer, PhD, CEO, Pluristyx, Inc.

A Risk Based Approach to Raw Material Applications Throughout Clinical Development of Advanced Therapies
Sara Mills, Senior Consultant, Dark Horse Consulting Group, Inc.

The Intricacies of Cell Therapy's Starting Materials
Andrew Ramelmeier, PhD, Executive Vice President, Technical Operations, Sangamo Therapeutics

11:30-11:45AM — **Break/Visit the Virtual Exhibit Hall**

11:45AM-12:45PM — **Session 5a:** Case Studies in Gene Therapy Development

Session Co-Chairs
Eliana Clark, Senior Vice President Technical Operations and Quality, Intellia Therapeutics, Inc.

Kirsten Messmer, PhD, RAC, Senior Research Analyst, POLITICO's Agency IQ

Gene therapies rely on new and innovative technologies aiming to modify, replace, add a missing or malfunctioning gene to treat human disease. Gene therapy products face very product specific CMC challenges due to the novelty and variety of technology employed. This session will discuss case studies for 2-3 different gene therapy technologies.

At the conclusion of this session, participants should be able to:

- Understand gene therapy specific CMC challenges during product development
- Determine approaches to address the challenges to ensure regulatory compliance
- Form strategies supporting gene therapy development programs

Speakers
Katherine Whitley, Group Lead, Gene Therapy Purification Development, Pfizer, Inc.

Khandan Baradaran, PhD, Vice President Regulatory CMC, Ultragenyx

Michael Chang, Director Regulatory CMC, PTC Therapeutics, Inc.

12:45-1:15PM — **Break/Visit the Virtual Exhibit Hall**

1:15-2:15PM — **Session 5b:** Case Studies on Challenges for Gene Editing Technology

Session Co-Chairs
Eliana Clark, Senior Vice President Technical Operations and Quality, Intellia Therapeutics, Inc.

Kirsten Messmer, PhD, RAC, Senior Research Analyst, POLITICO's Agency IQ

Genome edited products have recently entered the clinical development phase. These products face very specific CMC challenges due to the technology employed, e.g. off-target editing, translocation. This session will discuss case studies for 2-3 different gene edited products.

At the conclusion of this session, participants should be able to:

- Understand genome edited product specific CMC challenges during product development
- Determine approaches to address the challenges to ensure regulatory compliance
- Form strategies supporting development programs using genome editing technology

Kristy Wood, PhD, Vice President, Nucleic Acid Therapeutics Development & Manufacturing, Intellia Therapeutics

Paul Kopesky, PhD, Senior Director, Process Development, Beam Therapeutics

2:15-2:45PM — **Break/Visit the Virtual Exhibit Hall**

2:45-3:45PM — **Session 5c:** Case Studies on Challenges for Cell-based Therapeutics

Session Co-Chairs
Eliana Clark, Senior Vice President Technical Operations and Quality, Intellia Therapeutics, Inc.

Kirsten Messmer, PhD, RAC, Senior Research Analyst, POLITICO's Agency IQ

Cell-based products use 'living' cells which creates additional challenges. These products face very specific CMC challenges due technology employed and need for maintaining cell product viability. This session will discuss case studies for 2-3 different gene edited products.

At the conclusion of this session, participants should be able to:

- Understand cell-based product specific CMC challenges during product development
- Determine approaches to address the challenges to ensure regulatory compliance
- Form strategies supporting development programs using cell-based therapies

Speakers
Sunitha Lakshminarayanan, MS, MBA, Head and Executive Director of Cell Therapy Process Engineering, Bristol Myers Squibb

Richard Anderson, PhD, MSc, Senior Director, Fate Therapeutics

DAY THREE | WEDNESDAY, NOVEMBER 10

10:00-11:00AM — **Session 6:** Utilizing Regulatory Agency Emerging Technology Teams During Product Development

Session Co-Chairs:
M.Scott Furness, PhD, Deputy Director, Office of New Drug Products, OPQ, CDER, FDA

Kirsten Messmer, PhD, RAC, Senior Research Analyst, POLITICO's Agency IQ

Advanced therapies rely on new and innovative technologies. It is paramount to discuss those modalities with regulators early in the product development to form a consensus on understanding the technology and regulatory requirements. The FDA and EMA have various teams that support these most innovative technologies. The session will provide an overview of opportunities to work with regulators to ensure a streamlined product development process that will comply with regulatory requirements.

At the conclusion of this session, participants should be able to:

- Identify specific teams addressing innovative and developing technologies within regulatory agencies that support product development
- Develop an engagement plan to achieve maximum regulatory alignment
- Create an action plan to engage regulatory agency teams on innovative technology

Speakers
Joel Welch, PhD, Associate Director for Biosimilars and Regulatory Strategy, OBP, OPQ, CDER, FDA

Manuel Osorio, PhD, Senior Scientist for Emerging Technologies and Medical Countermeasures, CBER, FDA

Ana Hidalgo-Simon, DrMed, Head of Advanced Therapies, European Medicines Agency, The Netherlands

11:00-11:15AM — **Break/Visit the Virtual Exhibit Hall**

11:15AM-12:30PM — **Session 7:** Lessons Learned From Development of Vaccines and Therapeutics in Response to COVID-19

Session Chair
Cynthia Schnedar, JD, Principal, Regulatory Compliance, Greenleaf Health LLC

This session will focus on lessons learned from the expedited CMC development of vaccines and therapeutics in response to the COVID-19 pandemic. The session will discuss how sponsors expedited the development of vaccines and therapies; how regulators facilitated an accelerated pathway for

these products; and what lessons can be learned for the future. The session will focus on the CMC development of these products and will cover the benefits of a rolling review process and the application of Real World Evidence in the rollout of vaccines.

At the conclusion of this session, participants should be able to:

- Identify the key strategic pressure points for expedited CMC development for novel vaccines and therapeutics
- Understand the tools FDA is using to create expedited pathways.
- Strategize on how lessons learned from the pandemic can be implemented in the future

Speakers

Barbara Allen, PhD, International Quality Expert

Roger Nosal, PhD, Vice President and Head of Global CMC, Pfizer, Inc.

Norman Baylor, PhD, MS, President and Chief Executive Officer, Biologics Consulting Group, Inc.

12:30-1:00PM — **Break/Visit the Virtual Exhibit Hall**

1:00-2:30PM — **Session 8:** Fireside Chat: Regulatory Approaches to Emerging Technologies and Modalities Closing Remarks

Session Co-Chairs

Judy Chou, PhD, President and CEO, AltruBio Inc.

Kirsten Messmer, PhD, RAC, Senior Research Analyst, POLITICO's Agency IQ

The Biotech Industry is evolving rapidly introducing novel technologies and/or treatment modalities at an ever-increasing speed. Understanding the national and global regulatory landscape is crucial to ascertain rapid patient access to reap the benefits globally. During this Fireside Chat global regulators will discuss current and future regulatory approaches as well as the agency's expectations and requirements. The session will provide the opportunity to discuss regulatory questions with the participants from the regulatory agencies.

At the conclusion of this session, participants should be able to:

- Identify specific teams addressing innovative and developing technologies within regulatory agencies that support product development
- Develop an engagement plan to achieve maximum regulatory alignment
- Create an action plan to engage regulatory agency teams on innovative technology

Speakers

Steven Oh, PhD, Office of Cellular, Tissue, and Gene Therapies, DCGT, CBER, FDA

Ana Hidalgo-Simon, DrMed, Head of Advanced Therapies, European Medicines Agency, The Netherlands

Omar Tounekti, Manager, Gene Therapies Division, Center for Biologics Evaluation, Health Canada

2:30PM — **Conference Adjourns**