August 19-22, 2024 | Boston, MA | Sheraton Boston + Virtual

*Cambridge Healthtech Institute's*

# 16TH ANNUAL BIOPROCESSING SUMMIT

**Register by April 19 for Savings up to $500**

***SOLVING TODAY'S CHALLENGES, LEADING TO TOMORROW'S ADVANCES***

## 2024 PROGRAMS

- Stream #1 **UPSTREAM PROCESSING**
- Stream #2 **DOWNSTREAM PROCESSING**
- Stream #3 **GENE THERAPY**
- Stream #4 **CELL THERAPY**
- Stream #5 **mRNA MANUFACTURING & DELIVERY**
- Stream #6 **ANALYTICAL & QUALITY**
- Stream #7 **STABILITY & FORMULATION**
- Stream #8 **DIGITALIZATION**

## PLENARY KEYNOTE SPEAKERS

**Jerry A. Murry, PhD**
*Senior Vice President, Process Development, Amgen*

## PLENARY FIRESIDE CHAT

**MODERATOR**
**Ann Lee, PhD**
*CTO, Prime Medicine, Inc.*

**E. Morrey Atkinson, PhD**
*Executive Vice President, Chief Technical Operations Officer, Vertex Pharmaceuticals Inc.*

**Manmohan Singh, PhD**
*CTO, Beam Therapeutics*

**Heidi Zhang, PhD**
*Executive Vice President, Head, Technical Operations, Tune Therapeutics*

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Organized by Cambridge Healthtech Institute

#CHIBioprocessingSummit

BioprocessingSummit.com

**Explore** cutting-edge research, exchange insights with industry experts, and chart the course for advancements that will shape the future of bioprocessing.

This year's Bioprocessing Summit features main conference sessions, in-depth training seminars, a 1-day investor conference for C-level executives, a workshop dedicated to acquisition and retention strategies, an engaging exhibit hall, and a plethora of networking opportunities.

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#CHIBioprocessingSummit

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# PLENARY KEYNOTE SESSIONS

## MONDAY, AUGUST 19, 2024 | 4:20-5:30 PM

### SOLVING TODAY'S CHALLENGES

**READY: Addressing Current Challenges in Biomanufacturing with Reliability, Efficiency, Agility, Data, and (High)Yields**

**Jerry A. Murry, PhD**
*Senior Vice President, Process Development, Amgen*

The biopharmaceutical sector is currently producing vast amounts of data, a trend set to amplify with new tech like smart sensors, PAT, and process automation. This presentation will highlight the significance of a holistic digital strategy, incorporating AI, machine learning, predictive modeling, and data visualization, to spearhead the evolution of biomanufacturing. Emphasizing enhanced efficiency and innovation, this strategy will enable the efficient manufacture of complex biologic molecules with reliability of supply, agility, and differentiation. By leveraging these advanced technologies, biomanufacturing can achieve high throughput, ensuring metric tons of life-saving medicines to patients in need around the globe.

## WEDNESDAY, AUGUST 21, 2024 | 4:00-5:00 PM

### LEADING TO TOMORROW'S ADVANCES

**Plenary Fireside Chat: Genetic Medicines—Transforming the Future of Biotherapeutics**

**MODERATOR**

**Ann Lee, PhD**
*CTO, Prime Medicine, Inc.*

**PANELISTS**

**E. Morrey Atkinson, PhD**
*Executive Vice President, Chief Technical Operations Officer, Vertex Pharmaceuticals Inc.*

**Manmohan Singh, PhD**
*CTO, Beam Therapeutics*

**Heidi Zhang, PhD**
*Executive Vice President, Head, Technical Operations, Tune Therapeutics*

Genetic medicines have the potential to revolutionize the treatment of diseases by editing the genes responsible for illness. The landmark approval of CASGEVY, the world's first CRISPR-based treatment, has opened the door to an exciting new era of gene-editing therapies and technologies. Though not without challenges. This unique Fireside Chat brings together leading experts from the fields of CRISPR cas-9, prime editing, base editing, and epigenetics to discuss the technologies, tools, and strategies to succeed in the clinic and commercially.

Join us for an exclusive gathering of the leading investors, innovators, manufacturers, and suppliers who are driving the future of bioprocessing.

# BIOPROCESSING

## Venture, Innovation & Partnering Conference

**August 21, 2024**
**Boston, MA**

*Qualified attendance required*

*Innovation & Investment in Next-Gen Tools & Technologies, for Manufacturing Biologics and Advanced Therapeutics*

### Co-Chairs

**Daniella Kranjac**
Founding Partner & Managing Director,
Dynamk Capital LLC

**Ran Zheng, PhD**
CEO,
Landmark Bio

**Ann Lee, PhD**
CTO,
Prime Medicine


Learn More at Bioprocessingsummit.com/Investor »

## AUGUST 20

# TALENT IN BIOPHARMA WORKSHOP

*Prioritizing Your People Strategy to Accomplish Your Growth Objectives*

*In-person only. Registration required.*

In biopharmaceuticals, talent is arguably a business's greatest asset—and its best differentiator. Prioritizing and connecting your people strategy is essential to accomplishing your growth objectives.

Join us on August 20th for an exclusive interactive gathering of biopharmaceutical stakeholders—HR and talent acquisition leaders, business executives, divisional managers, investors, and allied business partners to learn, discuss, network, and tackle the challenges of hiring and retaining top-tier talent. Discover actionable insights, forge valuable connections, and shape the future of talent management in biopharma.

*Registration includes an invitation to Bioprocessing Summit Welcome Reception & access to Bioprocessing Summit Plenary Sessions!*

## AGENDA:

Registration, Coffee, Networking

Panel Discussion #1: The Power of Your Brand and Culture

Panel Discussion #2: Building Agility into Your Acquisition Strategies

Panel Discussion #3: Prioritizing Talent Retention and Build Strategies

Networking Lunch

Panel Discussion #4: Your Current Workforce Is the Key to Future Success

Panel Discussion #5: Leveraging Technology to Support Talent Objectives

Panel Discussion #6: Talent Is a Strategy Not an Afterthought

Coffee—Final Networking

**FOR MORE DETAILS ON THE WORKSHOP, PLEASE CONTACT:**

**Brian Caine**
Business Development Manager
(908) 809-0946
bcaine@cambridgeinnovationinstitute.com

Learn More »

# CONFERENCE-AT-A-GLANCE

## 2024 Conference Programs

| | AUGUST 19-20 | AUGUST 21-22 |
|---|---|---|
| Stream #1 **UPSTREAM** | Cell Line Engineering and Cell Culture Optimization | Digital Transformation and AI in Bioprocess |
| Stream #2 **DOWNSTREAM** | Intensified and Continuous Bioprocessing | Advances in Purification and Recovery |
| Stream #3 **GENE THERAPY** | Gene Therapy CMC and Analytics | Gene Therapy Manufacturing |
| Stream #4 **CELL THERAPY** | Cell Therapy CMC and Analytics | Cell Therapy Manufacturing |
| Stream #5 **mRNA MANUFACTURING & DELIVERY** | mRNA Development, Analytics and Manufacturing | Formulation and Delivery of High-Concentration Proteins and New Modalities |
| Stream #6 **ANALYTICAL & QUALITY** | Accelerating Analytical Development | Next Generation Analytical Methods |
| Stream #7 **STABILITY & FORMULATION** | Rapid Methods to Assess Stability and Impurities in Biologics | Formulation and Delivery of High-Concentration Proteins and New Modalities |
| Stream #8 **DIGITALIZATION** | Accelerating Analytical Development | Digital Transformation and AI in Bioprocess |
| Training SEMINARS By Cambridge Healthtech Institute | See page 7 for details | See page 7 for details |
| WORKSHOP | Talent in Biopharma Workshop | |
| BIOPROCESSING VENTURE, INNOVATION & PARTNERING CONFERENCE | | Bioprocessing Venture, Innovation & Partnering Conference |

# Training SEMINARS

By Cambridge Healthtech Institute

**In-Person only**

Cambridge Healthtech Institute Training Seminars offer real-life case studies, problems encountered, and solutions applied, along with extensive coverage of the academic theory and background. Each Training Seminar offers a mix of formal lecture and interactive discussions and activities to maximize the learning experience. These Training Seminars are led by experienced instructors who will focus on content applicable to your current research and provide important guidance to those new to their fields.

**Monday, August 19, 2024 10:00 am - 3:30 pm**
**Tuesday, August 20, 2024 8:00 am - 1:00 pm**

## TS8A: Introduction to Alternative Protein Production

***Instructor:***

***Matt McNulty, PhD, Associate Director, Tufts University Center for Cellular Agriculture***

This training seminar will provide a comprehensive introduction to alternative protein production, with an emphasis on cultivated meat and precision fermentation. We will cover production fundamentals in this emerging bioprocessing space and highlight the state of science and technology. The objective of this course is to provide bioprocess scientists with backgrounds in biotherapeutics with an orientation to the alternative protein space to expand their options for technology collaborations and career opportunities.

## TS9A: Introduction to Bioprocessing - Discovery to Commercialization

***Instructors:***

***Martin Hurley, Managing Director, BioPharma Technical Consulting (BPTC)***

***Tiffany D. Rau, PhD, Owner, Rau Consulting LLC***

The seminar will introduce participants to bioprocessing from a process development, manufacturing and regulatory perspective. The seminar will follow a "molecule" from discovery to commercialization that is produced using mammalian cell culture and the different unit operations will be introduced as well as CMC considerations. In addition, different modalities will be explored with regards to opportunities and challenges in development and production methods such as production of Advanced Therapies (Cell and Gene Therapies). In addition, data and its analysis is a critical component to ensure process understanding and minimize CMC challenges and best practices for data management and new statistical methods and tools will be introduced.

## TS10A: Holistic Data Management and Digital Twins for the Bioprocess Life Cycle

***Instructors:***

***Christoph Herwig, PhD, former Professor, Bioprocess Engineering, Vienna University of Technology; CPO, Fermify GmbH; Senior Scientific Advisor, Körber Pharma Austria***

***Sherwin Jayashinghe, Technical Sales Engineer, Koerber Pharma Software***

Regulatory expectations for statistically underpinned Process Validation (PV) have found their way into current guidelines leading to demonstrating Established Conditions (ECs) in ICH Q12. However, successful and accelerated biopharmaceutical process validation (Stage 1-3) remains unresolved in industrial practice. This is due to the necessity of using scale-down models, the cost-intensive setup of experiments, and the complexity due to the interactivity of a multitude of unit operations. The commonly accepted hypothesis is that sound data science and digital twin approaches will be a success factor in this endeavor.

*Please check our website for an updated agenda.*

**Wednesday, August 21, 2024 8:00 am - 3:00 pm**
**Thursday, August 22, 2024 8:00 am - 12:00 pm**

## TS8B: Introduction to Machine Learning and Artificial Intelligence for Bioprocessing Applications

***Instructors:***

***Avinash Dalal, Director of Data Science, Lumilytics***

***Varsha Daswani, PhD, PMP, Senior Director, Data Strategy, Lumilytics***

What does it take to be AI ready? Join us for an interactive 1.5-day training where we examine how to implement generative AI, large language models, and machine learning tools for a bioprocessing organization. We'll define proper use case requirements, explore the necessary components of data integrity, and outline how to go from a proof-of-concept to the deployment of a production level solution. Join us as we play games, compete for prizes, and learn what it means to create a truly fit for purpose digital solution.

## TS9B: Comparability and Potency Assays for Cell, Gene and Biotech Products

***Instructor:***

***Christopher Bravery, PhD, Consulting Regulatory Scientist, Advanced Biologicals Ltd.***

Comparability studies following process change is an inevitable part of drug development, but with wide ramifications for CMC and process development departments alike. Robust potency assays are fundamental also for comparability studies, process validation, and for stability testing. CHI's 1.5-day training seminar, Comparability and Potency Assays for Cell, Gene Therapy and Biotech products provides an in-depth look at the application of regulatory science and biological standardization to biological products; what is potency, and how potency assays differ between biotech and cell and gene therapy products; plus principles of comparability and how their application differs between biotech, cell, and gene therapy products.

## TS10B: Cost and Sustainability Analysis through Modeling

***Instructors:***

***Ken Hamilton, Distinguished Engineer, Genentech***

***Andrew Sinclair, MSc, CEng, FIChemE, FREng, President & Founder, BioPharm Services Ltd.***

***John Welsh, Principal Consultant, Rivanna Bioprocess Solutions LLC***

Optimising biomanufacturing processes in today's fast-paced world is more critical than ever. Applying process cost and sustainability modelling can help you achieve this goal, saving time and money while reducing your environmental impact.
This course is tailored to meet the needs of professionals in process development, tech transfer, and facility process improvement. The course will provide an introduction to predicting the impact of process and technology options on sustainability ( Process Mass Intensity, Water, Energy) and economic outcomes (Cost of Goods COGS, Capital). They are illustrated by practical examples and hands-on anonymised case studies based on mAbs and AAVs to illustrate the topics. The course will provide access to a modelling toolset and the latest optimisation methods based on Bayesian machine learning applicable to bioprocessing evaluating, for example, intensification options, continuous operation, single-use technologies, etc. Leveraging these powerful tools leads to better process understanding and identifies often overlooked areas for improvement.

## TS11B: Introduction to CMC for Biotech, Cell & Gene Therapy Products

***Instructor:***

***Kevin Zen, PhD, Senior Director, IGM Biosciences***

This interactive course will provide a comprehensive CMC overview of therapeutic biological products. It introduces a variety of therapeutic modalities including recombinant proteins, Mab and cell and gene therapy in the context of IMPD and IND regulatory filing. You will learn scientific, technical, and operational aspects of overall biologics CMC activities as well as quality compliance and regulatory requirements. The instructor will present common pitfalls and share the best industry practices.

# STREAM #1
# UPSTREAM PROCESSING

The biopharmaceutical industry has long chased the holy grail: achieving peak productivity, unwavering quality, and cost-efficiency. The past decade has seen remarkable progress in upstream processing, fueled by sophisticated host cell engineering, potent expression cell lines, optimized culturing techniques, and upstream improvements such as perfusion and intensified processing. The next chapter promises a paradigm shift, where intelligence takes center stage. We will witness next-generation platforms such as targeted integration and genetic editing of cell line development, while AI-optimized bioreactor and culture conditions, PAT, digital twins, and machine learning will lead the way to better process understanding, monitoring and control, simulation, and prediction.

## Conference Programs

**AUGUST 19-20**

Cell Line Engineering and Cell Culture Optimization

View Program »

**AUGUST 21-22**

Digital Transformation and AI in Bioprocess

View Program »

## MONDAY, AUGUST 19

**8:00 am Registration and Morning Coffee**

### ADVANCES IN CELL LINE ENGINEERING

**9:55 Chairperson's Remarks**
*Paula Meleady, PhD, Associate Professor, School of Biotechnology, Dublin City University*

**10:00 KEYNOTE PRESENTATION: A Multiomics Perspective on Cell Line Development**
*Susan Sharfstein, PhD, Professor, Nanobioscience, Nanoscale Science and Engineering, University of Albany*
While titers for monoclonal antibody production have increased significantly over the past decade due to extensive cell screening and improved bioprocessing, we still lack a fundamental understanding of the characteristics of high productivity cell lines. In this presentation, I will describe a multi-omics characterization of a parental cell line and its DHFR/MTX amplified progeny, demonstrating substantial physiologic differences between lower and higher productivity cell lines.

**10:30 Site-Specific Integration to Streamline Cell Line Development and Promote Speed to Clinic**
*Shengyuan Zhao, PhD, Senior Scientist, Process Cell Sciences, BPR&D, Merck & Co.*
Traditional cell line development relies on random transgene integration, and its intrinsic variability often requires more time and effort for the process development. This presentation will describe site-specific integration approach to accelerate cell line development by targeting integration of transgenes to support high and stable antibody expression.

**11:00 Targeting Dual Selection as an Expression Tool to Help Drive Stable Production of Correctly-Paired Multispecifics**
*Brian E. Hall, PhD, Distinguished Scientist, Large Molecule Research, Sanofi*
One of the complexities of multispecifics is their requirement for expression of multiple chains in similar ratios for correct molecule pairing. The talk will discuss the use of Targeted Dual Selection as an expression tool to help drive the stable production of correctly paired multispecifics. Use of this technology and strategy early in research can enable simplified purification strategies as well as increased production yields critically required for project progression.

**11:30 Presentation to be Announced**

ASIMOV

**12:00 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:30 Session Break**

**12:50 Chairperson's Remarks**
*Susan Sharfstein, PhD, Professor, Nanobioscience, Nanoscale Science and Engineering, University of Albany*

**12:55 Application of Transposase Technology for Cell Line Development**
*Saurabh Sen, PhD, Associate Director, Cell Line Development, Genomic Medicine Unit CMC, Sanofi*

**1:25 More, Faster, Better, Simpler in Transient Platforms for Protein Production**
*Sowmya Balasubramanian, PhD, Group Leader, Cell Culture, Genentech Inc.*
Transient transfection is used to generate research-grade material to support discovery and early development. Our goal is to develop high titer, automation-friendly transient platforms in CHO and HEK293 cells. We are able to achieve mAb titers of >1 g/L in HEK293 cells and titers of >2 g/L in CHO cells in a 7-day process in scales from 1 mL (96 deep well plates) to 10 L (wave bag).

**1:55 Presentation to be Announced**

BECKMAN COULTER

**2:25 Networking Refreshment Break**

### PROTEIN EXPRESSION STRATEGIES

**2:40 Streamlining DNA Production Using a Cell-Free Platform Technology**
*Beatrice Melinek, PhD, Bioprocess Engineer, University College London*
One serious limitation in the production of new modalities is demand for plasmid DNA, which forms the basis for many. The use of cell-free technologies enables a manufacturing process which, in our hands, is substantially faster, more productive, and more robust with significantly lower space and energy requirements. This presentation explores our experience with cell-free DNA production and infers its potential applications to streamlining of the DNA production process.

**3:10 Investigating the Impact of Codon Optimization on a Recombinantly Expressed Monoclonal Antibody under Different Process Parameters**
*Chava Kimchi-Sarfaty, PhD, Deputy Associate Director, Research, Office of Tissues and Advanced Therapies, CBER, FDA*
Manufacturers can consider different production parameters to meet desired criteria, like improved process yield, during the development process of monoclonal antibodies. We have performed a comprehensive and systematic study on the impact of different codon optimization and cell culture methods on product quality, biochemical features, and functional characteristics. We report changes to glycosylation profiles, charge variants, aggregation, fragmentation, and function among purified protein from combinations of these different production parameters.

**3:40 Session Break and Transition to Plenary Keynote Session**

### PLENARY KEYNOTE SESSION: SOLVING TODAY'S CHALLENGES

**4:20 Organizer's Remarks**
*Daniel Barry, Senior Conference Director, Cambridge Healthtech Institute*

**4:30 PLENARY KEYNOTE PRESENTATION: READY: Addressing Current Challenges in Biomanufacturing with Reliability, Efficiency, Agility, Data, and (High) Yields**
*Jerry A. Murry, PhD, Senior Vice President, Process Development, Amgen*
The biopharmaceutical sector is currently producing vast amounts of data, a trend set to amplify with smart sensors, PAT, and process automation. This presentation will highlight the significance of a holistic digital strategy, incorporating AI, machine learning, predictive modeling, and data visualization, to spearhead the evolution of biomanufacturing. Emphasizing enhanced efficiency and innovation, this strategy will enable the efficient manufacture of complex biologics with reliability of supply, agility, and differentiation.

**5:10 Plenary Q&A**

**5:30 Welcome Reception in the Exhibit Hall with Poster Viewing**

**6:30 Close of Day**

## TUESDAY, AUGUST 20

**7:30 am Registration and Morning Coffee**

## INCREASING PRODUCTIVITY AND STABILITY IN CELL LINES AND EXPRESSION SYSTEMS

**7:55 Chairperson's Remarks**

*Victoria Drake Carnein, Associate Scientist IV, Upstream Process Development, Alexion, AstraZeneca Rare Disease*

**8:00 Repressing Expression of Difficult-to-Express Recombinant Proteins during the Selection Process Increases Productivity of Stable CHO Pools**

*Yves Durocher, PhD, Research Officer & Head, Mammalian Cell Expression, National Research Council Canada*

Many recombinant therapeutic proteins remain challenging to produce in CHO cells. Using a cumate-inducible system permitting reduced DTE protein expression during stable pool selection, we show that pools generated without cumate are significantly more productive compared to selection in the presence of cumate. Reducing expression is associated with higher cell-viability and faster pool-recovery, suggesting reduced cellular stresses and metabolic burden, likely leading to better survival of high-expressing cells.

**8:30 Proteomic Investigation of ER Stress Mechanisms to Enhance Biotherapeutic Productivity from Recombinant Chinese Hamster Ovary Cells**

*Paula Meleady, PhD, Associate Professor, School of Biotechnology, Dublin City University*

ER stress mechanisms are poorly understood in CHO cells and are a major bottleneck in improving the efficiency of production of high-cost recombinant biopharmaceuticals. We have used bioprocess-relevant conditions and artificial inducers of ER-stress (e.g., UPR, ERAD) in recombinant CHO cell lines to characterize the proteome and the ubiquitinated proteome of CHO cells. Proteins of interest have potential to be cell engineering targets to improve efficiency of recombinant protein production.

**9:00 Effects of Osmotic Stress and Heat Shock in Recombinant Protein Expression**

*Yongxue Ding, PhD, Principal Scientist, Biologics Process Design R&D, Abbott Diagnostics Division, Abbott Laboratories*

The main goal of process development for the expression of recombinant proteins in *E. coli* systems is usually to obtain a high accumulation of the target product with proper folding, usually in a soluble form. Our results show that by growing cells under osmotic stress and heat shock, the proportion of soluble protein can be increased from less than 10% to 50%.

**9:30 PAT for mAb Production: Real-Time Upstream Monitoring** 908devices

*Graziella Piras, Senior Director, Strategic Marketing, 908 Devices Inc.*

This talk will highlight: importance of robust PAT for biopharmaceutical production efficiency. Potential of spectroscopic techniques like Raman. Current limitations in model development and validation for these techniques. Need for PAT with quantitative data for process control and building better spectroscopic models.

**10:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**11:00 Optimizing Cell Line Development to Facilitate Drug Development**

*Metewo S. Enuameh, PhD, Senior Scientist, Vector Core Cell Line Development, REGENXBIO, Inc.*

While evaluating a cell line, we realized it was intractable for gene editing to generate the desired homozygous edit. Upon further process development and optimization, we were able to preferentially generate several homozygous cell line clones for subsequent testing for the disease model under consideration. These disease cell line model clones may have a role in facilitating the robust advancement of gene therapy products from lab bench to the clinic.

**11:30 Prediction of CHO Cell Line Stability Using Expression of DNA Repair Genes**

*Hussain Nuruddin Dahodwala, PhD, Director, Upstream Process Development, NIIMBL*

Chinese hamster ovary (CHO) cells—commonly used in biopharmaceutical manufacturing—exhibit production instability. We evaluated five DNA repair genes in over 40 cell lines. Lig4 and Xrcc6 showed higher expression in unstable lines with copy number loss, while lower gene expression correlated with increased cell age. These insights may help predict CHO cell line stability.

**12:00 pm Reshaping Cell Line Development Strategy for Increased Productivity—Vaccine Research Center, NIH Case Studies**

*Nadia Amharref, PhD, Staff Scientist, Vaccine Production Program Lab, NIH NIAID*

In recent years, innovative advancements in cell line development have led to significant improvements in the biopharmaceutical landscape. Integrated with high-throughput screening methodologies, automated platforms have streamlined the traditionally time-consuming steps. However, the continued pressure to reduce timelines and costs while delivering on quality has created unique challenges. This talk will discuss how we adapted our CLD platform to address these challenges and increase productivity in different case studies.

**12:30 C.STATION: End-to-End Automation for Generating Stable Cell Lines for the Development of Advanced Therapeutics** CYTENA A BICO COMPANY

*John Caroll, Regional Sales Manager Biopharma East Coast, Sales, CYTENA GmbH*

Revolutionize cell line development (CLD) workflows with CYTENA's C.STATION. This turnkey automated solution offers efficient single cell isolation, documented clonality assurance, high producer/high-quality clone enrichment, increased throughput, process consistency, and improved data traceability and integrity. It is tailored and configured with the best-in-class instruments and software for monoclonal antibody development, viral vector production, and iPSCs for cell therapy.

**12:45 Presentation to be Announced** RESILIENCE

**1:00 Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**1:30 Refreshment Break in the Exhibit Hall with Poster Viewing**

## OPTIMIZING CELL CULTURE PROCESSES

**2:10 Chairperson's Remarks**

*Sowmya Balasubramanian, PhD, Group Leader, Cell Culture, Genentech Inc.*

**2:15 Development of Two Alternative CHO Culture Harvest Processes Using Acid Precipitation and Cationic Flocculation to Enable Process Scale-Up**

*Victoria Drake Carnein, Associate Scientist IV, Upstream Process Development, Alexion, AstraZeneca Rare Disease*

Advances in cell culture processes have increased cell densities and productivity but have added challenges to cell clarification. In this case study, extensive work was done to develop a centrifugation and depth filtration harvest for a CHO culture process, but these cell clarification methods alone were unable to meet process scale-up needs. Two alternative harvest processes using acid precipitation and cationic flocculation were developed to enable process scale-up.

**2:45 High-Yield Recombinant Adeno-Associated Viral Vector Production by Multivariate Optimization of Bioprocess and Transfection Conditions**

*Louis Coplan, Process Development Engineer II, Regeneron Pharmaceuticals Inc.*

This presentation explores strategies to significantly increase the yield of recombinant adeno-associated viral vectors (rAAVs) used in gene therapy by using multivariate optimization to fine tune both the bioreactor environment and the transfection process for maximum productivity.

**3:15 Controlling Metabolism of CHO Cells in Fedbatch Processes with Raman Models**

*Gayatri Dhara, PhD, Senior Scientist, Upstream Process Development, Pfizer Inc.*

Process analytical technologies (PAT) using Raman spectroscopy in biopharmaceutical development creates opportunities for advanced real-time process monitoring and control. We demonstrate a novel workflow for faster chemometric model development with increased precision using Raman spectral data for several biomolecules of interest from mammalian bioreactors. Resulting Raman models enable more accurate inline monitoring of glucose, lactate, and several amino acids aiding upstream process development for producing monoclonal antibodies/fusion proteins.

**3:45 Refreshment Break in the Exhibit Hall with Poster Viewing**

**4:30 Automated Fermenter Bioprocess in Vaccine Manufacturing: Transcription Trigger and Metabolite Feedback Control**

*Jennifer Reid, PhD, Senior Scientist, Vaccine Drug Substance Development, Sanofi*

An end-to-end automated system used mid-infrared spectroscopy to quantitate metabolites in complex media and biomass probes to control transcription triggers. This enabled continuous control of feed pumps that maintained nutrient levels as well as induction agent input during fed-batch stirred-tank fermentation. The method is adaptable to other systems and enables soft sensing. The ability to quickly develop in-line quantitative metabolite templates and automated transcription triggers is instrumental for project acceleration.

**5:00 Computational Modeling and Mathematical Approaches to Enhance Predictability in Cell Culture**

*Madhuresh Sumit, PhD, Principal Scientist, Pfizer Inc.*

**5:30 Close of Cell Line Engineering & Cell Culture Optimization Conference**

## WEDNESDAY, AUGUST 21

**7:30 am Registration and Morning Coffee**

### DIGITAL AND DATA STRATEGY, INFRASTRUCTURE, AND QUALITY

**7:55 Chairperson's Remarks**
*Mark Duerkop, CEO, Novasign GmbH*
*Angela Li, PhD, Senior Scientist, Sanofi Pasteur*

**8:00 Bringing Data Analysis on Par with Data Generation Speed**
*Christoph Herwig, PhD, former Professor, Bioprocess Engineering, Vienna University of Technology; CPO, Fermify GmbH; Senior Scientific Advisor, Körber Pharma Austria*
No matter if in development or in manufacturing, biopharmaceutical companies swim in data. However, data is not analyzed due to multiple reasons: Missing availability, missing contextualization, different frequency, and different dimensionality. As a result, experiments are not based on previous knowledge, creating an unnecessary waste of resources and costs. This contribution shows how to automatically organize and analyze data at the speed of its generation.

**8:30 UX & Data Quality: Two Sides of the Digital Transformation Coin**
*Madalene Crow, Senior ISA Product Manager, Genentech Inc.*
Case Study presentation to explore the relationship between scientific user experience and high quality data set generation in the context of evolving scientific methods and digital transformation. Digital product innovation guiding principles, a model for informatics product team/scientific user partnership and a summary of lessons learned will be shared.

**9:00 Digitalization of Tech Transfer Strategies: Why and How**
*Niki Wong, PhD, Director Global Tech Operations CMC, Global Tech Operations CMC, AbbVie Operations Singapore Pte Ltd.*
Tech transfer projects have always been stigmatized with tight timelines and limited resources. This presentation would like to tackle this challenge of increasing effectiveness and efficiency of tech transfer challenges by considering lessons learned and what can be done better through digitalization.

**9:30 Presentation to be Announced**



**10:00 Coffee Break in the Exhibit Hall with Poster Viewing**

### STRATEGIES FOR AI/ML APPLICATIONS IN BIOPROCESSING

**10:40 Use of AL/ML in Modeling and Simulation of Upstream and Downstream Bioprocesses for Vaccine Development**
*Angela Li, PhD, Senior Scientist, Sanofi Pasteur*
Hybrid models involving the combination of machine learning (ML) and physical knowledge hold promise to increased efficiency in process development. Case studies will be presented where a hybrid model of microbial cell culture process provides the flexibility and speed needed to bring value at early stages of process development. The presentation will also share the development of a novel hybrid model framework for modeling chromatography processes.

**11:10 Application of AI and Digital Twins for Bioprocessing: Pitfalls and Solution Paths for Accelerated Process Development and Automated Process Control**
*Mark Duerkop, CEO, Novasign GmbH*
In the slowly evolving landscape of bioprocess development and manufacturing, digital bioprocess-twins have emerged as potential accelerators. This presentation will illuminate the essential stages in developing robust process models, encompassing experimental design, customized modeling strategies, smooth scale-up processes, and the real-time application of models for effective monitoring and control. Concrete examples from both upstream and downstream processes will be provided to enhance comprehension of these principles.

**11:40 Perspectives on the Digitalization of the Biomanufacturing Industry**
*Antonio R. Moreira, PhD, Vice Provost, Academic Affairs & Advanced Technology Center, University of Maryland, Baltimore County*
The biopharmaceutical industry is undergoing a major transformation on the heels of the introduction of Pharma 4.0 concepts. The digitalization of the industry is impacting all aspects of biopharma from discovery, research, development, and manufacturing. New products as well as legacy products are exploring how to best employ these tools to optimize benefits. This presentation will discuss current and future perspectives of the digitalization of biopharma.

**12:10 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:40 Refreshment Break in the Exhibit Hall with Poster Viewing**

### KEYNOTE SESSION: THE FUTURE IN DIGITAL BIOMANUFACTURING

**1:25 Chairperson's Remarks**
*Mark Duerkop, CEO, Novasign GmbH*
*Angela Li, PhD, Senior Scientist, Sanofi Pasteur*

**1:30 KEYNOTE PRESENTATION: Global Digital Transformation Program—It's All About Data Consumption**
*Christian Airiau, PhD, Global Head, Data Sciences, CMC, R&D, Sanofi*
Sanofi CMC/Process Development is transforming the way we develop new medicines by driving a data centric approach at the core of our activities.Three use cases are presented to demonstrate how we support Sanofi projects by applying innovative methodologies of quantitative sciences, leveraging empirical, hybrid and mechanistic models to design, optimize and control our processes. We also share our vision for a Digitally mature, AI-enabled process development organization.

**2:00 KEYNOTE PRESENTATION: Applications of Machine Learning in Antibody Discovery, Process Development, Manufacturing, and Formulation: Current Trends, Challenges, and Opportunities**
*Bogdan Gabrys, PhD, Professor of Data Science, Data Science Institute, School of Computer Science, University of Technology Sydney*
While machine learning (ML) has made significant contributions to the biopharmaceutical field, its applications are still in the early stages in the development and manufacturing of biologics, hindering the enormous potential for bioprocesses automation from their development to manufacturing. In this talk we will discuss current applications, the main challenges, and offer insights into the adoption of innovative ML methods in the development of new digital biopharma solutions.

**2:30 Efficiency and Robustness in Process Development for Bio-Production** YOKOGAWA

*Soichiro Shimoda, Manager, Business Design, Yokogawa Electric Corp.*

Yokogawa Electric Corporation is a leading provider of process automation for more than 50 years. Expertise are in technologies for sensing, analyzing, controlling and information management for industrial automation. We would like to share our experience and efforts in the biopharmaceutical industry, such as inline sensing and advanced control algorithms using techniques represented by modeling and machine learning, aiming to realize efficiency and robustness in bio-production.

**3:00 Refreshment Break in the Exhibit Hall with Poster Viewing**

## PLENARY FIRESIDE CHAT: LEADING TO TOMORROW'S ADVANCES

**3:50 Organizer's Remarks**

*Nandini Kashyap, M.Pharm., Senior Director, Conferences and Social Media Strategy, Cambridge Innovation Institute*

**4:00 Genetic Medicines—Transforming the Future of Biotherapeutics**

*Moderator: Ann Lee, PhD, CTO, Prime Medicine, Inc.*
*Panelists:*
*E. Morrey Atkinson, PhD, Executive Vice President, Chief Technical Operations Officer, Head, Biopharmaceutical Sciences and Manufacturing Operations, Vertex Pharmaceuticals Inc.*
*Manmohan Singh, PhD, CTO, Beam Therapeutics*
*Heidi Zhang, PhD, Executive Vice President, Head, Technical Operations, Tune Therapeutics*

**5:00 Networking Reception in the Exhibit Hall with Poster Viewing**

**6:00 Close of Day**

## THURSDAY, AUGUST 22

**7:30 am Registration and Morning Coffee**

## MODELING AND SIMULATION IN UPSTREAM AND DOWNSTREAM PROCESS DEVELOPMENT

**7:55 Chairperson's Remarks**

*Christian Airiau, PhD, Global Head, Data Sciences, CMC, R&D, Sanofi*

**8:00 Evaluating Molecular-Scale, Coarse-Grained Mayer Sampling Simulations for Predicting the Self-Association of Commercial Monoclonal Antibodies**

*Jonathan Janke, PhD, Scientist, Biologic Drug Product Development and Manufacturing, Sanofi*

Screening for CMC protein liabilities is a crucial, although costly, step in mAb drug product development. The diffusion interaction parameter, kD, has been demonstrated to be a highly useful predictor for CMC liabilities, and kD, in conjunction with B22, can be predicted using molecular-scale simulations. After parameterizing coarse-grained simulations, we have determined that these simulations are both robust and efficient for predicting self-interactions of monospecific, commercial mAbs.

**8:30 Closed-Loop Control of Fed-Batch Bioreactors for Monoclonal Antibody Production**

*Anastasia Nikolakopoulou, Investigator—Modeling and Simulation, Pharmaceutical Development, R&D Medicinal Science and Technology, GSK*

In this talk, we discuss model predictive control (MPC) strategies for CHO fed-batch cell culture. MPC strategies have been investigated for their potential to achieve consistent end-of-run titer in the presence of unexpected process disturbances (i.e., iVCC deviations, pH or temperature controller errors). First, we discuss two different modeling frameworks and their integration with MPC. Then, we compare the impact of process disturbances on the process with and without MPC.

**9:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**9:30 Breakout Discussion Groups**

Breakout Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussions page on the conference website for a complete listing of topics and descriptions.

**IN-PERSON ONLY BREAKOUT: Post Model Establishment: Meaningfully Implementing Models in Process Development**

*Terrence Dobrowsky, PhD, Head, Gene Therapy Drug Substance, Biogen*

**IN-PERSON ONLY BREAKOUT: Digital Bioprocessing and Industry 4.0: How Far along Are We?**

*Mark Duerkop, CEO, Novasign GmbH*

This interactive roundtable discussion will cover the following topics:
- Critical evaluation of the current industrial evolution?
- AI vs. mechanistic modeling: what to choose?
- Workflow vs. data: where to invest?
- Outlook—how AI will change the way of bioprocessing in the future?

**10:00 Quantifying Catabolism to Predict and Model the Kinetics of CHO Cell Cultures**

*Sergio Rossell, PhD, Expert Scientist, Upstream Development, GSK*

Mammalian cell lines require complex media. Cells utilize the nutrients available to them as building blocks for biosynthesis, but also as substrates from which they derive the energy to drive biosynthesis and cell maintenance. Here we show how the rates of catabolic reactions can be dissected from the rest of metabolism, and show that catabolism governs the rates of growth and product and byproduct formation in antigen-producing CHO cells.

**10:30 CFD Simulations for Efficient Upscaling of Stem Cell Production in Bioreactors**

*Ramon van Valderen, PhD Candidate, Delft University of Technology*

*Ex-vivo* cultivation of iPSCs for the production of red blood cells is a promising therapeutic alternative to donor-based cell transfusion, yet scale-up of this bioprocess remains challenging. In this work, highly-resolved large-eddy simulations were performed to compare the hydrodynamics of a 125mL shake flask and 250mL bioreactor for various operating conditions, to help translate shake flask operating conditions to bioreactor operating conditions, which ultimately contributes to faster process development times.

**11:00 Industry Maturity Models as the North Star for Digital Transformation**

*Eugene Tung, PhD, Executive Director, Manufacturing IT, Merck & Co., Inc.*

**11:30 Sponsored Presentation** *(Opportunity Available)*

**12:00 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:30 Refreshment Break in the Exhibit Hall & Last Chance for Poster Viewing**

## MODELING AND SIMULATION IN UPSTREAM AND DOWNSTREAM PROCESS DEVELOPMENT (CONT.)

**1:05 Chairperson's Remarks**
*Anastasia Nikolakopoulou, Investigator—Modeling and Simulation, Pharmaceutical Development, R&D Medicinal Science and Technology, GSK*

**1:10 A DoE Approach to Identify and Model the Design Space for Worst-Case Upstream Bioprocessing**
*Wilhad H. Reuter, Lead Engineer, Upstream Process Development, Mural Oncology, Inc.*
Worst-case studies are a facet of late-stage process characterization that are used to model the combination of factors that have the least desirable outcome in a manufacturing process. In this case study, both screening and response surface DoEs were executed to identify the highest risk factors on a 14-day fed-batch cell culture process. These models were then leveraged for designating the Upstream Control Strategy AORs prior to PPQ manufacturing.

**1:40 A Novel Digital Twin for Enhancing rAAV Production in Sf9/ Baculovirus Cultures**
*Francesco Destro, PhD, Postdoctoral Associate, Chemical Engineering, Center for Biomedical Innovation, MIT*
This work introduces a groundbreaking digital twin designed to enhance the production of recombinant-adeno-associated virus (rAAV) within baculovirus/ Sf9 cultures—a platform responsible for producing 50% of commercial rAAV-based gene therapies. A mechanistic model is developed to systematically identify bottlenecks within the intracellular pathway for full rAAV capsid formation in producer cells. After experimental validation, the digital twin indicates genetic modifications and process enhancements aimed at boosting overall platform productivity.

**2:10 Digital Twin Strategy for Continuous Manufacturing of Biologics: Case Study**
*Pedro de Azevedo Delou, Senior Consultant Engineer, Siemens Industry Software*
*Robert Taylor, PhD, Associate Scientist, Bioseparation Sciences, Merck Manufacturing Division*
Through this work, we designed and conducted *in silico* DOE runs, decreasing the number of experiments, material, and the overall program timeline and costs of process development and commercialization phases. Currently, we are initiating our first mechanistic models for some of the operation units, and attempt to generate first feedback controls through integration of tangential flow filtration models as soft sensors for membrane fouling.

**2:40 Networking Refreshment Break and Transition into Town Hall Discussions**

## FACILITATED TOWN HALL DISCUSSIONS

**2:55 Facilitated Town Hall Discussions**
These Town Halls offer delegates the opportunity to participate in interactive discussions on important themes that were explored during the conference. Each Hall will have a host(s) to facilitate the conversation, and all are welcome to participate, share views and best practices and ask questions of colleagues.

**Harnessing ML/AI and Big Data for Biotherapeutic Development**
*Pin-Kuang Lai, PhD, Assistant Professor, Department of Chemical Engineering and Materials Science, Stevens Institute of Technology*

**Cell and Gene Therapy Manufacturing: In-House vs. Outsourced**
*Elben Guimaraes, Senior Manufacturing Manager, Upstream Manufacturing, Ultragenyx Pharmaceutical Inc.*
The decision of handling cell and gene therapy processes in-house or outsourcing them is crucial. This facilitated discussion explores the advantages and challenges of both approaches, analyzing their impact on cost, control, strategic direction, and innovation. Share experiences and best practices for managing internal and external manufacturing, while examining common scenarios faced by sponsors and vendors.

**Digital Transformation & AI in Bioprocess—What, Where, When, and How?**

*Christian Airiau, PhD, Global Head, Data Sciences, CMC, R&D, Sanofi*
*Irene Rombel, PhD, CEO & Co-Founder, BioCurie Inc.*
The bioprocessing industry is undergoing a digital revolution fueled by AI. This interactive session dives into current digital adoption and explores the latest trends in AI applications (AIML). Join the conversation to explore the potential of AI for process optimization and digital twins. Share real-world success stories and discuss ethical considerations along with potential workforce impacts.

**3:55 Close of Summit**

# STREAM #2
# DOWNSTREAM PROCESSING

The rise of high-potency therapies and complex biologics is pushing the boundaries of downstream processing. The bottleneck is shifting from upstream production to purifying these intricate molecules efficiently and sustainably. Companies are exploring AI-optimized process optimization, and continuous manufacturing for higher yield and agility. New materials such as membranes and resins, and innovations in affinity chromatography, microfluidics, rapid cycling, etc., continue to push improvements in the field. Meanwhile, the hunt for greener solutions is on, with the goal of minimizing waste and environmental impact. Join the Downstream Processing conferences to witness first-hand the tools and strategies shaping the future of biologics manufacturing.

## Conference Programs

**AUGUST 19-20**

Intensified and Continuous Bioprocessing

View Program »

**AUGUST 21-22**

Advances in Purification and Recovery

View Program »

## MONDAY, AUGUST 19

**8:00 am Registration and Morning Coffee**

### PERFUSION CELL CULTURE

**9:55 Chairperson's Remarks**
*Philip Probert, PhD, Technology Lead, CPI, United Kingdom*

**10:00 Optimization Strategies for Developing Robust Perfusion and Harvest Methods for High Cell Density Mammalian Cultures**
*Jessica Pedroso, PhD, Process Development Principal Scientist, Pivotal Drug Substance Technologies, Amgen Inc.*
High cell density mammalian cultures using continuous perfusion is prevalent in the biotech industry; however, as patient demand increases, continuous optimization is required to achieve higher yields while lowering cost of goods. Robust high cell density cultures must be able to maintain the health of high cell density cultures, minimize membrane fouling of cell retention devices, and maintain high harvest yields.

**10:30 Contributions of CHO Extracellular Vesicles and Other Cellular Materials to Hollow Fiber Filter Fouling during Perfusion Manufacturing of Monoclonal Antibodies**
*Yixiao Zhang, PhD, Senior Scientist, Merck*
We present the first comprehensive analysis of the dynamics of Chinese hamster ovary (CHO) cell-derived extracellular vesicles during the perfusion production process. The structural and elemental analysis of the fouled hollow fiber tangential flow filtration filters sheds light on hollow fiber filter fouling mechanism and strategies to mitigate filter fouling.

**11:00 Evaluate and Optimize Perfusion Cell Culture in Manufacturing Using Bayesian Optimization Linked to Process Models**
*Andrew Sinclair, MSc, CEng, FIChemE, FREng, President & Founder, BioPharm Services Ltd.*
Many degrees of freedom are available in the operation of perfusion cell culture at scale. What is the optimal configuration accounting for target cell density, VVD, duration of the perfusion run, seeding density, etc.? By looking at competing objectives of capital, cost of goods, and sustainability, a design space is identified that allows optimal configurations and an understanding of the trade-offs between economics and sustainability.

**11:30 Presentation to be Announced**

REPLIGEN

**12:00 pm Luncheon Presentation to be Announced**

PAK BioSolutions

**12:30 Session Break**

### SUSTAINABILITY IN BIOPROCESSING

**12:50 Chairperson's Remarks**
*Andrew Sinclair, MSc, CEng, FIChemE, FREng, President & Founder, BioPharm Services Ltd.*

**12:55 KEYNOTE PRESENTATION: Enabling Sustainability in Biotechnology via Innovation**
*David J. Roush, PhD, CEO & Distinguished Scientist, Roush Biophama Panacea*
Innovation is the foundation for biotechnology, creating many life-saving therapies. Delivery of technologies and medicines globally requires a recognition that resources are limited, requiring highly productive and efficient processes. A new perspective is that sustainability is an integral part of research, process/technology development, manufacturing, and supply chain—and is intrinsically linked to innovation. A holistic identification of new opportunities (e.g., modeling) and measurements enables innovation and affords sustainability of bioprocessing.

**1:25 Balance Sustainability and Profitability: Evaluating Process Intensification and Continuous Processing through Economic and Ecological Modeling**
*Lijuan Li, Senior Staff Engineer (in silico CMC), Technology Development & Implementation, Takeda*
We integrated economic and ecological modeling to assess the impact of emerging technologies, including N-1 perfusion and continuous capture on biopharmaceutical manufacturing processes. By evaluating key metrics such as cost of goods, waste generation, and energy consumption across various manufacturing scenarios, we aimed to elucidate the trade-offs and synergies between sustainability and profitability. Our findings provide valuable insights towards the development of more cost-effective and sustainable biomanufacturing processes.

**1:55 Presentation to be Announced**

Purolite An ecolab company

**2:25 Networking Refreshment Break**

**2:40 Green Metrics to Reduce Environmental Impact of Biologics**
*Felix Dieringer, PhD Student, BOKU Vienna*
As the market share of biologics continues to grow, the biopharmaceutical industry is placing increasing emphasis on sustainable production. However, quantifying the environmental impact of manufacturing processes remains a challenge. This talk will delve into existing green metrics and explore novel ones, looking at different scales, modalities, and process options. While assessing strengths and weaknesses of said metrics, opportunities for a more sustainable production are identified.

**3:10 PANEL DISCUSSION: Sustainability in Bioprocessing**
*Moderator: Alois Jungbauer, PhD, Professor & Head, Biotechnology, Institute of Bioprocess Science and Engineering, University of Natural Resources and Life Sciences (BOKU)*
- Chasing the goal of net-zero
- Renewable raw materials and energy sources
- Reduction of water consumption and carbon emissions
- Recycling of plastics and residual waste materials
- How to transform large footprints of legacy biomanufacturing to smaller footprint, sustainable manufacturing?
- Comparison of sustainability manufacturing using batch vs. continuous mode; stainless steel vs. single-use bioreactors; small-scale vs. large-scale
- Comparison between biologics and cultivated meat processing and manufacturing
- Sustainability scoring and performance indicators
- Circular bio-economy

*Panelists:*
*Lijuan Li, Senior Staff Engineer (in silico CMC), Technology Development & Implementation, Takeda*
*Matt McNulty, PhD, Associate Director, Tufts University Center for Cellular Agriculture*
*David J. Roush, PhD, CEO & Distinguished Scientist, Roush Biophama Panacea*

**3:40 Session Break and Transition to Plenary Keynote Session**

### PLENARY KEYNOTE SESSION: SOLVING TODAY'S CHALLENGES

**4:20 Organizer's Remarks**
*Daniel Barry, Senior Conference Director, Cambridge Healthtech Institute*

**4:30 PLENARY KEYNOTE PRESENTATION: READY: Addressing Current Challenges in Biomanufacturing with Reliability, Efficiency, Agility, Data, and (High) Yields**

***Jerry A. Murry, PhD, Senior Vice President, Process Development, Amgen***

The biopharmaceutical sector is currently producing vast amounts of data, a trend set to amplify with smart sensors, PAT, and process automation. This presentation will highlight the significance of a holistic digital strategy, incorporating AI, machine learning, predictive modeling, and data visualization, to spearhead the evolution of biomanufacturing. Emphasizing enhanced efficiency and innovation, this strategy will enable the efficient manufacture of complex biologics with reliability of supply, agility, and differentiation.

**5:10 Plenary Q&A**

**5:30 Welcome Reception in the Exhibit Hall with Poster Viewing**

**6:30 Close of Day**

## TUESDAY, AUGUST 20

**7:30 am Registration and Morning Coffee**

## INTEGRATED AND INTENSIFIED DOWNSTREAM PROCESSES

**7:55 Chairperson's Remarks**

***Jessica Pedroso, PhD, Process Development Principal Scientist, Pivotal Drug Substance Technologies, Amgen Inc.***

**8:00 Evaluation of Capture SMB Continuous Chromatography as a Means to Intensify an Existing Antisense Oligonucleotide Process**

***Samuel Fredericksen, Oligonucleotide Purification Development Engineer, Biogen***

Demand for therapeutic oligonucleotides is rapidly increasing, resulting in the need for intensified downstream processes. CaptureSMB is a continuous chromatography method that has shown great promise in increasing the capacity and productivity of chromatographic processes without increasing column size. In this presentation we describe an instance in which a CaptureSMB approach improved both the productivity and purity of a high-volume, historically challenging ASO process.

**8:30 Development of an Integrated Continuous mRNA Precipitation-Based Purification Process**

***Carme Pons Royo, PhD, Postdoctoral Associate, Massachusetts Institute of Technology***

mRNA-based therapeutics have emerged as cutting-edge technologies for treating various diseases. Current downstream processing, which relies on a series of chromatography methods and TFF, remains challenging with low yields and significantly impacted final production costs. We will present our integrated and continuous manufacturing process for mRNA production and purification. We are investigating novel methods for continuous mRNA precipitation-based purification, including various precipitating agents, and following precipitation with continuous flow filtration.

**9:00 Innovations Enabling the Development of Intensified Processes for RNA-LNPs**

***Philip Probert, PhD, Technology Lead, CPI, United Kingdom***

mRNA-LNP-based products continue to show promise, with various companies taking candidates through clinical trial. With the need for greater quantities of product, particularly for high-dose therapeutics, cost of goods and supply chain limitations may limit access to potentially revolutionary and life-saving products. This talk will discuss opportunities and challenges of mRNA process intensification, including approaches trialed at CPI with associated case data.

**9:30 Presentation to be Announced**


**10:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**10:45 Breakout Discussion Groups**

Breakout Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussions page on the conference website for a complete listing of topics and descriptions.

**IN-PERSON ONLY BREAKOUT: What Are the Remaining Technical Barriers Limiting the Widespread Adoption of More Sustainable Manufacturing?**

***Philip Probert, PhD, Technology Lead, CPI, United Kingdom***

Despite interest and demonstration of intensified and continuous platforms for biologics manufacturing, uptake remains low.

- What are the perceived risks in moving away from conventional batch-based processing for new products?
- Will there ever be a sufficiently strong driver for moving approved drugs to more sustainable manufacturing approaches?
- What further advice could regulators give to guide process developers and manufacturers to de-risk more complex production approaches?

**IN-PERSON ONLY BREAKOUT: Process Intensification Strategies: What To Do When and Where in Your Product Lifecycle**

***Stefan R. Schmidt, PhD, MBA, CEO, evitria AG***

During early development, processes are primarily intensified to increase speed and to reduce material consumption to get as fast as possible to the clinical stage. In late-stage development or commercial manufacturing, the space time yield for the product is more relevant as robustness and output must be maximized. In this session we will discuss when to do what at which stage.

**11:30 Primary Recovery Platform—Next-Generation Bioprocessing (NGB)**

***Alex Cohen, Senior Principal Engineer, Bioproduct R&D, Eli Lilly & Co.***

NGB and upstream process intensification in monoclonal cell culture have challenged platform primary recovery centrifuge and depth-filtration operations with high-harvest solids. NGB primary recovery changes have been evaluated such as optimization of a continuous-discharge centrifuge and depth-filter media to improve operational process control, improve capacity, and reduce shear. Changes have been implemented to provide a robust and capable process and have been recommended for platform updates.

**12:00 pm Continuous High-Concentration Formulation of Biologics**

***Daniel P. LaCasse, Senior Principal Scientist, Early Stage Biologics Process Development, Pfizer Inc.***

To enable a continuous high concentration formulation process, three evolving technologies, single-pass TFF (SPTFF) for volume reduction, Cadance In-line Diafiltration (ILDF) for buffer exchange, and Flow VPE for direct in-line concentration measurement were optimized and integrated. A simple integrated system enabled volume reduction upto 20-fold while delivering above 3 $\log_{10}$ washout of small solutes, matching the capabilities of conventional batch ultrafiltration with diafiltration.

**12:30 Sponsored Presentation** *(Opportunity Available)*

**1:00 Luncheon Presentation to be Announced**

**1:30 Refreshment Break in the Exhibit Hall with Poster Viewing**

METTLER TOLEDO

**2:10 Chairperson's Remarks**
***Robert S. Gronke, Senior Principal Scientist, Technical Development, Biogen***

**2:15 A Fully Continuous Downstream Process for mAbs with Precipitation-Based Capture and Flowthrough Polish**
***Todd M. Przybycien, PhD, Professor, Chemical and Biological Engineering, Rensselaer Polytechnic Institute***
We are developing an intensified continuous downstream process for monoclonal antibody (mAb) production with target precipitation for capture purification and flow-through chromatography for polish purification. The process addresses the volumetric throughput, buffer usage, and cost-of-goods bottlenecks associated with the platform Protein A-based capture step that currently limits mAb manufacturing capacity. We have processed four commercial harvest cell culture fluids and will report the corresponding process performance metrics and mAb CQAs.

**2:45 Demonstration of Impurity Removal, Viral Clearance, Resin Cleaning, and Resin Lifetime on an Intensified Flowthrough CEX Step**
***Joanne Gilchrist, Scientist I, Process Biochemistry, Biogen***
Intensified upstream processes exert pressure downstream to efficiently purify therapeutic proteins while maintaining product quality. This work focuses on the development of a step for HMW removal, trace impurity clearance, and viral clearance, using a CEX resin designed for flow-through chromatography. Promising results were observed at high mass loading without sacrificing impurity removal. Resin cleaning and reuse studies were used to evaluate resin suitability for long-term reuse at manufacturing scale.

**3:15 Residence Time Distribution of Batch and Continuous Viral Filtration**
***Alois Jungbauer, PhD, Professor & Head, Biotechnology, Institute of Bioprocess Science and Engineering, University of Natural Resources and Life Sciences (BOKU)***
Regulatory authorities recommend using RTD to address material traceability in continuous manufacturing. Continuous virus filtration is an essential but poorly understood step in biologics manufacturing in respect to fluid dynamics and scale-up. A model that considers non-ideal mixing and film resistance for RTD prediction in continuous virus filtration, and its experimental validation using the inert tracer NaNO3 is described. Effect of RTD on startup and shut-down will be shown.

**3:45 Refreshment Break in the Exhibit Hall with Poster Viewing**

## INCORPORATING CONTINUOUS MANUFACTURING IN EARLY PROCESS DESIGN

**4:30 Continuous Manufacturing Process Technologies Enable Productivity and Reliability**
***Madiha Khurshid, Senior Associate, Pivotal Drug Substance Process Development, Amgen Inc.***
Continuous manufacturing (CM) of biologics has gained significant interest in industry due to its flexibility and smaller facility footprint. Though the capital investment required by a CM suite into an existing plant is lower, optimization of the process design is important to minimize operating cost. There are also opportunities to improve the process monitoring and control strategies to ensure reliability.

**5:00 Advancing Bioprocessing Development with Early Manufacturing Incorporation**
***Hamza Ahsan, Principal Engineer, Pharma Tech Development, Genentech Inc.***
Bioprocess development typically ensures that aspects of product quality, robustness, and productivity are taken into consideration. However, these aspects are then subject to the capabilities available when implementing the bioprocesses into manufacturing facilities. At Roche/Genentech, within bioprocess development we have focused on incorporating manufacturing capabilities into process-design spaces. This has ensured processes do not experience late changes during tech transfer and enabled better robustness of processes.

**5:30 Close of Intensified and Continuous Bioprocessing Conference**

## WEDNESDAY, AUGUST 21

**7:30 am Registration and Morning Coffee**

### DOWNSTREAM PROCESSING FOR NON-MABs AND COMPLEX FORMATS

**7:55 Chairperson's Remarks**

*Abraham M. Lenhoff, PhD, AP Colburn Professor, Chemical & Biomolecular Engineering, University of Delaware*

*Pranali Shah, Senior Associate Scientist, Process Development, Amgen Inc*

**8:00 KEYNOTE PRESENTATION: A Crystallization-Based Approach for the Separation of Full and Empty AAV Capsids**

*Richard D. Braatz, PhD, Edwin R. Gilliland Professor, Chemical Engineering, Massachusetts Institute of Technology*

The Adeno-Associated Virus (AAV) capsids produced by cells for gene therapy applications are a mixture of capsids that contain the full-length gene and capsids that do not. The commercial separation processes for increasing the proportion of full capsids have low yields. A high-yield crystallization-based approach is demonstrated for the separation of full and empty AAV capsids. The capsids obtained by dissolution of the full capsid crystals are biologically active.

**8:30 NC-VVIRAL Case Studies: Downstream Processing of Adeno-Associated Virus (AAV), Lentivirus (LV), Adenovirus (AdV), Baculovirus (BeV), and Influenza Vaccines—Purification Technologies and Custom Analytics**

*Stefano Menegatti, PhD, Associate Professor, Chemical & Biomolecular Engineering, North Carolina State University*

Viral vectors and vaccines are poised to become an integral part of modern medicine. As new vector designs are introduced with improved efficacy and safety—but also growing complexity—a question looms at the horizon: how to affordably produce clinically-relevant amounts of viral vectors and vaccines with high purity and activity? NC-VVIRAL bridges the technology gap in viral biomanufacturing through a suite of innovative expression and purification tools!

**9:00 Harvest Filtration Strategies to Clarify High-Cell Density rAAV Productions**

*Dennis P. Chen, Senior Scientist, Downstream Process Development, Ultragenyx Pharmaceutical*

We employed a scalable rAAV production process using our Pinnacle PCL platform that reduces the high costs associated with transfection-based processes. However, at cell densities that yield high volumetric productivity, the increased impurity burden prohibits the practical use of conventional filtration schemes. Through optimization of our filtration scheme, we have designed a robust downstream process capable of handling the challenging feed stream of a high-cell density production.

**9:30 Presentation to be Announced** CYGNUS TECHNOLOGIES part of Maraval LifeSciences

**10:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**10:40 Overcoming Challenges to a Non-Platform Antisense Oligonucleotide Purification Process for Late-Stage Clinical Studies**

*Patrick Banzon, Associate Scientist, Biogen*

ASO-based therapies have offered effective treatments for many neurodegenerative diseases through addressing the pathology as opposed to the symptoms. Next-generation ASO therapies via new chemistries pose even greater clinical promise (e.g., longer duration), though they can disrupt existing ASO processing platforms. This presentation summarizes the purification of a late-stage, next-generation oligonucleotide that encountered, and later overcame, several challenges to deliver a drug substance of expected high purity and yield.

**11:10 Overcome Modality-Related Challenges and Develop Effective Downstream Processes for Non-mAb Protein Therapeutics**

*Mark Yang, PhD, Vice President, CMC, Palleon Pharmaceuticals*

Non-mAb proteins are known for their complex structure, poor expression titer, prone to aggregation, and sensitivity to process stresses. These modality related issues often complicate the downstream processes and compromise their performance. This presentation discusses the common challenges and strategies to improve the non-mAb harvest recovery, streamline the chromatography layout and operations, enhance the process effectiveness for viral and HCP clearance, and minimize product and process impurities in the bulk.

**11:40 Development of a Platform Purification Process for Novel Non-Viral Gene Therapy Modality: Harvest, Lysis, and Clarification Optimization**

*Ronit Ghosh, PhD, Purification Process Development Scientist, Genomic Medicine Unit, Sanofi*

This presentation details the platform development of a purification process for a new class of non-viral gene therapies. The talk will emphasize the optimization of initial steps including harvesting, lysis, and clarification.

**12:10 pm Luncheon Presentation to be Announced** SUNRESIN

**12:40 Refreshment Break in the Exhibit Hall with Poster Viewing**

**1:25 Chairperson's Remarks**

*Abraham M. Lenhoff, PhD, AP Colburn Professor, Chemical & Biomolecular Engineering, University of Delaware*

*Pranali Shah, Senior Associate Scientist, Process Development, Amgen Inc*

**1:30 Capture Redox: An Efficient Method for Generation of Multispecific Antibodies**

*Michael King, PhD, Senior Scientist, Pfizer Inc.*

This work highlights the development of a redox reaction that occurs during the capture chromatography step resulting in the efficient formation of multispecific antibodies. The method consists of simultaneously binding two separate homodimers to a chromatography resin then applying a reductant wash to reduce the interchain disulfide bonds in both antibodies. The antibodies are then eluted and neutralized in the presence of an oxidant to form the heterodimer.

**2:00 What to Consider in Early-Stage Development of Complex Biologics—Learning from Multispecific mAb Case Studies**

*Guannan He, PhD, Principal Research Scientist I, CMC Bioprocess, Development Sciences, Abbvie Biotherapeutics Inc.*

Multi-specific antibodies can bind to multiple antigens/epitopes and enable simultaneous engagement of different targets. More multi-specific antibodies are entering clinical development than ever before. However, the complex structure of the multi-specific antibodies has caused more product related

impurities, such as aggregates and half antibody. The case studies describe the significant challenges encountered during the FIH downstream process development and methodologies employed to achieve a successful GMP production campaign.

**2:30 Presentation to be Announced**

**3:00 Refreshment Break in the Exhibit Hall with Poster Viewing**

## PLENARY FIRESIDE CHAT: LEADING TO TOMORROW'S ADVANCES

**3:50 Organizer's Remarks**
*Nandini Kashyap, M.Pharm., Senior Director, Conferences and Social Media Strategy, Cambridge Innovation Institute*

**4:00 Genetic Medicines—Transforming the Future of Biotherapeutics**

*Moderator: Ann Lee, PhD, CTO, Prime Medicine, Inc.*
*Panelists:*
*E. Morrey Atkinson, PhD, Executive Vice President, Chief Technical Operations Officer, Head, Biopharmaceutical Sciences and Manufacturing Operations, Vertex Pharmaceuticals Inc.*
*Manmohan Singh, PhD, CTO, Beam Therapeutics*
*Heidi Zhang, PhD, Executive Vice President, Head, Technical Operations, Tune Therapeutics*

**5:00 Networking Reception in the Exhibit Hall with Poster Viewing**

**6:00 Close of Day**

## THURSDAY, AUGUST 22

**7:30 am Registration and Morning Coffee**

## REGULATORY UPDATES AND PROCESS VALIDATION

**7:55 Chairperson's Remarks**
*Stefano Menegatti, PhD, Associate Professor, Chemical & Biomolecular Engineering, North Carolina State University*

**8:00 Regulatory Updates and Guidances on Downstream Processing and Viral Safety**
*Tiffany D. Rau, PhD, Owner, Rau Consulting LLC*
ICH Q5A was recently updated, which addresses viral safety of biotechnology products derived from cell lines of human or animal origin, and the updates will be discussed along with best practices to address and manage the changes within CMC programs. In addition, updates to Annex 1 and how it applies to downstream operations will be presented.

**8:30 Process Validation of a Self-Removing Affinity Tag for cGMP Biologics Manufacturing**
*David W. Wood, PhD, Professor, Chemical & Biomolecular Engineering, The Ohio State University*
Self-removing affinity tags provide a powerful platform for purifying untagged recombinant proteins without the need for proteolytic tag removal and have been successfully applied to a variety of biosimilars and other therapeutic protein classes. This work focuses on methods for validation of tag removal from the purified product as part of a cGMP manufacturing platform. Several case studies will be provided, with specific steps and data provided.

**9:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**9:30 Breakout Discussion Groups**
Breakout Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussions page on the conference website for a complete listing of topics and descriptions.

**IN-PERSON ONLY BREAKOUT: Challenges and Opportunities in Membrane-Based Separations in Bioprocessing**
*Jian Ren, PhD, Principal Scientist, AbbVie*
- Membrane-based separation techniques are essential in bioprocessing to enable clarification, virus filtration, UFDF, and sterile filtration etc.
- There is strong demand for high performance membrane-based separations for high cell density culture, high throughput viral filtration, and high concentration formulations
- Opportunities also arise in using membrane-based techniques for novel modes of separation, such as membrane chromatography

## NOVEL METHODS AND APPROACHES FOR DOWNSTREAM OPTIMIZATION

**10:30 Protein Adsorption on Core Shell Resins for Flow-through Purification—Structure and Mechanisms**
*Giorgio Carta, PhD, Lawrence R. Quarles Professor Emeritus of Engineering and Applied Science, Chemical Engineering, University of Virginia*
Purification of large biomolecules and bioparticles, including large plasmids, virus, virus-like-particles, and vesicles, by flow-through chromatography has been made practical with the availability of effective core-shell resins. We examine the structural and functional properties of commercial agarose-based core-shell resins and develop models to describe the kinetics of binding for proteins with a broad range of molecular mass in single and multiple component systems and predict the dynamic binding capacity.

**11:00 Optimizing Multicolumn Chromatography for Protein A Capture Step**
*Alexander Way, Scientist, AbbVie*
Multicolumn chromatography is an established strategy to improve productivity and reduce resin usage. However, the increased column loading can lead to elevated levels of impurities, resulting in a trade-off between productivity and product quality. In this presentation we describe different strategies, including harvest improvements, column loading optimization, and wash condition screening, to mitigate this challenge and improve impurity clearance while maintaining comparable product quality and process performance to batch processing.

**11:30 Presentation to be Announced**

**12:00 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:30 Refreshment Break in the Exhibit Hall & Last Chance for Poster Viewing**

## NOVEL METHODS AND APPROACHES FOR DOWNSTREAM OPTIMIZATION (CONT.)

**1:05 Chairperson's Remarks**
*David W. Wood, PhD, Professor, Chemical & Biomolecular Engineering, The Ohio State University*

**1:10 Mechanisms and Modeling of Primary Depth Filtration**
*Abraham M. Lenhoff, PhD, AP Colburn Professor, Chemical & Biomolecular Engineering, University of Delaware*
Depth filtration is routinely used for primary clarification of cell culture fluid, but its analysis and design are almost entirely empirical. We present a conceptual mechanistic model that can account for sieving, adsorption, and caking in modeling the pressure drop and filtrate turbidity, and also use multiple experimental methods to obtain supporting data to aid in model discrimination regarding the mechanisms involved.

**1:40 Harvest Development and Optimization Using pDADMAC Flocculation**
*Kate Zhao, PhD, Scientist I, Alexion*
Advances in cell culture processing have not only resulted in increased cell densities and productivity, but also in increased level of sub-micron particles, which decrease the efficiency of the cell separation step. A harvest method using a polymer, pDADMAC, was investigated for the removal of colloids and for improvements in cell clarification. This presentation will focus on the implementation of large-scale harvest using pDADMAC and examination of pDADMAC flocculation performance.

**2:10 Novel Approach to Affinity Capture Elution Design**
*Wei Lu, PhD, Staff Engineer, Bioprocess Development, Takeda*
Affinity Capture is the preferred method for primary capture in biotherapeutic downstream processing. However, the hash elution condition may be incompatible with product stability and negatively impact product quality. We propose a novel approach to the design of affinity elution buffers for challenging products by leveraging our new discovery, which could maintain product quality, achieve high yield, and assure maximum compatibility with subsequent step.

**2:40 Networking Refreshment Break and Transition into Town Hall Discussions**

## FACILITATED TOWN HALL DISCUSSIONS

**2:55 Facilitated Town Hall Discussions**
These Town Halls offer delegates the opportunity to participate in interactive discussions on important themes that were explored during the conference. Each Hall will have a host(s) to facilitate the conversation, and all are welcome to participate, share views and best practices and ask questions of colleagues.

**Harnessing ML/AI and Big Data for Biotherapeutic Development**
*Pin-Kuang Lai, PhD, Assistant Professor, Department of Chemical Engineering and Materials Science, Stevens Institute of Technology*

**Cell and Gene Therapy Manufacturing: In-House vs. Outsourced**
*Elben Guimaraes, Senior Manufacturing Manager, Upstream Manufacturing, Ultragenyx Pharmaceutical Inc.*
The decision of handling cell and gene therapy processes in-house or outsourcing them is crucial. This facilitated discussion explores the advantages and challenges of both approaches, analyzing their impact on cost, control, strategic direction, and innovation. Share experiences and best practices for managing internal and external manufacturing, while examining common scenarios faced by sponsors and vendors.

**Digital Transformation & AI in Bioprocess—What, Where, When, and How?**

*Christian Airiau, PhD, Global Head, Data Sciences, CMC, R&D, Sanofi*
*Irene Rombel, PhD, CEO & Co-Founder, BioCurie Inc.*
The bioprocessing industry is undergoing a digital revolution fueled by AI. This interactive session dives into current digital adoption and explores the latest trends in AI applications (AIML). Join the conversation to explore the potential of AI for process optimization and digital twins. Share real-world success stories and discuss ethical considerations along with potential workforce impacts.

**3:55 Close of Summit**

# STREAM #3
# GENE THERAPY

The Gene Therapy stream focuses on the critical challenges facing the analysis, characterization, quality control and manufacture of gene therapies for clinical and commercial supply, viral and non-viral-based. Split across two back-to-back tracks, Gene Therapy CMC and Analytics, and Gene Therapy Manufacturing, topics include product and process characterization, CMC, upstream development, molecular biology, potency assays, comparability, emerging analytical technologies, impurities, quality control, comparability, process development, purification, formulation, scale-up and commercial manufacturing.

## Conference Programs

**AUGUST 19-20**

Gene Therapy CMC and Analytics

View Program »

**AUGUST 21-22**

Gene Therapy Manufacturing

View Program »

# Gene Therapy CMC and Analytics

*Improving the Analysis, Control, and Quality of Gene Therapies*

**AUGUST 19-20**
All Times EDT

## MONDAY, AUGUST 19

**8:00 am Registration and Morning Coffee**

## COMMERCIALIZING GENE THERAPIES

**9:55 Chairperson's Remarks**
*James Richardson, PhD, Senior Director, Analytical Development, Interus BioTherapeutics*

**10:00 KEYNOTE PRESENTATION: Technical Development and Comparability Strategies for Gene Therapies**
*Phillip Ramsey, Senior Vice President, Head, Technical Development, Sangamo Therapeutics*
Viral-based gene therapy products are rapidly moving through the clinic for diseases with a high unmet medical need and offering a potential one-time treatment and effective cure. They move quickly through the development cycle with many changes requiring comparability assessment. This talk will focus on the evolution of analytical methods during this process, looking at several examples of method modifications and the correlation of orthogonal methods for comparability assessment.

**10:30 PANEL DISCUSSION: GENE THERAPY CMC LEADERS PANEL**
*Moderator: James Richardson, PhD, Senior Director, Analytical Development, Interus BioTherapeutics*

*Panelists:*

*Van M. Hoang, PhD, Senior Vice President, Head, Analytical & Quality Control, Spark Therapeutics Inc.*

*Phillip Ramsey, Senior Vice President, Head, Technical Development, Sangamo Therapeutics*

*James Warren, PhD, Senior Vice President, Pharmaceutical Development, Ultragenyx Pharmaceutical*

**11:30 Presentation to be Announced**

UNCHAINED LABS

**12:00 pm LUNCHEON PRESENTATION: Engineered Innovation: The Making-of DENARASE High Salt for High-Efficiency DNA Removal in Viral Vector Manufacturing**

c-LEcta A KERRY COMPANY

*Raphael Gübeli, Vice President , Marketing & Sales, c-LEcta GmbH*
c-LEcta now introduces DENARASE High Salt, a genetically engineered version of the Serratia marcescens endonuclease, which retains high activity at elevated salt concentrations.
In this presentation we will show you how we used our proprietary engineering platform ENESYZ for the development of DENARASE High Salt and provide more application details for this new enzyme.

**12:30 Session Break**

## COMPARABILITY AND CHARACTERIZATION

**12:50 Chairperson's Remarks**
*Santoshkumar L. Khatwani, PhD, Director, Analytical Development, Sangamo Therapeutics*

**12:55 Comparability Challenges and Opportunities for Late-Stage Gene Therapy Programs**
*Xiaohui Lu, PhD, Director, Analytical Development, Ultragenyx Pharmaceutical*
In this presentation, we explore the multifaceted comparability challenges facing late-stage gene therapy programs, including regulatory standards, analytical methodologies, and product consistency.

## SUPPORTIVE ANALYTICS AND QUALITY CONTROL

**1:25 Development of USP Standards to Support Gene Therapy Products**
*Anthony Blaszczyk, PhD, Senior Scientist, Global Biologics, US Pharmacopeia*
The complexity of gene therapy makes production and characterization challenging. These challenges are amplified by the lack of applicable reference standards. USP is developing physical and documentary standards to support gene therapy, with a focus on AAV. Several AAV-related standards are currently in development, with the intent of supporting manufacturers from production through release testing. These standards will focus on three areas—raw materials, impurities, and viral vector characterization.

**1:55 Presentation to be Announced**

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**2:10 Presentation to be Announced**

**2:25 Networking Refreshment Break**

**2:40 Advanced Analytics for Structure Activity Relationship Studies**
*Santoshkumar L. Khatwani, PhD, Director, Analytical Development, Sangamo Therapeutics*
This presentation will discuss emerging analytics for AAV characterization, including: early vs. late-state characterization; examples of emerging analytics, all supported by a case study.

**3:10 Characterizing AAV Quality Attributes & Process Contaminants Using Liquid Phase Separations Coupled to Mass Spectrometry**
*Jonathan Bones, PhD, Principal Investigator, Characterisation and Comparability Laboratory, National Institute for Bioprocessing Research and Training (NIBRT), Ireland*
AAV-based gene therapies present a considerable analytical challenge due to their molecular size and complexity. Strategies for the characterization of various quality attributes of AAV using liquid phase separations and mass spectrometry will be presented. Examples include the characterization of intact viral proteins using LC-MS and CE-MS, determination of the capsid full state using LC-MS, and charge-detection mass spectrometry for mass-based analysis of capsid fill state and heterogeneity.

**3:40 Session Break and Transition to Plenary Keynote Session**

## PLENARY KEYNOTE SESSION: SOLVING TODAY'S CHALLENGES

**4:20 Organizer's Remarks**
*Daniel Barry, Senior Conference Director, Cambridge Healthtech Institute*

**4:30 PLENARY KEYNOTE PRESENTATION: READY: Addressing Current Challenges in Biomanufacturing with Reliability, Efficiency, Agility, Data, and (High) Yields**
*Jerry A. Murry, PhD, Senior Vice President, Process Development, Amgen*
The biopharmaceutical sector is currently producing vast amounts of data, a trend set to amplify with smart sensors, PAT, and process automation. This presentation will highlight the significance of a holistic digital strategy, incorporating AI, machine learning, predictive modeling, and data visualization, to spearhead the evolution of biomanufacturing. Emphasizing enhanced efficiency and innovation, this strategy will enable the efficient manufacture of complex biologics with reliability of supply, agility, and differentiation.

**5:10 Plenary Q&A**

**5:30 Welcome Reception in the Exhibit Hall with Poster Viewing**

6:30 **Close of Day**

## TUESDAY, AUGUST 20

7:30 am **Registration and Morning Coffee**

### IN-DEPTH CHARACTERIZATION OF VIRAL VECTORS

7:55 **Chairperson's Remarks**
*Rajeev Boregowda, PhD, Associate Director, Bioassay and Molecular Analytical Development, Genomic Medicine CMC, Sanofi*

8:00 **Evaluating a Combinatorial UV-Vis/DLS/SLS Analytic Platform for Rapid, High-Throughput rAAV Quantification and Multi-Attribute Characterization**
*Xueyuan Liu, Director Research Vector Core, Pathology, Childrens Hospital of Philadelphia*
We evaluated the platform for AAV quantification and characterization, comparing it to established analytical methods. The platform offers empirical, data-driven measurements with minimal sample requirements. Upon testing hundreds of rAAV vectors comprising diverse serotypes and transgenes, the data showed strong correlations with established analytical methods and exhibited high reproducibility. Its capability also extends to in-process samples from various purification processes, meeting the demand for rapid, high-throughput analysis.

8:30 **Evaluation of Residual Host Cell DNA Clearance and Sizing during Production of a Lentiviral Vector**
*Elaine M. Youngman, PhD, Principal Scientist, Analytical Development, Interius Bio*

9:00 **Manufacturing Challenges and Control Strategies for Dual AAV Vectors**
*Christine Le Bec, PhD, Head, CMC Gene Therapy, Sensorion*
Sensorion is a biotech company dedicated to the development of therapies for genetic forms of hearing loss. Two novel gene therapy programs include deafness due to otoferlin deficiency as well as GJB2 mutation. Since the otoferlin gene is large and exceeds the AAV packaging capacity, two AAV vectors have been developed. The product manufacturing and a deep characterization of the dual vectors will be presented.

9:30 **Presentation to be Announced**

Texcell

10:00 **Coffee Break in the Exhibit Hall with Poster Viewing**

10:45 **Breakout Discussion Groups**
Breakout Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussions page on the conference website for a complete listing of topics and descriptions.

### POTENCY ASSAYS, IN-PROCESS TESTING

11:30 **Streamlined Approach to Potency During Clinical Development for AAV GT Products**
*Dorota A. Bulik, PhD, Senior Director, Pharmaceutical Development, Ultragenyx Pharmaceutical*
Development of a functional potency is one of the major analytical challenges facing the sponsors of the cell and gene therapy products. An MOA-based potency bioassay is expected to be a part of a release panel prior to the pivotal clinical studies. Strategies to streamline development of the potency assays for the rAAV therapeutics will be presented with the emphasis on building a toolbox and leveraging platform knowledge.

12:00 pm **In-Process Stability Testing with Novel AAV Capsid Variants**
*Seth Levy, PhD, Director, Bioprocess Development, Modalis Therapeutics*
Modalis Therapeutics employs novel AAV capsid variants with enhanced tissue targeting and transduction to deliver our gene modulation technology, known as Guide Nucleotide Directed Modulation (GNDM). Among the many challenges faced in process development is ensuring consistent product quality throughout process iterations. Here, we will delve into key factors affecting in-process product stability, different testing methodologies, and their implications on final product quality.

12:30 **Presentation to be Announced**

PENDOTECH

1:00 **Luncheon Presentation to be Announced**

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1:30 **Refreshment Break in the Exhibit Hall with Poster Viewing**

### FORMULATION, FORCED DEGRADATION, PTM ANALYSIS

2:10 **Chairperson's Remarks**
*DingJiang Dean Liu, PhD, Senior Director, Formulation Development, Regeneron Pharmaceuticals Inc.*

2:15 **Reducing Process Developmental Timelines with Rapid, High-Throughput Orthogonal Assays and a Robust Downstream Process**
*Srivatsan Ramesh, PhD, Scientist, Downstream Process Development, BridgeBio*
As the field of gene therapies evolves, there is an increasing demand for analytical techniques that are rapid and high-throughput to facilitate the quick iteration of process development. This presentation will explore the creation of a suite of straightforward yet robust analytical methods to expedite the characterization process, thereby shortening developmental timelines. Case studies illustrate how these technologies have reduced downstream process development timelines in response to changing bioreactor productivities.

2:45 **A Comprehensive Degradation Temperature Panel Is Vital for AAV Development**
*Ronald T. Toth, PhD, Senior Scientist, Characterization, Sanofi*
AAV pre-development testing has been minimal causing developability challenges that could be avoided. An understanding of where degradation transitions occur is vital before development begins. This talk introduces high-throughput, microplate-based methods to aid in the measurement of degradation transitions based on intrinsic and extrinsic fluorescence in addition to anisothermal dynamic light scattering, and shows how they have been used to solve developability challenges and increase our product understanding.

**Comprehensive PTM Analysis of AAV Product Insights from Forced Degradation Study**
***Jin Park, PhD, Associate Director, Ultragenyx***
Studies of the exposure of the AAV product to extreme conditions help in understanding the intrinsic stability of the molecule and the degradation pathways. Temperature changes, pH variations, and oxidation conditions were tested to evaluate exposure to harsh conditions. The impacts on critical quality attributes (CQAs) such as quantity, purity, and activity are summarized. The primary impacts observed were genome loss, aggregation, PTM changes, and loss of activity.

3:45 **Refreshment Break in the Exhibit Hall with Poster Viewing**

4:30 **Comparison of Vector Production Kinetics between Novel Dual Transfection and Traditional Triple Transfection Systems in Suspension HEK293 Cells**
***William Lee, Research Associate, AAV Analytical Method Development, Alexion-AstraZeneca Rare Disease***
The mAAVRx manufacturing process utilizes a 2-plasmid transient transfection along with several design optimizations to greatly increase bioreactor productivity while reducing packaged impurity levels. Transfected cell culture samples were collected over time to identify the potential effects that the 2-plasmid mAAVRx system had on AAV production. In-depth analytical characterization demonstrated several noteworthy differences in product quality and vector production kinetics between the two systems.

## NEXT-GENERATION SEQUENCING FOR VIRAL VECTORS

5:00 **Investigating the Impact of ITR Deletions in rAAV Production Plasmids on rAAV Vector Quality Using Next-Generation Sequencing**
***Michael Boyd, Senior Scientist II, Novartis***
The inverted terminal repeats (ITRs) are essential elements of the recombinant rAAV genome and are involved in several key steps in the AAV life cycle. The repetitive nature of these segments can create some challenges during development and in-depth characterization is of great importance. In this study, we employed next-generation sequencing to investigate the effects of ITR deletions at the plasmid level on AAV vector yield and genomic quality.

5:30 **Close of Gene Therapy CMC and Analytics Conference**

## WEDNESDAY, AUGUST 21

**7:30 am Registration and Morning Coffee**

### SCALING UP VIRAL VECTORS

**7:55 Chairperson's Remarks**
*Nathalie Clément, PhD, Vice President, Vector Development, Translational Gene Therapies, Siren Biotechnology*

**8:00 Strategy and Lessons Learned from Upstream Process Characterization of AAV Gene Therapy Products**
*Daniel C. Odenwelder, PhD, Senior Engineer III, Gene Therapy and Upstream Process Development, Biogen*
As the AAV gene therapy field continues to grow and mature, historical knowledge and insights from late-stage process characterization will serve as valuable guidance for improving future process development and control strategies. This talk will focus on upstream process characterization of an adherent transient transfection AAV production process. It will cover topics related to experimental strategy, scale down model validation, production robustness, and raw material variability.

**8:30 Switching from Adherent to Suspension? What We've Learned**
*Shaoying Wang, PhD, Senior Scientist, Upstream Process Development, Passage Bio*

**9:00 FEATURED PRESENTATION: Significance of Manufacturability Assessment during Novel AAV Capsid Early Discovery Process**
*Davide Gianni, PhD, Principal Scientist, Biogen*
To address some of the limitations emerging from the first generation of Adeno-Associated Virus (AAV)–based therapeutics, development of novel capsids with improved tropism for target tissues and reduced immune response is still a primary goal for the field. This presentation will focus on manufacturability assessment strategies to integrate at early stage in the capsid discovery process to improve the confidence in the selection of novel candidates with superior commercial viability.

**9:30 Development and Scale Up of Adenovirus Process in Adherent Cells—Case Study** BIOVIAN
*Monica Lazaro, Director, Business Development, Commercial, 3PBIOVIAN*
Since 2004, Biovian, now known as 3PBIOVIAN, has provided Viral Vector CDMO services to a global client base. Our site in Turku, Finland, holds the license for manufacturing of Viral Vector products for clinical trials and commercial use. Case study of iCELLis technology utilization in adenovirus production: Successful scaling up of adenovirus production in adherent cells in perfusion mode from single-use fixed-bed iCELLis 1.07 $m^2$ bioreactor to large scale 133 $m^2$ bioreactor.

**10:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**10:40 Data-Driven Robust Producer Cell Line Development Platform for AAV Gene Therapy**
*Amit Mathur, PhD, Senior Scientist, Genomic Medicine Unit, Sanofi*
This presentation will focus on the producer cell line process for AAV production, the front runner AAV production platform at Sanofi. The talk will highlight how automation can help build a robust cell line generation platform.

**11:10 Development, Optimization, and Scale-Up of an Upstream Process for the Production of an AAV Gene Therapy: Case Study**
*Julien Robitaille, Research Council Officer, Cell Culture Scale Up, National Research Council Canada*
Several adeno-associated virus (AAV)–based gene therapies have been approved in recent years and are providing benefits to patients with rare diseases. However, the price point limits their accessibility. Here we will focus on the use of scale-down models, high-throughput optimization methods, and the use of different transfection and cell culture additives to increase volumetric titers and obtain a cost-effective process for the development of AAV-based therapy for lipoprotein lipase deficiency.

**11:40 Process Development for Efficient and Scalable Production of FBX-101 AAV Gene Therapy for Patients with Krabbe Disease**
*Frank K. Agbogbo, PhD, Vice President, Process Development, Forge Biologics*
Krabbe disease is caused by mutations in the gene encoding the lysosomal enzyme galactocerebrosidase (GALC), which is essential for normal metabolism of myelin components. Forge Biologics has developed an efficient and scalable process to produce FBX-101 (rAAV expressing GALC) and scaled it under cGMP conditions. In this talk, data will be presented on process development at Forge Biologics to produce drug products for clinical trials for patients with Krabbe disease.

**12:10 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:40 Refreshment Break in the Exhibit Hall with Poster Viewing**

### OPTIMIZING PRODUCTIVITY

**1:25 Chairperson's Remarks**
*Johannes C.M. Van Der Loo, PhD, Director Clinical Vector Core, Perelman Center for Cellular & Molecular Therapeutics, Children's Hospital of Philadelphia*

**1:30 Optimizing Upstream Development—Alexion Case Study**
*Nick DiGioia, CMC Process Development, Alexion Genomic Medicines*
Implementation of a wide range of AAV capsid variants has provided a unique challenge to process development groups, as manufacturing attributes of the AAV differ drastically between serotypes. The Alexion team has developed a manufacturing process with the goal of improving the consistency of the productivity and the quality of AAV produced in the bioreactor, as well as providing flexibility in the purification process to handle performance differences between serotypes.

**2:00 PANEL DISCUSSION: Optimizing Viral Vector Process Development**
*Moderator: Johannes C.M. Van Der Loo, PhD, Director Clinical Vector Core, Perelman Center for Cellular & Molecular Therapeutics, Children's Hospital of Philadelphia*
*Panelists:*
*Xiaozhi Ren, PhD, Director, Plasmid and Cell Line Development, Nvelop Therapeutics*
*David McNally, Director, Process Development, MassBiologics*

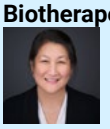
**2:30 Presentation to be Announced** ChromaTan

**3:00 Refreshment Break in the Exhibit Hall with Poster Viewing**

## PLENARY FIRESIDE CHAT: LEADING TO TOMORROW'S ADVANCES

**3:50 Organizer's Remarks**
*Nandini Kashyap, M.Pharm., Senior Director, Conferences and Social Media Strategy, Cambridge Innovation Institute*

**4:00 Genetic Medicines—Transforming the Future of Biotherapeutics**




*Moderator: Ann Lee, PhD, CTO, Prime Medicine, Inc.*
*Panelists:*
*E. Morrey Atkinson, PhD, Executive Vice President, Chief Technical Operations Officer, Head, Biopharmaceutical Sciences and Manufacturing Operations, Vertex Pharmaceuticals Inc.*
*Manmohan Singh, PhD, CTO, Beam Therapeutics*
*Heidi Zhang, PhD, Executive Vice President, Head, Technical Operations, Tune Therapeutics*

**5:00 Networking Reception in the Exhibit Hall with Poster Viewing**

**6:00 Close of Day**

## THURSDAY, AUGUST 22

**7:30 am Registration and Morning Coffee**

## VIRAL VECTOR PRODUCTION

**7:55 Chairperson's Remarks**
*Susan D'Costa, PhD, CTO, Genezen*

**8:00 Understanding Large-Scale Transient Transfection Process Using CFD Models in Manufacturing of Gene Therapy Viral Vectors**
*Mukesh Mayani, PhD, Head of Process Development, Gene Therapy, National Resilience, Inc.*
Large-scale (≥200L) transient transfection is susceptible to high variability, hindering achievement of consistent vector titer and quality. This process is characterized through a scale-down model, which lacks true comprehension of mixing behavior at-scale. We studied various mixing attributes using 20L RM bags, employing computational fluid dynamics (CFD) and colored dye experiments. This provided crucial insight and deeper understanding into mixing patterns and intricate interplay of transfection attributes on vector titers.

**8:30 Development, Optimization, and Scale-Up of Suspension Vero Cell Culture Process for High Titer Production of Oncolytic Herpes Simplex Virus-1**
*Martin Loignon, PhD, Team Leader, Cell Engineering, National Research Council Canada*
Adherent Vero cell platforms are approved for manufacturing of human viral vaccines, but their use is labor-intensive and costly. We have improved cost-effectiveness by developing a chemically-defined media and adapted Vero cells in suspension culture to simplify sub cultivation and process scale-up. We obtained $2.7\times10^{8}$ TCID50 $mL^{-1}$ in a 3L batch process and $1.1\times10^{9}$ TCID50 $mL^{-1}$ in a perfusion culture for HSV-1 and competitive titers for other viruses.

**9:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**9:30 Advancing AAV Production with TruStable Cell Line Technology**
*Sandhya Pande, PhD, Associate Director, Cell Sciences, Shape Therapeutics Inc.*
TruStable Cell Line Technology Platform is a serum-free suspension human cell line engineered for stable rAAV production. This flexible system packages diverse payloads into a variety of capsid serotypes with high titer and exceptional packaging. Polyclonal pools demonstrate productivity at ~8e14 vg/L and ~70% full when measured from crude extracts. Monoclonal derivatives exhibit outstanding productivity, often exceeding 100,000 viral genomes/cell. The TruStable platform is readily scalable for large-scale manufacturing.

## PROCESS INTENSIFICATION FOR VIRAL VECTORS

**10:00 Process Intensification Approach for High-Yield rAAV Vector Production in Suspension Cell Culture of Mammalian Cell Line**
*Pranav Joshi, PhD, Associate Director, Upstream Process Development, University of Pennsylvania*
High-yield production of rAAV is of pivotal importance yet it remains a critical challenge in current times. Classic rAAV vector manufacturing processes based on transient transfection are limited to low cell density suspension cell culture of mammalian cell lines. By alleviating limitations related to high cell density transfection step and cell culture productivity via process intensification strategies, we achieved improved rAAV production yields with consistent vector quality.

**10:30 FEATURED PRESENTATION: Continuous Downstream Purification of Viral Vectors**
*Caryn L. Heldt, PhD, Professor, Chemical Engineering, Michigan Technological University*
Continuous manufacturing and purification is a key to reducing the cost of viral gene therapies. Our focus is on continuous downstream. We have developed an end-to-end continuous purification based in aqueous two-phase systems (ATPS). Recoveries of 66-100% have been found with four different virus models. We have also developed an AFM analytical method to study empty and full AAV vectors, providing new information for downstream separation of AAV vectors.

## OPTIMIZING DOWNSTREAM PROCESSING

**11:00 KEYNOTE PRESENTATION: Evolution of Downstream Manufacturing Process Design for Productivity, Product Quality, and Process Consistency**
*Mi Jin, PhD, Head, Downstream and Drug Product Development, Spark Therapeutics Inc.*

**11:30 Presentation to be Announced**
GYROS PROTEIN Technologies

**12:00 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:30 Refreshment Break in the Exhibit Hall & Last Chance for Poster Viewing**

## ADVANCING DOWNSTREAM PROCESSING

**1:05 Chairperson's Remarks**
*Meisam Bakhshayeshi, PhD, Senior Director, Process Development, Obsidian Therapeutics*

**1:10 Accelerating Downstream Process Development of Gene Therapy Products for a Commercial-Ready Platform**

***Rashmi Bhangale, PhD, Senior Scientist, Downstream Process Development, Asklepios BioPharmaceutical Inc.***

Despite numerous advances in the field of gene therapy, the efficiency and cost of producing rAAV drug products to meet a rapidly-growing industry has significant room for improvement. At AskBio, we have developed a platform-based approach for downstream process development that we believe has resulted in highly scalable and GMP-ready processes. This has accelerated the process development timelines while considerably reducing overall costs of vector production.

**1:40 Innovations in Downstream AAV Purification**

***Ohnmar Khanal, PhD, Downstream Technology Lead, Downstream Purification and Drug Product Development, Spark Therapeutics***

This talk will demonstrate chromatographic and non-chromatographic approaches to AAV capsid separation, enrichment, and stabilization. The impact of resin geometry, chemistry, kosmotropic buffer agents, and metal ions will be illustrated. Innovative tools such as mechanistic models and multicolumn chromatography are applied to AAV separation. Using these strategies, we demonstrate > 90% empty capsid removal with a yield of > 80%.

**2:10 Emerging AAV Technologies and Program Strategies for the Acceleration of Rare Disease Applications**

***Kenneth Yancey, Senior Director, Downstream Process Development, University of Pennsylvania***

The field of gene therapy has shown the potential to change the paradigm of medicine but faces challenges in the areas of high cost of goods, limited access, challenges to insurance reimbursement, and challenging commercial models, especially for rare disease. This talk focuses on recent advancements in the field and technical development approaches for clinical and commercial success. Topics include emerging technology, development approaches for AAV, program-specific issues, and common hurdles.

**2:40 Networking Refreshment Break and Transition into Town Hall Discussions**

## FACILITATED TOWN HALL DISCUSSIONS

**2:55 Facilitated Town Hall Discussions**

These Town Halls offer delegates the opportunity to participate in interactive discussions on important themes that were explored during the conference. Each Hall will have a host(s) to facilitate the conversation, and all are welcome to participate, share views and best practices and ask questions of colleagues.

**Harnessing ML/AI and Big Data for Biotherapeutic Development**

***Pin-Kuang Lai, PhD, Assistant Professor, Department of Chemical Engineering and Materials Science, Stevens Institute of Technology***

**Cell and Gene Therapy Manufacturing: In-House vs. Outsourced**

***Elben Guimaraes, Senior Manufacturing Manager, Upstream Manufacturing, Ultragenyx Pharmaceutical Inc.***

The decision of handling cell and gene therapy processes in-house or outsourcing them is crucial. This facilitated discussion explores the advantages and challenges of both approaches, analyzing their impact on cost, control, strategic direction, and innovation. Share experiences and best practices for managing internal and external manufacturing, while examining common scenarios faced by sponsors and vendors.

**Digital Transformation & AI in Bioprocess—What, Where, When, and How?**


***Christian Airiau, PhD, Global Head, Data Sciences, CMC, R&D, Sanofi***
***Irene Rombel, PhD, CEO & Co-Founder, BioCurie Inc.***

The bioprocessing industry is undergoing a digital revolution fueled by AI. This interactive session dives into current digital adoption and explores the latest trends in AI applications (AIML). Join the conversation to explore the potential of AI for process optimization and digital twins. Share real-world success stories and discuss ethical considerations along with potential workforce impacts.

**3:55 Close of Summit**

# STREAM #4
# CELL THERAPY

The Cell Therapy stream explores the critical challenges facing the manufacture, analysis and quality of cell-based therapies across clinical and commercial development. Featuring two back-to-back conferences, Cell Therapy CMC and Analytics, and Cell Therapy Manufacturing, topics include product and process characterization, CMC strategies, decentralized manufacturing, autologous and allogeneic manufacturing strategies, automation, the role of AI, scale-up and supply of CAR Ts and next-generation cell therapies such as NK cells, TILs, iPSCs, gamma deltas, and TCR-based therapies.

## Conference Programs

**AUGUST 19-20**

Cell Therapy CMC and Analytics

View Program »

**AUGUST 21-22**

Cell Therapy Manufacturing

View Program »

# Cell Therapy CMC and Analytics

*Improving Product and Process Quality Across All Stages of Development*

**AUGUST 19-20**
All Times EDT

## MONDAY, AUGUST 19

**8:00 am Registration and Morning Coffee**

### ADVANCING CELL THERAPY CMC

**9:55 Chairperson's Remarks**
*Mo Heidaran, PhD, Head, Translational and Regulatory Strategy, GC Therapeutics; Former FDA Reviewer*

**10:00 Regulatory Science and Translational Research in Cell Therapy Development**
*Steven R. Bauer, PhD, Chief Regulatory Science Affairs Program Officer, Wake Forest Institute for Regenerative Medicine (WFIRM)*
Understanding the goals of regulatory science is a crucial first step in the development of strategies that will satisfy FDA cell therapy CMC regulatory requirements appropriate for each stage of the IND and BLA process. This talk will illustrate potential strategies to improve translation of laboratory findings into rigorous and predictive science that improves cell therapy characterization to fulfill regulatory expectations regarding potency, purity, and comparability.

**10:30 Developing Cell Therapies at Dana Farber**
*Felicia Ciuculescu, MD, Director, Technology Transfer, Cell Manipulation Core Facility, Dana Farber Cancer Institute*

### ANALYTICAL DEVELOPMENT

**11:00 Standardization Efforts for Analytical Methods**
*Laura Pierce, Biomedical Engineer, Biosystems & Biomaterials, NIST*
Cellular therapy products (CTPs) require high quality, robust, and validated analytical methods. In recent years, several NIST-led ISO standards have been developed that address common testing needs for CTPs including cell characterization and count, and current efforts aim to develop a cell viability standard. Here, we describe the recently published and upcoming standards and the cell-counting COMET application, and give practical examples for the development of fit-for-purpose analytical methods.

**11:30 Presentation to be Announced**

**12:00 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:30 Session Break**

### PROCESS ANALYTICAL TECHNOLOGY, FLOW CYTOMETRY

**12:50 Chairperson's Remarks**
*Zhimei Du, PhD, CSO, BlueSphere Bio*

**12:55 Real-Time, Automated Monitoring of CAR T Cell Phenotypes in Autologous Cell Therapy Manufacturing via Holographic Imaging**
*Sarah Rajani, Scientist, Cell Therapy Drug Product Process Development, Bristol Myers Squibb*
The implementation of Process Analytical Technologies (PAT) for cell therapies is needed to further process product understanding; however, few technologies enable real-time, continuous monitoring of product quality attributes. We utilize in-line holographic imaging through experimental, computational vision and machine learning techniques to predict multiple CAR T cell phenotypic attributes within the expected measurement variability of offline analytical methods. This approach enables phenotype monitoring across a variety of processing modalities.

**1:25 NIST Flow Cytometry Standards Consortium Enables Quantitative and Comparable Measurements for Cell and Gene Therapies**
*Lili Wang, PhD, Research Chemist, Biomarker & Genomic Sciences Group, NIST*
Flow cytometry assays have been used to measure critical quality attributes, including viability, identity, purity, and potency of cellular therapeutic products. However, the lack of result comparability remains a significant challenge. NIST launched Flow Cytometry Standards Consortium by providing metrology and standards development expertise to work with the consortium members and stakeholders for developing measurement solutions and standards needed to accelerate translation, manufacturing, and approval of cell and gene therapies.

**1:40 Overcoming Challenges in Using Multi-Parameter Flow Cytometry in Cell and Gene Therapy**
*Caraugh Albany, PhD, Research Scientist, Analytical Development, Autolus Therapeutics plc*
Flow cytometry is a crucial analytical tool for in-process monitoring, release of cell therapy drug products, and clinical monitoring of patients. Despite its prevalent use, its complex design and high-dimensional capabilities require substantial consideration during development of flow-based analytical procedures and also for subsequent routine execution in GMP settings. Herein, we will discuss the current flow cytometry-related guidelines, challenges, and approaches to overcome them.

**1:55 Sponsored Presentation** *(Opportunity Available)*

**2:25 Networking Refreshment Break**

### ENSURING PRODUCT QUALITY

**2:40 Quality Considerations for Plasmid DNA as a Raw Material**
*Ben Clarke, PhD, Senior Scientist, USP*
USP is continuing to develop reference standards, informational chapters, and compendial analytical methods to safeguard raw, starting, and ancillary materials for cell therapies. USP's standards give best practice guidance to developers and manufacturers, simplify risk assessments, accelerate analytical development, and support raw material qualification and release. This presentation will describe existing standards and USP's recent development related to plasmid DNA and rapid microbial methods.

**3:10 Adventitious Agent Controls for Biological Raw Materials**
*Christopher Bravery, PhD, Consulting Regulatory Scientist, Advanced Biologicals Ltd.*
Compared to other medicinal products, cell therapy products (including gene-modified) tend to use a lot of biological raw materials. These can be human, animal, microbial, or even plant-derived. Without understanding how these materials are made, it is not possible to ensure their adventitious agent risks are addressed. Using real examples, this talk will discuss the principles and how to assess and mitigate the identified risks.

**3:40 Session Break and Transition to Plenary Keynote Session**

### PLENARY KEYNOTE SESSION: SOLVING TODAY'S CHALLENGES

**4:20 Organizer's Remarks**
*Daniel Barry, Senior Conference Director, Cambridge Healthtech Institute*

**4:30 PLENARY KEYNOTE PRESENTATION: READY: Addressing Current Challenges in Biomanufacturing with Reliability, Efficiency, Agility, Data, and (High) Yields**

*Jerry A. Murry, PhD, Senior Vice President, Process Development, Amgen*

The biopharmaceutical sector is currently producing vast amounts of data, a trend set to amplify with smart sensors, PAT, and process automation. This presentation will highlight the significance of a holistic digital strategy, incorporating AI, machine learning, predictive modeling, and data visualization, to spearhead the evolution of biomanufacturing. Emphasizing enhanced efficiency and innovation, this strategy will enable the efficient manufacture of complex biologics with reliability of supply, agility, and differentiation.

**5:10 Plenary Q&A**

**5:30 Welcome Reception in the Exhibit Hall with Poster Viewing**

**6:30 Close of Day**

## TUESDAY, AUGUST 20

**7:30 am Registration and Morning Coffee**

## POTENCY ASSAYS FOR CELL AND GENE THERAPIES

**7:55 Chairperson's Remarks**

*Christopher Bravery, PhD, Consulting Regulatory Scientist, Advanced Biologicals Ltd.*

**8:00 Considerations for Potency Assurance of Cellular and Gene Therapy Products**

*Diana Colleluori, PhD, MBA, Consultant, CMC Analytical, Biologics Consulting Group*

The regulatory expectations for potency of CGTs remain critical to consider during drug development. A review of the recent FDA guidance will be discussed, along with considerations to reduce risk with respect to potency assurance. Significant challenges will be encountered during potency assay development. While the assessment of potency may change over time, it is imperative that potency assays are developed incrementally and in parallel with clinical development activities.

**8:30 Potency Assay Matrix for a Complex Multimodal Autologous Cell Therapy**

*Damian Marshall, PhD, Vice President, Analytical Development, Resolution Therapeutics*

Developing potency assays and demonstrating that they measure appropriate biological activities has long been a significant challenge, particularly with the evolving regulatory landscape. But what if you are developing a pioneering new therapy with a multimodal mechanism-of-action? This presentation will showcase the challenges of developing a potency assay matrix for an engineered macrophage therapy and will consider how these assays support future commercial manufacturing strategies.

**9:00 Early Correlation Studies between Potency Quality Attributes and Characterization Assays—Are They Critical in Design of Potency Assays for Release of Cell Therapy Products?**

*Pavan Puligujja, PhD, Director, Analytical Development, Adicet Bio*

A well-designed potency assay ensures lot-to-lot consistency of cellular drug products and contributes to the reliability of the drug development process. Identifying and understanding the correlation between multiple cell product potency critical attributes and release assays designed to monitor them at an early stage has many benefits. Early commitment to these potency assurance strategies reduces the risk of poor potency assay design, accelerates product development, and avoids regulatory hold-ups.

**9:30 Sponsored Presentation** *(Opportunity Available)*

**10:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**10:45 Breakout Discussion Groups**

Breakout Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussions page on the conference website for a complete listing of topics and descriptions.

## ANALYTICS FOR EMERGING CELL THERAPIES

**11:30 Analytical Strategies for Cell-Based Therapies: B Cell Medicines**

*Lisa Bradbury, PhD, Senior Director, Analytical Development, Be Bio*

Precision genome editing can now be used to engineer B cells that produce therapeutic proteins of interest, driving a new class of cellular medicines—Engineered B Cell Medicines (BCMs)—with the potential to be durable, allogeneic, redosable, and administered without pre-conditioning. The promise of BCMs could transform therapeutic biologics with broad application—across protein classes, patient populations, and therapeutic areas. Analytical strategies will be discussed.

**11:50 Process Development of Interneuron Cell Therapy for Mesial Temporal Lobe Epilepsy (MTLE)**

*Michael W. Watson, PhD, Associate Director, Assay Development & Quality Control, Neurona Therapeutics*

Neurona Therapeutics is a clinical-stage biotherapeutics company developing an allogeneic GABAergic inhibitory interneuron cell therapy candidate (NRTX-1001) for drug-resistant MTLE. NRTX-1001 clinical product is manufactured at Neurona's cGMP facility, cryopreserved, and delivered to the clinic for MRI-guided deposition into the seizure-onset region of the temporal lobe. Analytical development that underpins manufacturing of NRTX-1001 and early first-in-human clinical data from the ongoing open-label Phase 1/2 study (NCT05135091) will be discussed.

**12:10 pm Quantitative Label-Free Imaging of Individual iPSCs for Monitoring Cell Behavior and Pluripotency**

*Anthony Asmar, PhD, Biologist, National Institute of Standards and Technology*

The ability to quantitatively image induced pluripotent stem cells (iPSC) to monitor their dynamic and spatial behavior and state of pluripotency in a non-invasive manner is important for establishing better metrics for pluripotency and to assure consistency and efficiency in iPSC manufacturing.

**12:30 Sponsored Presentation** *(Opportunity Available)*

**1:00 Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**1:30 Refreshment Break in the Exhibit Hall with Poster Viewing**

## EFFECTIVE DUE DILIGENCE AND TECH TRANSFER

**2:10 Chairperson's Remarks**

*Scott R. Burger, Principal, Advanced Cell & Gene Therapy LLC*

**2:15 Effective Due Diligence for CGT Products and Technology**
*Scott R. Burger, Principal, Advanced Cell & Gene Therapy LLC*
Errors in due diligence often delay or derail development of CGT products. Expectations based on drugs and biologics overlook challenges of CGT development, particularly when evaluating products developed in academic settings. This presentation will discuss key points for big pharma/investors to investigate and identify CGT products ready for further development and obstacles to successful development; data biotech start-ups/academic researchers need to show to make a compelling case in due diligence.

**2:45 Effective Technology Transfer—It's Not a One-Way Street**
*William E. Janssen, PhD, Principal, WEJ Cell & Gene Therapy Consulting Services LLC*
This session is about nurturing the seeds of a good CGT concept to a healthy plant, ready to bear the fruit of early-phase clinical study. Seeds and young plants need fertilizer (funding), planting (tech transfer), and proper gardening tools. This talk will address effective communication between research laboratories (seed planters) and early-phase CDMOs (plant tenders), to arrive at manufacturing methods designed to continue growth to full commercialization.

**3:15 Technology Development and Partnering in Cell Therapy Manufacturing**
*Akihiro Shimosaka, PhD, Chairman, Asian Cellular Therapy Organization (ACTO)*

**3:45 Refreshment Break in the Exhibit Hall with Poster Viewing**

## HIGHER-ORDER STRUCTURES, CRYOPRESERVATION

**4:30 Modifications (PTMs) and Higher-Order Structures (HOS) of Proteins: Analysis, Contributing Factors, and Effects on Gene and Cell Therapies (GCT)**
*Sunny Zhou, PhD, Professor, Chemistry & Chemical Biology, Northeastern University*
Proteins are subjected to numerous modifications (PTMs), such as degradation, oxidation, and crosslinking. Moreover, higher-order structures (HOS) also play critical roles. In this talk, their analyses and contributing factors—such as reactive metabolites and cell culture changes—will be discussed, as well as the potential effects (e.g., immunogenicity and off-target binding). One pertinent issue is the chemical and physical degradation during cryopreservation of cells; and potential remediations are proposed.

**5:00 Cryopreservation of Cell Therapies**
*Yuechen Zhu, PhD, Process Development Scientist, Gene & Cell Therapy Process Development, Bayer Healthcare Pharmaceuticals*
This presentation will discuss optimizing containers, volumes, and formulations-dependent freezing profile to maximize yield on cryopreservation; best practices on the cell bioprocess from freezing through thawing, to maintain consistent cell viability; post-thaw cell characterization (ex: apoptotic markers) for process development and to reduce cell death; novel methods on cryopreservation for future cell therapies.

**5:30 Close of Cell Therapy CMC and Analytics Conference**

## WEDNESDAY, AUGUST 21

**7:30 am Registration and Morning Coffee**

### POC: IMPROVING AFFORDABILITY AND ACCESS OF CELL THERAPIES

**7:55 Chairperson's Opening Remarks**

*Patrick J. Hanley, PhD, Associate Professor, Pediatrics; Chief & Director, Cellular Therapy Program, Children's National Hospital*

**8:00 KEYNOTE PRESENTATION: Improving Affordability and Access of CAR T Cell and Other Gene-Modified Cell Therapies**

*Boro Dropulic, PhD, Co-Founder & Executive Director, Caring Cross*

A one-time commercial CAR T cell therapy costs at least $350,000 per dose, not including hospital expenses. Unfortunately, this expensive treatment is not affordable for everyone, especially in low- and middle-income countries. Caring Cross is working with hospitals to develop and improve local production of CAR T and other gene-modified cellular therapies. By producing these products locally, the cost will be significantly reduced, making them more accessible to patients.

**8:30 KEYNOTE PRESENTATION: Point-of-Care Manufacturing at an Academic Center: Increasing Accessibility to Cell Therapies**

*Nirav N. Shah, MD, Associate Professor, Hematology, Medical College of Wisconsin*

This talk will focus on the different models and potential benefits of point-of-care or decentralized manufacturing models for CAR T cell therapy. Dr. Shah will also discuss his single-center outcomes utilizing the CliniMACS Prodigy for point-of-care CAR manufacturing within an academic center to advance novel CAR constructs in B cell malignancies.

**9:00 PANEL DISCUSSION: Point-of-Care Manufacturing**

*Moderator: Patrick J. Hanley, PhD, Associate Professor, Pediatrics; Chief & Director, Cellular Therapy Program, Children's National Hospital*

*Panelists:*

*Boro Dropulic, PhD, Co-Founder & Executive Director, Caring Cross*

*Nirav N. Shah, MD, Associate Professor, Hematology, Medical College of Wisconsin*

**9:30 Sponsored Presentation** *(Opportunity Available)*

**10:00 Coffee Break in the Exhibit Hall with Poster Viewing**

### MANUFACTURING CELL THERAPIES FOR AUTOIMMUNE DISORDERS

**10:35 Chairperson's Remarks**

*Ravi Bhatia, Scientific Director, Cell Technology, Johnson & Johnson Pharmaceutical R&D*

**10:40 FEATURED PRESENTATION: Successful Generation of Anti-CD19 CAR T Cells for Clinical Use in Patients with Diverse Autoimmune Disorders**

*Ranjita Sengupta, PhD, Senior Director, Process Development, CMC Lead, KYV-101C, Kyverna Therapeutics Inc.*

There is an increasing interest in B cell-targeting CAR T cell therapies in B cell-mediated autoimmune disease. CAR T therapy is well established in hematology-oncology. One of the challenges in autologous CAR T therapy in oncology is manufacturability because of the cell health from very sick cancer patients. Here we explore manufacturability of KYV-101, a fully humanized anti-CD19 CAR T cell therapy from clinical patients with diverse autoimmune disease.

### AUTOMATION AND CLOSED SYSTEMS

**11:05 Roadmap towards Fully Automated Cell Therapy Manufacturing**

*Claire State, Scientist, Drug Product Process Development, Bristol Myers Squibb*

Current cell therapies' workflows are highly manual and generally consist of islands-of-automation. As the cell therapy industry continues to scale, novel manufacturing technologies are needed to automate, integrate, and streamline these complex workflows. This talk will discuss current technology limitations, opportunities for improvement, and product lifecycle stage-specific strategies to manage these changes in manufacturing.

**11:35 A Closed, Autologous Bioprocess Optimized for TCR T Cell Therapies**

*Eugenia Zah, Process Development Principal Scientist, Amgen Inc.*

Autologous cell therapies for solid tumors are on the horizon, however the high cost and complexity of manufacturing these therapies remain a challenge. We have developed a fully closed, autologous bioprocess for generating MAGE-B2-specific TCR-expressing T cells, with enriched memory T cell phenotype and enhanced metabolic fitness. This bioprocess supports scale-out feasibility by enabling the processing of multiple patients' batches in parallel within a Grade C cleanroom.

**12:05 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:40 Refreshment Break in the Exhibit Hall with Poster Viewing**

### MANUFACTURING CELL THERAPIES

**1:25 Chairperson's Remarks**

*Ruud Hulspas, PhD, Technical Director, Process Development, Dana-Farber Cancer Institute*

**1:30 Selection of Specific T Cell Populations in Manufacturing Therapeutics Cells**

*Ruud Hulspas, PhD, Technical Director, Process Development, Dana-Farber Cancer Institute*

Due to the need for robust manufacturing large numbers of specific T cells, conventional GMP-compliant cell selection methods such as centrifugation, directed cell culture, and magnetic field-based selection are no longer suitable. Purification of specific T cells by multiparameter cell sorting is a well-established procedure in research, but the technique is difficult to scale up and generally lacks robustness and safety. We present requirements and status quo of this technique.

**2:00 The (Re)emerging Field of Xenotransplantation**
***Knut Niss, PhD, CTO, eGenesis, Inc.***
Through our transformative research, we are developing HuCo organs and cells to meet the increasing need. Our eGenesis Genome Engineering and Production (EGEN) platform leverages advances in gene editing technologies to address the historical challenges of xenotransplantation.

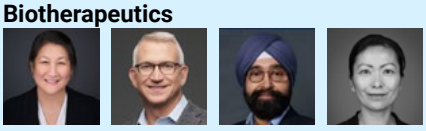
**2:30 Sponsored Presentation** *(Opportunity Available)*

**3:00 Refreshment Break in the Exhibit Hall with Poster Viewing**

## PLENARY FIRESIDE CHAT: LEADING TO TOMORROW'S ADVANCES

**3:50 Organizer's Remarks**
***Nandini Kashyap, M.Pharm., Senior Director, Conferences and Social Media Strategy, Cambridge Innovation Institute***

**4:00 Genetic Medicines—Transforming the Future of Biotherapeutics**



***Moderator: Ann Lee, PhD, CTO, Prime Medicine, Inc.***
***Panelists:***
***E. Morrey Atkinson, PhD, Executive Vice President, Chief Technical Operations Officer, Head, Biopharmaceutical Sciences and Manufacturing Operations, Vertex Pharmaceuticals Inc.***
***Manmohan Singh, PhD, CTO, Beam Therapeutics***
***Heidi Zhang, PhD, Executive Vice President, Head, Technical Operations, Tune Therapeutics***

**5:00 Networking Reception in the Exhibit Hall with Poster Viewing**

**6:00 Close of Day**

## THURSDAY, AUGUST 22

**7:30 am Registration and Morning Coffee**

## VIRAL VECTOR PRODUCTION

**7:55 Chairperson's Remarks**
***Susan D'Costa, PhD, CTO, Genezen***

**8:00 Understanding Large-Scale Transient Transfection Process Using CFD Models in Manufacturing of Gene Therapy Viral Vectors**
***Mukesh Mayani, PhD, Head of Process Development, Gene Therapy, National Resilience, Inc.***
Large-scale (≥200L) transient transfection is susceptible to high variability, hindering achievement of consistent vector titer and quality. This process is characterized through a scale-down model, which lacks true comprehension of mixing behavior at-scale. We studied various mixing attributes using 20L RM bags, employing computational fluid dynamics (CFD) and colored dye experiments. This provided crucial insight and deeper understanding into mixing patterns and intricate interplay of transfection attributes on vector titers.

**8:30 Development, Optimization, and Scale-Up of Suspension Vero Cell Culture Process for High Titer Production of Oncolytic Herpes Simplex Virus-1**
***Martin Loignon, PhD, Team Leader, Cell Engineering, National Research Council Canada***
Adherent Vero cell platforms are approved for manufacturing of human viral vaccines, but their use is labor-intensive and costly. We have improved cost-effectiveness by developing a chemically-defined media and adapted Vero cells in suspension culture to simplify sub cultivation and process scale-up. We obtained $2.7\times10^{8}$ TCID50 $mL^{-1}$ in a 3L batch process and $1.1\times10^{9}$ TCID50 $mL^{-1}$ in a perfusion culture for HSV-1 and competitive titers for other viruses.

**9:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**9:30 Breakout Discussion Groups**
Breakout Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussions page on the conference website for a complete listing of topics and descriptions.

## PROCESS DEVELOPMENT FOR NON-T CELL THERAPIES

**10:30 Promises and Challenges of Bioprocessing for PSC-Derived NK Cell Therapies**
***Allen Qiang Feng, PhD, Founder and CSO, HebeCell Corp.***
Human pluripotent stem cells (PSCs) offer an unlimited cell source for cell therapies. Major challenges are (1) complexity of bioprocessing, and (2) outdated regulatory guidelines. HebeCell's proprietary protoNK platform is a first-in-class technology enabling large-scale PSC-derived NK cell production. To translate protoNK platform into clinic, we have (1) successfully established internal manufacturing capability, and (2) identified disease indication. The unique process to manufacture protoNK also eliminates PSC contamination.

**11:00 NK and CAR-NK Processing Development**
***Dongfang Liu, PhD, Associate Professor, Director Immunoassay Development, Pathology & Immunology & Lab Medicine, Rutgers University***
Currently available technologies for expanding NK and CAR-NK cells using feeder cells (e.g., K562 cells) and cytokines (e.g., IL-2 ) are invaluable. However, these NK and CAR-NK expansion technologies show several limitations. Previous studies show that a 721.221-mIL21 as a feeder cell can rapidly expand NK and CAR-NK. Based on this technology, we developed a novel, non-feeder cell system to expand NK and CAR-NK cells *in vitro*.

**11:30 Sponsored Presentation** *(Opportunity Available)*

**12:00 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:30 Refreshment Break in the Exhibit Hall & Last Chance for Poster Viewing**

## AI/ML AND DIGITAL TWIN APPROACHES IN CELL THERAPY MANUFACTURING

**1:05 Chairperson's Remarks**
***Wei Xie, PhD, Assistant Professor, Mechanical & Industrial Engineering, Northeastern University***

**1:10 The Role of AI/ML in Cell Therapy Manufacturing**
***Wei Xie, PhD, Assistant Professor, Mechanical & Industrial Engineering, Northeastern University***
The rapidly expanding market for regenerative medicines and cell therapies highlights the need to advance the understanding of cellular metabolisms, improve the prediction of cultivation production processes, and support large-scale manufacturing of human induced pluripotent stem cells (iPSCs). A novel Biological System-of-Systems (Bio-SoS) model and risk-based PAT framework is proposed to model cell-to-cell interactions, spatial and metabolic heterogeneity, and cell response to micro-environmental variation.

**1:40 Feedback Control and Automation Integration for Cell Therapy Manufacturing**
***Bryan Wang, PhD, Senior Scientist, TreeFrog Therapeutics***
To address manufacturing challenges of cell therapies regarding product yield, quality, and reproducibility, we designed a digital twin-enabled closed-loop manufacturing platform with automation and feedback controls. This platform integrates process analytical technologies to enable deeper process understanding and provide real-time control of process variables. The digital twin-enabled bioreactor platform was shown to reduce costs, labor, time, and, more importantly, perturbations, and could improve yield while maintaining the quality of the products.

**2:10 Digital Twin for Cell Growth Modeling of Cell Therapies**
***Keshav Patil, PhD, Scientist, Advanced Therapies, Janssen Pharmaceuticals Inc.***

**2:40 Networking Refreshment Break and Transition into Town Hall Discussions**

## FACILITATED TOWN HALL DISCUSSIONS

**2:55 Facilitated Town Hall Discussions**
These Town Halls offer delegates the opportunity to participate in interactive discussions on important themes that were explored during the conference. Each Hall will have a host(s) to facilitate the conversation, and all are welcome to participate, share views and best practices and ask questions of colleagues.

**Harnessing ML/AI and Big Data for Biotherapeutic Development**
***Pin-Kuang Lai, PhD, Assistant Professor, Department of Chemical Engineering and Materials Science, Stevens Institute of Technology***

**Cell and Gene Therapy Manufacturing: In-House vs. Outsourced**
***Elben Guimaraes, Senior Manufacturing Manager, Upstream Manufacturing, Ultragenyx Pharmaceutical Inc.***
The decision of handling cell and gene therapy processes in-house or outsourcing them is crucial. This facilitated discussion explores the advantages and challenges of both approaches, analyzing their impact on cost, control, strategic direction, and innovation. Share experiences and best practices for managing internal and external manufacturing, while examining common scenarios faced by sponsors and vendors.

**Digital Transformation & AI in Bioprocess—What, Where, When, and How?**

***Christian Airiau, PhD, Global Head, Data Sciences, CMC, R&D, Sanofi***
***Irene Rombel, PhD, CEO & Co-Founder, BioCurie Inc.***
The bioprocessing industry is undergoing a digital revolution fueled by AI. This interactive session dives into current digital adoption and explores the latest trends in AI applications (AIML). Join the conversation to explore the potential of AI for process optimization and digital twins. Share real-world success stories and discuss ethical considerations along with potential workforce impacts.

**3:55 Close of Summit**

# STREAM #5

## mRNA MANUFACTURING & DELIVERY

Over the past few years, the landscape of mRNA technology and drug delivery has witnessed transformative evolution, marked by groundbreaking developments in mRNA vaccine design, therapeutic applications, analytics, and manufacturing. CHI's mRNA Manufacturing and Delivery Stream will serve as an incubator of ideas, fostering collaboration and discussion on the latest trends in mRNA vaccine design, analytics, and scalable manufacturing. Additionally, experts will share formulation and delivery strategies for mRNAs, proteins, novel modalities therapies, and non-traditional modalities through case studies, unpublished work, and winning strategies.

## Conference Programs

**AUGUST 19-20**

mRNA Development & Manufacturing

View Program »

**AUGUST 21-22**

Formulation & Delivery

View Program »

## MONDAY, AUGUST 19

**8:00 am Registration and Morning Coffee**

### REGULATORY LANDSCAPE AND STANDARDIZATION

**9:55 Chairperson's Opening Remarks**
*Craig Martin, PhD, Professor, Chemistry, University of Massachusetts, Amherst*

**10:00 CMC Regulatory Trends from the FDA on mRNA Therapies and Potential Strategies**
*Ageliki Tzovolos, Principal Consultant, CMC Biologics, Eliquent Life Sciences; Individual Consultant*
mRNA gene therapies have been impactful and common in our industry with many examples and health authority trends available. FDA manufacturing and regulatory expectations are also better understood through the various inputs in submissions, interactions with the Agency, as well as guidances published.

**10:30 FEATURED PRESENTATION: DNA Starting Material Quality: A Copy Can Only Be As Good As The Template**
*Lawrence C. Thompson, PhD, Senior Principal Scientist, Analytical R&D, Pfizer Inc.*
- The impact of nicked DNA starting material on the quality of rAAV vs mRNA drug substance
- Off-target "star activity" during enzymatic linearization
- Fidelity of plasmid vs cell-free DNA manufacturing

**11:00 Improving RNA Structure Predictions with Diverse Data and Machine Learning**
*Silvi Rouskin, PhD, Assistant Professor, Harvard Medical School*
Our research tackles a key issue in RNA biology: predicting RNA secondary structures accurately, especially for long RNAs like mRNAs and ncRNAs. We developed eFold, a novel deep learning approach trained on our expansive RNAndria database, featuring over 2,500 complex RNA structures. eFold shows superior accuracy in predicting long RNA structures, marking a substantial advancement in RNA biology and computational modeling.

**11:30 Sponsored Presentation** *(Opportunity Available)*

**12:00 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:30 Session Break**

### PROCESS, SCALABILITY, AND MANUFACTURING ADVANCES

**12:50 Chairperson's Opening Remarks**
*Rajiv Gangurde, PhD, Vice President, Technical Operations, Parexel*

**12:55 Biophysical Properites of mRNA: Scalability and Manufacturing Advances**
*Alois Jungbauer, PhD, Professor & Head, Biotechnology, Institute of Bioprocess Science and Engineering, University of Natural Resources and Life Sciences (BOKU)*
The biophysical properties of mRNA such as size, diffusivity, density, and elasticity is not well understood. This in-depth knowledge of these properties is important to optimize purification processes in a rational way. An overview of the current information of these properties are provided and how they can be used to design chromatography and filtration processes.

**1:25 A Scalable Continuous-Flow RNA Manufacturing Platform Using Functionally Co-Immobilized Enzyme and DNA**
*Craig Martin, PhD, Professor, Chemistry, University of Massachusetts, Amherst*
Current RNA manufacturing generates dsRNA impurities that must be removed, along with enzyme(s) and DNA, in purification. Functional co-immobilization of enzyme and DNA to a solid support prevents formation of dsRNA, eliminates costly purification and allows a continuous flow reactor for a single-path workflow from NTPs to highly pure RNA of any length. New analytics allow for real-time quality and yield optimizations in long continuous production runs at all scales.

**1:55 Presentation to be Announced**

SARTORIUS

**2:25 Networking Refreshment Break**

**2:40 mRNA Development, Automation, Scale-Up, and Tech Transfer**
*Joseph Elich, Senior Engineer, Prime Medicine*
The adoption of mRNA for novel vaccines and therapeutics has presented new CMC challenges for drug developers. A successful mRNA drug candidate requires a manufacturing process that is robust, well-controlled, and cost-effective. Importantly, developers must also consider the specific requirements of their drug product indication, from global vaccines to gene therapies. This presentation highlights important strategies, tools, and examples to accelerate mRNA process development from research through regulatory submission.

**3:10 Key Learnings during mRNA-LNP DP Process Optimization**
*Huu Thuy Trang Duong, Senior Scientist, Formulation Development Group, Regeneron Pharmaceuticals Inc.*

**3:40 Session Break and Transition to Plenary Keynote Session**

### PLENARY KEYNOTE SESSION: SOLVING TODAY'S CHALLENGES

**4:20 Organizer's Remarks**
*Daniel Barry, Senior Conference Director, Cambridge Healthtech Institute*

**4:30 PLENARY KEYNOTE PRESENTATION: READY: Addressing Current Challenges in Biomanufacturing with Reliability, Efficiency, Agility, Data, and (High) Yields**
*Jerry A. Murry, PhD, Senior Vice President, Process Development, Amgen*
The biopharmaceutical sector is currently producing vast amounts of data, a trend set to amplify with smart sensors, PAT, and process automation. This presentation will highlight the significance of a holistic digital strategy, incorporating AI, machine learning, predictive modeling, and data visualization, to spearhead the evolution of biomanufacturing. Emphasizing enhanced efficiency and innovation, this strategy will enable the efficient manufacture of complex biologics with reliability of supply, agility, and differentiation.

**5:10 Plenary Q&A**

**5:30 Welcome Reception in the Exhibit Hall with Poster Viewing**

**6:30 Close of Day**

## TUESDAY, AUGUST 20

**7:30 am Registration and Morning Coffee**

### CMC & FORMULATION DEVELOPMENT

**7:55 Chairperson's Remark**
*Weiyi Li, PhD, Scientist II, Prime Medicine Inc.*

# mRNA Development, Analytics and Manufacturing

*Development, Analytics, Delivery, and Manufacturing of mRNA Therapies and Vaccines*

**AUGUST 19-20**
All Times EDT

**8:00 Next-Generation mRNA Vaccines and Therapies for Various Infectious Diseases and Cancers**

***Paolo Lusso, MD, PhD, Chief, Senior Investigator, Viral Pathogenesis Section, NIAID, NIH***

We developed a multiclade VLP-forming HIV-1 *env-gag* mRNA vaccine that encompasses sequential immunizations with germline bNAb-engaging Envs followed by repeated heterologous Env boosts. The platform was further optimized by inclusion of the viral protease (pro) to yield mature VLPs. Preclinical studies in macaques documented efficient early priming with the recruitment of bNAb precursors against the CD4-binding site and, eventually, elicitation of heterologous tier-2 neutralization and protection from heterologous SHIV challenge.

**8:30 Detection of dsRNA Impurities in mRNA Drug Substance Samples Using ddPCR**

***Snaha Dogiparthi, Scientist Bioassay Development, Early Bioprocess, Pfizer Inc.***

Double-stranded (dsRNA) RNA impurities pose a significant challenge in mRNA-based therapeutics due to their potential immunogenicity and off-target effects. Droplet Digital PCR has emerged as a sensitive and precise tool for the detection and quantification of nucleic acid impurities. In this study, we present a robust ddPCR-based method for the detection of dsRNA impurities in mRNA drug substance samples. The process involves cDNA generation using target-specific RT primer, followed by ddpcr.

**9:00 Next-Generation RNA Vaccines: Addressing CMC, Developability, and Scalability**

***Rajiv Gangurde, PhD, Vice President, Technical Operations, Parexel***

**9:30 Sponsored Presentation** *(Opportunity Available)*

**10:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**10:45 Breakout Discussion Groups**

Breakout Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussions page on the conference website for a complete listing of topics and descriptions.

**IN-PERSON ONLY BREAKOUT: Leveraging Thought Leadership & AI on Social Media for Scientific Advancement**

***Nandini Kashyap, M.Pharm., Senior Director, Conferences and Social Media Strategy, Cambridge Innovation Institute***

- Harnessing AI Tools for Literature Research, Content Planning, Creation and Distribution
- Leverage Publications, Speaking Opportunities, and Social Media to Increase Influence and Search Rankings
- Forming Meaningful Connections and Building and Nurturing Scientific Communities both Online and In-Person
- Overcoming Challenges and Barriers to Adoption

**11:30 Evolving Analytical Trends for the Characterization of mRNA**

***Khaled Yamout, Analytical Sciences, Quality and Manufacturing, Consultant Y-Chem Consulting, LLC***

The emergence of mRNA technology has ushered a new era of medicine. As the regulatory landscape continues to evolve to ensure mRNA-based products are of high quality, safe and effective. To meet these requirements, suitable testing methodologies are needed to properly measure critical quality attributes such as identity, content, purity and functionality. As such, we will discuss new and enhanced analytical trends for the characterization of mRNA.

**12:00 pm Computational Tools and Sequencing Technologies for Heightened Characterization of mRNA Therapeutics**

***Joe Saelens, PhD, Senior Principal Scientist, Computational Biology, Molecular Informatics, Pfizer***

Sequencing technologies combined with computational tools can enhance our understanding of nucleic acid therapeutics. This talk will provide an overview of these methods that we have developed for heightened characterization of mRNA.

**12:30 Sponsored Presentation** *(Opportunity Available)*

**1:00 Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**1:30 Refreshment Break in the Exhibit Hall with Poster Viewing**

**2:10 Chairperson's Remarks**

***Jianmei D. Kochling, PhD, Senior Director, Head of Analytical Development and QC, mRNA Center of Excellence, Sanofi***

**2:15 KEYNOTE PRESENTATION: What We Know and Do Not Know about Analytical Testing for mRNA Characterization**

***Jianmei D. Kochling, PhD, Senior Director, Head of Analytical Development and QC, mRNA Center of Excellence, Sanofi***

Despite the deepened analytical understanding of the mRNA molecule structure and mechanism of action and function of mRNA-LNP, the unique properties of IVT mRNA molecules and the mRNA-LNP complex add challenges to the characterization of the drug substance and drug product. This presentation will illustrate what we have learned from the past few years and what we still need to work on for mRNA-LNP characterization.

**3:15 Characterization for mRNA Therapies**

***Francis Poulin, PhD, Vice President, Analytical Sciences, Sail Biomedicines***

Introducing Sail Biomedicines' platform and discussing various methods for the analysis of circular RNAs. The presentation will identify key challenges in the analytical development of high-quality Endless RNA (eRNA). The discussion will focus on purity evaluation of circular RNAs and a novel AEX-HPLC analytical method used for eRNA.

**3:45 Refreshment Break in the Exhibit Hall with Poster Viewing**

**4:30 Quality Control and Analytical Characterization of mRNA LNP Drug Products in Early Clinical-Phase**

***Eivor Örnskov, PhD, Principal Scientist, AstraZeneca***

The presentation will outline critical quality attributes of mRNA lipid nanoparticle (LNP) drug products, with a focus on early clinical phases. It will also address potential impurities and degradation pathways pertinent to mRNA LNP formulations. A selection of key analytical methods essential for quality control and analytical characterization will be showcased.

**5:00 PANEL DISCUSSION: Analytical Techniques for Characterization of RNA and mRNA Products**

***Moderator: Jianmei D. Kochling, PhD, Senior Director, Head of Analytical Development and QC, mRNA Center of Excellence, Sanofi***

***Panelists:***

***Francis Poulin, PhD, Vice President, Analytical Sciences, Sail Biomedicines***

***Eivor Örnskov, PhD, Principal Scientist, AstraZeneca***

***Khaled Yamout, Analytical Sciences, Quality and Manufacturing, Consultant Y-Chem Consulting, LLC***

**5:30 Close of mRNA Development, Analytics and Manufacturing Conference**

# Formulation and Delivery of High-Concentration Proteins and New Modalities

*Strategies to Overcome Challenges in Viscosity, Aggregation, and Delivery*

**AUGUST 21-22**
All Times EDT

## WEDNESDAY, AUGUST 21

**7:30 am Registration and Morning Coffee**

### HIGH-CONCENTRATION PROTEIN FORMULATIONS

**7:55 Chairperson's Opening Remarks**
*Kanika Sarpal, PhD, Senior Scientist, Biologics Drug Product Development, Sanofi*

**8:00 Understanding Formulation and Process Needs for High-Concentration Protein Therapeutics**
*Kanika Sarpal, PhD, Senior Scientist, Biologics Drug Product Development, Sanofi*
High-concentration protein therapeutics have become more popular as they favor subcutaneous (SC) administration. Successful development of high dose biologics requires adopting certain formulation approaches to overcome technical challenges such as viscosity, solubility, stability, process issues, and delivery limitations. There is no one approach that fits all. This talk will outline some key aspects while designing high concentration protein therapeutics from the formulation and process standpoint.

**8:30 Ongoing Challenges and Considerations to Develop High-Concentration Protein Formulation**
*Jia He, Senior Scientist, Amgen*

**9:00 One-Step Formulation Development of Biologics**
*Slobodanka (Dina) Manceva, Associate Director Drug Product and Technology Development, Teva Branded Pharmaceuticals*
The accelerated timelines in the evaluation of novel drug products and getting 1st to the market, demand a fast formulation development. Here we present one step global formulation development approach that is able to select a formulation based on malty factor interaction in less than 4 months.

**9:30 Anatomy of High-Concentration Biologics**
*Twinkle Christian, MS, Senior Scientist, Amgen, Inc.*
High-concentration biologics are complex to manufacture and deliver with patient centric initiatives. This presentation will focus on the design space with an optimized TPP (target product profile), early engagement of pivotal multidisciplinary stakeholders, interdependency of critical attributes during product development and key patient centric milestones across product development lifecycle of a high-concentration biologic.

**10:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**10:40 KEYNOTE PRESENTATION: Applying Deep Learning to Predict High-Concentration Antibody Viscosity**
*Pin-Kuang Lai, PhD, Assistant Professor, Department of Chemical Engineering and Materials Science, Stevens Institute of Technology*
Highly concentrated antibody solutions are necessary for developing subcutaneous injections but often exhibit high viscosity. We measured a large panel of 229 antibody viscosity to develop predictive models for screening viscosity at high concentrations. DeepViscosity was developed based on artificial neural network models to classify low-viscosity and high-viscosity antibodies at 150 mg/mL. The DeepViscosity model exhibited an accuracy of 87.5% and an AUC score of 90% on 16 independent antibodies.

**11:40 Automated Formulation Development across Modalities**
*Peter Soler, PhD, Senior Research Investigator, Bristol Myers Squibb Co.*
Biologics drug development has experienced rapid growth in recent years. To meet the need biologics formulation development has quickly acquired a set of automation tools and analytical techniques to provide robust drug products for patients. This has motivated the adaptation of our tools to meet the increases in process complexity for the benefit of patients globally.

**12:10 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:40 Refreshment Break in the Exhibit Hall with Poster Viewing**

### NOVEL DRUG DELIVERY TECHNOLOGIES & DEVICES

**1:25 Chairperson's Remarks**
*Sean Bedingfield, PhD, Senior Advisor, Lilly Genetic Medicine, Eli Lilly and Company*

**1:30 AAV Drug Product Local Delivery Administration Device Consideration**
*Xin Jin, PhD, Scientist, Biological Drug Product Development, Sanofi*
Adeno-associated viruses (AAVs) have been widely used as the delivery vehicles for CNS gene therapies. Intra-cisterna magna (ICM) administration was one of the local delivery administrations, which has benefit of widespread transgene delivery in both brain and spinal cord. This presentation summarized the work of an AAV drug product ICM administration device selection and studies for both animal tox study and clinical trial study.

**2:00 RNA Delivery in the Central Nervous System**
*Sean Bedingfield, PhD, Senior Advisor, Lilly Genetic Medicine, Eli Lilly and Company*
The clinical use of small interfering RNA (siRNA) and antisense oligonucleotides has required, in some cases, the implementation of invasive routes of administration such as intrathecal or intraocular injection. However, improved durability is mitigated by clearance of siRNA. We present a microcapsule-based method to extend activity of cholesterol-conjugated siRNA locally. We show that microcapsules protect the siRNAs from being cleared and enable release over 3 months compared to unencapsulated siRNAs.

**2:30 Sponsored Presentation** *(Opportunity Available)*

**3:00 Refreshment Break in the Exhibit Hall with Poster Viewing**

### PLENARY FIRESIDE CHAT: LEADING TO TOMORROW'S ADVANCES

**3:50 Organizer's Remarks**
*Nandini Kashyap, M.Pharm., Senior Director, Conferences and Social Media Strategy, Cambridge Innovation Institute*

**4:00 Genetic Medicines—Transforming the Future of Biotherapeutics**




*Moderator: Ann Lee, PhD, CTO, Prime Medicine, Inc.*
*Panelists:*
*E. Morrey Atkinson, PhD, Executive Vice President, Chief Technical Operations Officer, Head, Biopharmaceutical Sciences and Manufacturing Operations, Vertex Pharmaceuticals Inc.*
*Manmohan Singh, PhD, CTO, Beam Therapeutics*
*Heidi Zhang, PhD, Executive Vice President, Head, Technical Operations, Tune Therapeutics*

**5:00 Networking Reception in the Exhibit Hall with Poster Viewing**

**6:00 Close of Day**

## THURSDAY, AUGUST 22

**7:30 am Registration and Morning Coffee**

### FORMULATION DEVELOPMENT OF CELL AND GENE THERAPIES

**7:55 Chairperson's Remark**
*Bharathi Vellalore, PhD, Senior Scientist, Therapeutics Development and Supply, Janssen Pharmaceuticals*

**8:00 Comparing the Outlook of Developability Assessment of Monoclonal Antibodies to AAV Therapeutics for Successful Lead Candidate Selection from Discovery to Development**
*Yogapriya Murugesan, Scientist I, Gene Therapy & Drug Product Development, Biogen*
Molecular properties that impact developability attributes and outcomes comprises of conformational, chemical, colloidal, and other interactions. These attributes are measured using relevant analytical methods to assess the developability/ manufacturability of the molecule in different formulation. Developability assessment of mAbs has been studied and applying this assessment using the right tools to new modalities such AAV will help streamline capsid selection and candidate selection from discovery to development for new modalities

**8:30 Drug Product Consideration for AAV-Based Gene Therapy Products**
*Paria Moxley, PhD, Scientist, Biologics Drug Product Development & Manufacturing, Sanofi*
Recombinant adeno-associated virus (AAV) has emerged as a promising gene delivery vector for the treatment of various diseases. There are marked differences in buffer selection for formulation development with AAVs and protein therapeutics, which must be considered in the context of product manufacturing, long-term storage, and shipping/handling. This entails screening for buffer pH, ionic strength, and the impact of added surfactants on stability/degradation trends.

**9:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**9:30 Breakout Discussion Groups**
Breakout Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussions page on the conference website for a complete listing of topics and descriptions.

**IN-PERSON ONLY BREAKOUT: Process Development and Manufacturing Considerations for Novel Modalities**
*Bharathi Vellalore, PhD, Senior Scientist, Therapeutics Development and Supply, Janssen Pharmaceuticals*
- Scale-out vs scale-up for allogeneic and autologous cell therapies
- Manufacturing considerations for lentivirus
- Large-scale manufacturing of gene therapies and other novel modalities

**10:30 FEATURED PRESENTATION: Concentrating siRNA by Ultrafiltration for Gene Therapy Applications**
*Ken K. Qian, PhD, Scientific Director, Eli Lilly & Co.*
The present study is focused on developing a fundamental understanding of the factors controlling the ultrafiltration behavior of a siRNA drug product during tangential flow filtration (TFF). A dependence of the filtrate flux on the logarithm of the siRNA concentration was observed, consistent with classical concentration polarization models. Our work demonstrates the importance of both concentration polarization and membrane fouling on the ultrafiltration behavior of highly concentrated solutions of siRNA.

**11:00 Cell Therapy Drug Product Development**
*Bharathi Vellalore, PhD, Senior Scientist, Therapeutics Development and Supply, Janssen Pharmaceuticals*
- Process considerations for manufacturing autologous and allogeneic cell therapy products
- Drug product considerations for hematological malignancies and solid tumor indications
- Clinical vs commercial supply chain needs: Integrated drug product design

**11:30 Sponsored Presentation** *(Opportunity Available)*

**12:00 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:30 Refreshment Break in the Exhibit Hall & Last Chance for Poster Viewing**

### LNPs & NOVEL DELIVERY APPROACHES

**1:05 Chairperson's Remarks**
*Niels Delamotte, Director Analytical Development, Etherna*

**1:10 Analytical Lifecycle Management to expand Analytical Capabilities in support of product/process development.**
*Niels Delamotte, Director Analytical Development, Etherna*
We'll discuss our strategy for overcoming some of the QC challenges and strengthening analytical capabilities in support of mRNA Drug Substance and LNP product and process development. It will delve into the development of some of the traditional analytics used within Quality Control as well as emerging trends in analytical techniques for more in-depth characterization. The goal is to share insights to foster open dialogue to collectively advance the field.

**1:40 Formulation Developability Assessment for Viral Vector Delivery Agents: A Closer Look into Physical and Functional Particle Assessment**
*Ahmet Bekdemir, PhD, Senior Scientist II, Formulation & Analytics, Novartis Institutes for BioMedical Research Inc.*
Maintaining the stability of viral vectors through formulation assessment is essential for cell and gene therapy products. In this presentation, I will describe a study conducted to evaluate the stability of particle characteristics and functional titer for lentiviral vectors under varying buffer, pH, and excipients conditions. Through our screening experiments and comprehensive analytics, I will discuss how stability for these complex modalities is multifaceted and requires careful investigation.

**2:10 Process Development and CMC Considerations for the Development of Prime Editor Lipid Nanoparticles to Correct Disease-Causing Mutations**
*Weiyi Li, PhD, Scientist II, Prime Medicine Inc.*
Prime editing is a next-generation genome editing technology that could theoretically correct up to 90% of known genetic variants associated with human diseases. We have developed a universal lipid nanoparticle (LNP) for the delivery of Prime Editors (PE) to the liver. This presentation will highlight process development and CMC considerations for the development of PE-LNPs and provide selected case studies for PE RNA components and LNP-formulated PE process unit optimization.

**2:40 Networking Refreshment Break and Transition into Town Hall Discussions**

## FACILITATED TOWN HALL DISCUSSIONS

**2:55 Facilitated Town Hall Discussions**

These Town Halls offer delegates the opportunity to participate in interactive discussions on important themes that were explored during the conference. Each Hall will have a host(s) to facilitate the conversation, and all are welcome to participate, share views and best practices and ask questions of colleagues.

**Harnessing ML/AI and Big Data for Biotherapeutic Development**

*Pin-Kuang Lai, PhD, Assistant Professor, Department of Chemical Engineering and Materials Science, Stevens Institute of Technology*

**Cell and Gene Therapy Manufacturing: In-House vs. Outsourced**

*Elben Guimaraes, Senior Manufacturing Manager, Upstream Manufacturing, Ultragenyx Pharmaceutical Inc.*

The decision of handling cell and gene therapy processes in-house or outsourcing them is crucial. This facilitated discussion explores the advantages and challenges of both approaches, analyzing their impact on cost, control, strategic direction, and innovation. Share experiences and best practices for managing internal and external manufacturing, while examining common scenarios faced by sponsors and vendors.

**Digital Transformation & AI in Bioprocess—What, Where, When, and How?**


*Christian Airiau, PhD, Global Head, Data Sciences, CMC, R&D, Sanofi*
*Irene Rombel, PhD, CEO & Co-Founder, BioCurie Inc.*

The bioprocessing industry is undergoing a digital revolution fueled by AI. This interactive session dives into current digital adoption and explores the latest trends in AI applications (AIML). Join the conversation to explore the potential of AI for process optimization and digital twins. Share real-world success stories and discuss ethical considerations along with potential workforce impacts.

**3:55 Close of Summit**

# STREAM #6
# ANALYTICAL & QUALITY

In an era of unprecedented pressure for efficiency and innovation, biotherapeutics development is demanding a paradigm shift in analytical capabilities. The Analytical and Quality Stream offers four days of immersive presentations to help you respond to this challenge. You'll learn about new higher throughput technologies and workflows and explore the transformative potential of AI and big data, leveraging predictive insights to unlock new insights and critical unknowns. Then explore the evolving landscape of next-generation analytical methods, mastering state-of-the-art solutions for protein characterization, precision manufacturing, and advanced spectroscopic techniques. This pipeline fosters profound learning through interactive discussions, poster presentations, and invaluable networking opportunities, ensuring you emerge empowered to revolutionize your biotherapeutics development programs.

## Conference Programs

**AUGUST 19-20**

Accelerating Analytical Development

View Program »

**AUGUST 21-22**

Next Generation Analytical Methods

View Program »

## MONDAY, AUGUST 19

**8:00 am Registration and Morning Coffee**

### OPTIMIZING PLATFORMS AND WORKFLOWS

**9:55 Chairperson's Opening Remarks**
*Rosalind Ang, PhD, Associate Principal Scientist, Merck*

**10:00 Platform Validation for Process Impurities Workflows**
*Rosalind Ang, PhD, Associate Principal Scientist, Merck*
Successful biologic drug characterization demands meticulous identification and control of process impurities. This presentation will explore the development and implementation of a robust platform validation strategy for process impurities. We'll discuss critical parameters, analytical techniques, and best practices for ensuring comprehensive validation. Attendees will gain insights to streamline impurity characterization, enhance product safety, and meet regulatory requirements.

**10:30 Overcoming the Barriers to Further Adoption of MAM**
*Hao Zhang, PhD, Senior Principal Scientist and Team Lead, Pivotal Attribute Sciences, Amgen*
The advances of new therapeutic modalities drive the development of liquid chromatography (LC)-mass spectrometry (MS)-based Multi-Attribute Method (MAM). MAM has successfully demonstrated its capability in replacing some of the traditional chromatographic and electrophoretic testing methods for monitoring product quality attributes for both release and in-process testing. We list several hurdles encountered along the way of MAM adoption and discuss the approaches to overcome them based on the latest development efforts.

**11:00 From Insight to Impact: Prior Knowledge and Streamlined Workflows in Analytical Development**
*Weichen Xu, PhD, Director, Analytical Sciences, Macrogenics*
To expedite new medicines to patients, the biopharmaceutical industry is focusing on platform technologies and prior knowledge. The application in analytical development is not a one-size-fits-all approach, but a dynamic strategy shaped by the unique historical wealth of knowledge tied to each method at each company. Beyond this, streamlining processes plays an integral role in optimizing operational efficiency. This presentation discusses how MacroGenics strategically employs these approaches to accelerate analytical development.

**11:30 Sponsored Presentation** *(Opportunity Available)*

**12:00 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:30 Session Break**

### AUTOMATION AND MINIATURIZATION

**12:50 Chairperson's Remarks**
*Lasse Stach, PhD, Principal Investigator & Leader, Developability Profiling Team, GSK*

**12:55 ML-Enabled Image Analysis to Characterize Formulation Aggregates**
*Theodore Randolph, PhD, Professor, Chemical and Biological Engineering, University of Colorado*
Many drug product manufacturing processes require characterization of microparticulate products and contaminants. Machine learning analyses of flow imaging microscopy datasets can be used for these applications, including monitoring cell health and debris during manufacture of cell-based therapies, detection of particulate matter formed during processing of adjuvanted vaccine suspensions, and exploration of root-causes for protein aggregation. We will discuss advances in unsupervised and supervised machine learning for these analytical tasks.

**1:25 Automation for All: Developing Workflows for Broad Deployment**
*Jon Jurica, PhD, Principal Scientist, Analytical Research and Development, Merck & Co.*
The use of automation provides significant opportunities in biologics analytical development to enable increased efficiency and improved experimental design. At Merck, we have strategically positioned a group of automation experts with an explicit goal to develop user-friendly tools, templates, and designs that are shared with our scientists, including simple bench-top platforms and larger liquid handling systems. We discuss implementation of this strategy that has resulted in an automation-first mindset.

**1:55 Sponsored Presentation** *(Opportunity Available)*

**2:25 Networking Refreshment Break**

**2:40 Development of a Custom-Automated Method for AAV Capsid Titer in Gene Therapy Products**
*Matthew J. Lotti, Senior Research Associate II, Ultragenyx Pharmaceutical, Inc.*
For viral vectors used in gene therapies, monitoring concentration throughout manufacture is vital for product consistency and quality. Using automation to assess AAV capsid titer enhances throughput while reducing assay hands-on time. The following presentation describes the development of an AAV capsid titer assay that combines two forms of automation: automated sample preparation and automated immunoassay and analysis. The resulting assay produces high-throughput, accurate sample results while reducing hands-on time.

**3:10 Scaling Lab Automation: Proactive Semi-Automation in Assay Development for Efficient Transition to Full Automation**
*Kentaro Marchionni, Automation Engineer, Cellino Biotech*
Semi-automation is a proactive approach to assay development that ensures the entire process is aligned with assay requirements and is automation-compatible. Semi-automated assays are inherently designed with miniaturization and optimization considerations, ensuring seamless scalability and efficiency. This approach prevents redevelopment of assays that are incompatible with automation and greatly simplifies the process of transitioning them into fully automated systems.

**3:40 Session Break and Transition to Plenary Keynote Session**

### PLENARY KEYNOTE SESSION: SOLVING TODAY'S CHALLENGES

**4:20 Organizer's Remarks**
*Daniel Barry, Senior Conference Director, Cambridge Healthtech Institute*

**4:30 PLENARY KEYNOTE PRESENTATION: READY: Addressing Current Challenges in Biomanufacturing with Reliability, Efficiency, Agility, Data, and (High) Yields**
*Jerry A. Murry, PhD, Senior Vice President, Process Development, Amgen*
The biopharmaceutical sector is currently producing vast amounts of data, a trend set to amplify with smart sensors, PAT, and process automation. This presentation will highlight the significance of a holistic digital strategy, incorporating AI, machine learning, predictive modeling,

and data visualization, to spearhead the evolution of biomanufacturing. Emphasizing enhanced efficiency and innovation, this strategy will enable the efficient manufacture of complex biologics with reliability of supply, agility, and differentiation.

**5:10 Plenary Q&A**

**5:30 Welcome Reception in the Exhibit Hall with Poster Viewing**

**6:30 Close of Day**

## TUESDAY, AUGUST 20

**7:30 am Registration and Morning Coffee**

## PREDICTIVE MODELING AND MACHINE LEARNING IN BIOPROCESS ANALYTICS

**7:55 Chairperson's Remarks**
*Bo Zhai, PhD, Principal Scientist, Analytical Method Development, Janssen*

**8:00 Higher Throughput Antibody Characterization to Improve Candidate Quality and Enable Machine Learning**
*Lasse Stach, PhD, Principal Investigator & Leader, Developability Profiling Team, GSK*
At the interface between discovery and CMC, the developability team at GSK characterizes lead molecules to identify stable molecules for progression. Making use of significant investment in protein production facilities, we are now collecting biophysical data at a higher throughput and at near formulation strength. This talk will focus on how these rich data are used to improve candidate quality as well as to feed predictive models.

**8:30 *In silico* CQA Identification and Assessment**
*Michael Kim, PhD, Technical Development Senior Principal Scientist, Protein Analytical Chemistry, Genentech*
Protein therapeutics contain heterogenous product variants, often due to post-translational modifications (PTM). A specific PTM's criticality depends on its potential impact to a therapeutic's efficacy and safety, which is traditionally evaluated empirically. With the burgeoning rise in computational power and biological structure elucidation, we explore the use of *in silico* biophysical modeling—specifically thermodynamic integration for relative binding free energies—to inform functional impacts of PTMs.

**9:00 KEYNOTE PRESENTATION: Where Are the Data—Solving One Challenge at a Time for Developing Digital Technologies to Support All Phases of Analytical Method Lifecycle**
*Neeraj Agrawal, PhD, Director, Attribute Science Data Engineering, Amgen*
FAIR data is required to derive maximum value from the recent developments in generative AI, ML, and other digital technologies. Extraction of FAIR data from diverse source systems that are used throughout the lifecycle of analytical methods while maintaining data integrity, as required in the regulated environment, requires substantial investments. This presentation will showcase Amgen's strategy for developing digital technologies to support all phases of analytical method lifecycle.

**9:30 Sponsored Presentation** *(Opportunity Available)*

**10:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**10:45 Breakout Discussion Groups**
Breakout Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussions page on the conference website for a complete listing of topics and descriptions.

**11:30 Making Data Work for You—Transformational Data Analytics Solutions**
*Brian Good, PhD, Senior Research Advisor, Eli Lilly and Company*
As scientists, we have expected electronic data to deliver us, only to find we are subjugated by it. The time has come to realize the unfulfilled promise. New technologies like NoSQL, ontologies, and AI/ML are rushing towards us and have outmoded our current platforms. We will explore how these technologies are changing our laboratories and increasing the value we can bring to our organizations through streamlined information delivery.

**12:00 pm Capture and Assimilation of Historical Analytical and Process Data**
*Christina Vessely, PhD, Senior Consultant, CMC Analytics & Formulation Development, Biologics Consulting Group, Inc.*
The development of biologics generally spans years, and we build on our past experiences as we advance. As we start working on our BLA filing, we find ourselves floating in a sea of data with no clear direction, and often with databases that are only partially searchable. How do we assimilate our big data and how do we assure that future data will be better organized and more searchable?

**12:30 Sponsored Presentation** *(Opportunity Available)*

**1:00 Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**1:30 Refreshment Break in the Exhibit Hall with Poster Viewing**

**2:10 Chairperson's Remarks**
*Chaojie Wang, Scientist, Biologics, Bristol Myers Squibb Co.*

**2:15 SPECIAL PRESENTATION: Building a Roadmap for Implementation of the Multi-Attribute Method in QC**
*Diane McCarthy, PhD, Senior Scientific Director, Global Biologics, US Pharmacopeia*
While the multi-attribute method (MAM) has potential to improve the efficiency and specificity of analytical testing, several challenges remain to implementation in QC. This presentation will provide an overview of considerations and best practices for use of MAM in QC from <1060> Mass Spectrometry-Based Multi-Attribute Method for Therapeutic Proteins. An update on a study of MAM versus conventional methods, funded through a cooperative agreement with FDA, will also be provided.

## NEW STRATEGIES AND TECHNOLOGIES

**2:45 A Systems Biology Approach to Modeling CHO Cell Cultures and Predicting Outcomes**
*Bo Zhai, PhD, Principal Scientist, Analytical Method Development, Janssen*
CHO cell biopharmaceutical production faces challenges due to the demand for high-titer and complex molecules. The genome-scale metabolic model serves as a powerful tool for exploring cellular physiology and predicting cellular behaviors. By integrating omics data and advanced computational

techniques, it guides metabolic engineering strategies for bioprocess optimization. Moreover, the model will guide in-process analytical testing strategies ensuring consistent product quality across all stages of production.

3:15 **Analytical Insights into Innovative Biologics and Biosimilars: Unveiling the Key Differences in Analytical Development**

***Miha Vodnik, PhD, Senior Expert Science & Technology, Novartis***

Analytics represent a fundamental pillar for development of biosimilars and innovative biologics. Although they are both biopharmaceuticals, the analytical strategies diverge in terms of purpose, scope, methods, and timelines. Novartis has years of experience in development of biologics and has recently transitioned into a fully innovative medicines-focused company. This presentation aims to delineate the critical distinctions between biosimilars and innovative biologics, underscoring the scientific and organizational aspects of analytical development.

3:45 **Refreshment Break in the Exhibit Hall with Poster Viewing**

4:30 **Case Study: NGS for Deep Characterization**

***Chaojie Wang, Scientist, Biologics, Bristol Myers Squibb Co.***

Monoclonality is expected for a biologics-producing cell line. Retrospective analysis of clonality using Southern blot raised questions about clonality vs. genetic plasticity of a cell line. Long-read sequencing is an innovative assay for plasmid integration structure analysis. CRISPR/Cas9-targeted Nanopore long-read sequencing provided accurate information on the integration structure, and helped solve the clonality vs. plasticity issue. Using Southern, Sanger sequencing, and NGS as orthogonal assays confirmed the conclusions.

5:00 **Fully Automated Immuno-µPlaque Assay for Live-Attenuated Quadrivalent Dengue Vaccine Development**

***Yi Wang, PhD, Senior Scientist, Vaccine Analytical R&D Merck***

A 96-well plate format immuno-µPlaque assay was developed for a viral potency test to support the development of a live-attenuated quadrivalent dengue vaccine. Full automation of the assay via an integrated robotic system illustrated the potential of high-throughput cell-based analytics in the vaccine development space. A deep learning-based plaque-counting algorithm further accelerates the assay by providing analysts with precise analysis results and robust workflow.

5:30 **Close of Accelerating Analytical Development Conference**

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## WEDNESDAY, AUGUST 21

**7:30 am Registration and Morning Coffee**

### EVOLUTION OF CORE ANALYTICAL METHODS

**7:55 Chairperson's Remarks**
*Hirsh Nanda, PhD, Director, Analytical Sciences, Janssen*

**8:00 Evolution of Core Analytical Methods during the Development Lifecycle for mAb Products**
*Claudia Gributs, PhD, Senior Director, Research and Development, Eli Lilly and Company*
Teams often strive to implement appropriate analytical methods in early-phase development and minimize changes as product development progresses. To this end, platform methods have gained popularity for monoclonal antibodies (mAbs). Nevertheless, at least one analytical method inevitably evolves between FHD and commercialization. This presentation will discuss factors that drive method changes and approaches to analytical method bridging that balance the desire for exhaustive datasets with material and resource availability.

**8:30 New Methods and Strategies for Particle Analysis**
*Shawn Cao, PhD, Scientific Director, Amgen*
This presentation surveys the current landscape and explores innovative methods and strategies for analyzing particles in biologic drugs. It delves into recent advancements that enhance our understanding of particle characterization, origin, and the ongoing pursuit and future direction of improved analytical tools for ensuring the quality and effectiveness of biologic therapies.

**9:00 Evolving Core Methods via Automation, Platforming, and Improved Usability**
*Bharathi Govindarajan, PhD, Principal Scientist, Bioanalytical Sciences, Sanofi, United States*
Platform methods act as a great tool to support efficient and faster readiness to onboard or advance a new program through the clinical phases. It is important to have a comprehensive understanding of platform methods to build confidence in the use of these tools to support the development of new molecules. This presentation will focus on strategies to support the implementation of robust platform methods with focus on ELISA-based assays.

**9:30 Sponsored Presentation** *(Opportunity Available)*

**10:00 Coffee Break in the Exhibit Hall with Poster Viewing**

### CHARACTERIZATION OF mRNA, OLIGO, AND ANTISENSE THERAPEUTICS

**10:40 Biophysical Characterization for Antisense Oligos**
*Nicholas Larson, PhD, Scientist, Biogen*
Antisense oligonucleotides (ASOs) are short nucleotide sequences designed antisense to target RNAs. To increase their nuclease resistance the phosphate (PO) backbone is often modified to phosphorothioate (PS), creating a new chiral center. ASOs with many PS chiral centers are mixtures of thousands of diastereomers. We compared circular dichroism, Phosphate-31 NMR, and LC-MS for characterizing the diastereomeric distribution of ASOs.

**11:10 Evaluation of Current and Advanced Analytical Technologies for the Comprehensive Characterization of mRNA and Its Impurities**
*Axel Guilbaud, PhD, Principal Scientist, Genetech*
This study delves into the comprehensive profiling of *in vitro* transcribed (IVT) mRNA impurities, crucial for enhancing safety and efficacy in biotechnological applications. Leveraging advanced analytical tools such as ion-pair reversed-phase liquid-chromatography, capillary gel-electrophoresis, microcapillary-electrophoresis, mass-photometry, and native mass-spectrometry, we unveil impurities related to mRNA variants and double-stranded mRNA byproducts. Our findings emphasize the need for improved analytical characterization, offering valuable insights for optimizing IVT mRNA production in biotechnological contexts.

**11:40 Diastereomer Characterization of Phosphorothioate Synthetic Oligonucleotides Using a Tandem IMS-MS Method**
*Shannon A. Raab, PhD, Research Scientist, Bioproduct R&D, Eli Lilly & Co.*
Synthetic oligonucleotides have emerged as effective treatments for genetic diseases. Oligonucleotide therapeutics are commonly modified with a substitution of a phosphorothioate linkage along the phosphodiester backbone which creates a mixture of diastereomer structures. Analytical methods to measure the resulting diastereomers are currently lacking despite recent draft guidance highlighting the importance of their characterization. Here, we present a method combining tandem MS and tandem IMS to study diastereomers in modified oligonucleotides.

**12:10 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:40 Refreshment Break in the Exhibit Hall with Poster Viewing**

### CHARACTERIZATION OF BISPECIFICS AND CONJUGATES

**1:25 Chairperson's Remarks**
*Shannon A. Raab, PhD, Research Scientist, Bioproduct R&D, Eli Lilly & Co.*

**1:30 Building an LC-MS-Based Analytical Toolbox for Characterization of Polysaccharide-Protein Conjugate Vaccines**
*Pavlo Pristatsky, Associate Principal Scientist, Merck & Co. Inc.*
To improve understanding of the structure-function relationship of a Pneumococcal conjugate vaccine, several LC-MS assays were developed for process development and characterization. Notably, Serotype 5 polysaccharide which contains a ketone group in its repeating unit is included in some vaccine formulations. An LC-MS based assay procedure paired with an isotope-labeling strategy was developed and will be presented to characterize the integrity of the ketone group after the conjugation reaction.

**2:00 KEYNOTE PRESENTATION: Structural MS Techniques for Understanding Highly Engineered Multispecifics**
*Hirsh Nanda, PhD, Director, Analytical Sciences, Janssen*
The advent of multispecific biotherapeutics, capable of engaging multiple targets simultaneously, marks a significant milestone in both disease treatment and the ability to design complex protein modalities. This class of drugs demands precise optimization of bioprocess conditions and analytical verification of structure and function. Structural mass-spectrometry techniques are used to map engineered disulfides and identify misfolded regions causing aggregation, thereby leading to molecule designs with better manufacturability and efficacy.

**2:30 Presentation to be Announced**

**3:00 Refreshment Break in the Exhibit Hall with Poster Viewing**

## PLENARY FIRESIDE CHAT: LEADING TO TOMORROW'S ADVANCES

**3:50 Organizer's Remarks**
*Nandini Kashyap, M.Pharm., Senior Director, Conferences and Social Media Strategy, Cambridge Innovation Institute*

**4:00 Genetic Medicines—Transforming the Future of Biotherapeutics**

*Moderator: Ann Lee, PhD, CTO, Prime Medicine, Inc.*
*Panelists:*
*E. Morrey Atkinson, PhD, Executive Vice President, Chief Technical Operations Officer, Head, Biopharmaceutical Sciences and Manufacturing Operations, Vertex Pharmaceuticals Inc.*
*Manmohan Singh, PhD, CTO, Beam Therapeutics*
*Heidi Zhang, PhD, Executive Vice President, Head, Technical Operations, Tune Therapeutics*

**5:00 Networking Reception in the Exhibit Hall with Poster Viewing**

**6:00 Close of Day**

## THURSDAY, AUGUST 22

**7:30 am Registration and Morning Coffee**

## NEW TECHNOLOGIES

**7:55 Chairperson's Remarks**
*Varsha Daswani, PhD, PMP, Senior Director, Data Strategy, Lumilytics*

**8:00 Host Cell Protein Analysis for Adeno-Associated Virus (AAV)-Based Gene Therapy by Differential Digestion-Based LC-MS Method**
*Yunli Hu, PhD, Senior Principal Scientist, Regeneron Pharmaceuticals Inc.*
The identification and monitoring of residual host cell proteins (HCPs) in adeno-associated virus (AAV) by LC-MS is critical for maintaining product quality. However, applying LC-MS-based techniques to AAV poses unique challenges. In this presentation, we will explore these challenges and strategies to overcome them in the context of AAV HCP analysis. Additionally, we will present a newly developed, highly sensitive method that preserves AAV integrity while preferentially digesting HCPs.

**8:30 Integrating and Correlating Instrument Outputs for the Study of AAV Aggregation**
*Adebowale Shoroye, Scientist, Biogen*
Aggregation is a critical quality attribute that needs to be appropriately controlled in any biopharmaceutical product. For AAV-based therapeutics, methods to assess aggregation present a unique challenge. In this study, we compared strengths and weaknesses of SEC, AF4, analytical ultracentrifugation, and mass photometry. We present a case study in which very large aggregates were generated to evaluate the performance of each method in the separation and quantitation of aggregates.

**9:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**9:30 Breakout Discussion Groups**
Breakout Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussions page on the conference website for a complete listing of topics and descriptions.

**10:30 Advances in mRNA Vaccine Analytics**
*Sarah Muse, PhD, Senior Scientist, Sanofi*
mRNA vaccines offer transformative potential for disease prevention, but their rapid development demands advanced analytical techniques. This presentation delves into the latest breakthroughs in mRNA vaccine analytics. Attendees will explore cutting-edge methods for characterizing mRNA integrity, purity, and potency. Discover how these innovations optimize vaccine stability, safety, and efficacy throughout the preclinical development process.

**11:00 Incorporating *in silico* 3D Structure Determination into Characterization of Biotherapeutics**
*Varsha Daswani, PhD, PMP, Senior Director, Data Strategy, Lumilytics*
The *in silico* methods of identifying three-dimensional protein structure from primary sequence data are active areas of research with applications to drug discovery and development. We will be presenting our approach to adapt recent advancements in structure prediction technology to risk-site identification and validation in biologics. We will also discuss a newly developed model for scoring the accuracy of a predicted protein structure in the absence of experimental results.

**11:30 Sponsored Presentation** *(Opportunity Available)*

**12:00 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:30 Refreshment Break in the Exhibit Hall & Last Chance for Poster Viewing**

## NEW MS APPLICATIONS AND TECHNOLOGIES

**1:05 Chairperson's Remarks**
*Alayna George Thompson, PhD, Senior Research Scientist, AbbVie*

**1:10 Characterizing Monoclonal Antibodies and Antibody-Drug Conjugates by Top-Down and Middle-Down Mass Spectrometry**
*Benqian Wei, PhD, Senior Scientist, Merck*
Top-down and middle-down mass spectrometry (TD/MD-MS) are emerging techniques that minimize sample preparation and preserve endogenous post-translational modifications (PTMs) compared to bottom-up MS. Here, we show that assigning non-canonical internal fragments in TD-/MD-MS helps recover nearly 100% of the sequence and reveals important disulfide connectivity information of an intact mAb. In addition, drug conjugation sites can also be determined for a heterogeneous lysine-linked ADC using this novel approach.

**1:40 New Mass Spectrometry Approaches in Forced Degradation for Biologics Lead Optimization/Early Development**
*Alayna George Thompson, PhD, Senior Research Scientist, AbbVie*
Our group gathers chemical liability data to inform biologics candidate design or advancement in the late-discovery pipeline. We miniaturized forced degradation by focusing on mass spectrometry because of the richness of data and broad applicability across biologic formats. The recent

construction of a structured data warehouse allows broader access to data by collaborating scientists. Overall, these approaches enable comprehensive, timely, and pipeline-appropriate decisions on biologic candidates.

**2:10 Novel Approaches and Practical Applications of New Peak Detection in Drug Development**

***Qinjingwen Cao, PhD, Principal Scientist, Technical Development, Genentech***

New Peak Detection (NPD), a critical component of Multi-Attribute Method (MAM), detects peak variations effectively. An efficient NPD method with improved sensitivity is vital for monitoring process-related attributes. This study presents the development of a robust NPD method that successfully enhances sensitivity and maintains controlled false positives. The efficacy of this innovative NPD approach was assessed at various applications to fulfill pipeline needs, exemplifying its significant potential in drug development.

**2:40 Networking Refreshment Break and Transition into Town Hall Discussions**

## FACILITATED TOWN HALL DISCUSSIONS

**2:55 Facilitated Town Hall Discussions**

These Town Halls offer delegates the opportunity to participate in interactive discussions on important themes that were explored during the conference. Each Hall will have a host(s) to facilitate the conversation, and all are welcome to participate, share views and best practices and ask questions of colleagues.

**Harnessing ML/AI and Big Data for Biotherapeutic Development**

***Pin-Kuang Lai, PhD, Assistant Professor, Department of Chemical Engineering and Materials Science, Stevens Institute of Technology***

**Cell and Gene Therapy Manufacturing: In-House vs. Outsourced**

***Elben Guimaraes, Senior Manufacturing Manager, Upstream Manufacturing, Ultragenyx Pharmaceutical Inc.***

The decision of handling cell and gene therapy processes in-house or outsourcing them is crucial. This facilitated discussion explores the advantages and challenges of both approaches, analyzing their impact on cost, control, strategic direction, and innovation. Share experiences and best practices for managing internal and external manufacturing, while examining common scenarios faced by sponsors and vendors.

**Digital Transformation & AI in Bioprocess—What, Where, When, and How?**


***Christian Airiau, PhD, Global Head, Data Sciences, CMC, R&D, Sanofi***
***Irene Rombel, PhD, CEO & Co-Founder, BioCurie Inc.***

The bioprocessing industry is undergoing a digital revolution fueled by AI. This interactive session dives into current digital adoption and explores the latest trends in AI applications (AIML). Join the conversation to explore the potential of AI for process optimization and digital twins. Share real-world success stories and discuss ethical considerations along with potential workforce impacts.

**3:55 Close of Summit**

# STREAM #7
# STABILITY & FORMULATION

The Stability and Formulation stream brings together experts in formulation, analytical sciences, drug delivery, and process science to share knowledge and foster dialogue and collaborations. These two conferences will feature practical insights, case studies, and rapid approaches for predicting protein instabilities and strategies for impurity detection, with a focus on host cell proteins (HCPs). The second part will focus on formulation, analytical, and AI/ML-driven strategies for high-concentration protein formulations, cell and gene therapies, and non-traditional modalities. It also explores drug-device combinations and smart drug-delivery devices.

## Conference Programs

**AUGUST 19-20**

Stability and Impurities

View Program »

**AUGUST 21-22**

Formulation and Delivery

View Program »

## MONDAY, AUGUST 19

**8:00 am Registration and Morning Coffee**

## REGULATORY CONSIDERATIONS AND GUIDELINES FOR HCPs & OTHER IMPURITIES

**9:55 Chairperson's Opening Remarks**
*Erika M. Friedl, PhD, Quality Expert, Haematology & Transfusion Medicine, Paul Ehrlich Institute, Germany*

**10:00 FEATURED PRESENTATION: Efficient HCP Risk Control in Line with Regulatory Perspectives**
*Erika M. Friedl, PhD, Quality Expert, Haematology & Transfusion Medicine, Paul Ehrlich Institute, Germany*
Process-related impurities such as HCPs are critical quality attributes. Removal and tight control of HCPs is necessary to ensure efficacy and safety of biotherapeutics. Regulatory expectations are outlined to implement suitable control strategies throughout the product life cycle. To mitigate regulatory pitfalls and to support product development and process optimization, appropriate HCP assays tailored to the product development stage should be used. Established methods/emerging technologies could facilitate market access.

**10:30 Best Practices and Tools to Support HCP Analysis by Mass Spectrometry**
*Anthony Blaszczyk, PhD, Senior Scientist, Global Biologics, US Pharmacopeia*
This presentation provides an update on USP's initiatives to enhance the quality and consistency of MS-based HCP analysis. This chapter, currently under review by the USP Expert Panel following public comments, outlines best practices for HCP identification and quantification by LC-MS/MS. The USP's strategy for developing and characterizing physical reference materials, including intact protein and SIL peptides to support identification and quantitation of high-risk and abundant HCPs, will be discussed.

## DETECTION, ANALYSIS, AND CONTROL OF HOST CELL PROTEINS

**11:00 What We Can Learn from HCP Analysis of +500 Projects Using LC-MS**
*Thomas Kofoed, PhD, Co-Founder & CEO, Alphalyse, Denmark*
At Alphalyse, we've curated a comprehensive database comprising Mass Spec (MS) data on 34,865 uniquely quantified Host Cell Proteins (HCPs) from hundreds of HCP projects spanning various drug categories and a spectrum of sample complexities, ranging from early process samples to final purified drug substances. The extensive database provides information about commonly found HCPs in similar drug types and insights into the process clearance of problematic HCPs.

**11:30 Sponsored Presentation** *(Opportunity Available)*

**12:00 pm Luncheon Presentation** *(Sponsorship Opportunity Available)*
**or Enjoy Lunch on Your Own**

**12:30 Session Break**

## DETECTION, ANALYSIS, AND CONTROL OF HOST CELL PROTEINS (CONT.)

**12:50 Chairperson's Remarks**
*Harsha Gunawardena, PhD, Principal Scientist, Mass Spectrometry, Janssen Pharmaceutical Companies of Johnson & Johnson*

**12:55 Analysis of Host Cell Proteins in AAV Products with ProteoMiner Protein Enrichment Technology**
*Sisi Zhang, Principal Scientist, Regeneron Pharmaceuticals, Inc.*
HCPs in adeno-associated virus (AAV) products can be effectively enriched by ProteoMiner beads and the detergent Pluronic F-68 can be simultaneously removed without loss of low-abundance HCPs. Up to a 34-fold increase in the enrichment of HCPs can be achieved by using ProteoMiner beads comparing to direct digestion. After applying ProteoMiner beads on AAV products, HCPs at a level as low as 0.1 ng/mL can be detected.

**1:25 Affinity Capture, Characterization, and Activity-Based Profiling of Host Cell Proteins (HCPs)**
*Michael Dolan, Staff Engineer, Process Development US, Takeda Pharmaceuticals*
*Sunny Zhou, PhD, Professor, Chemistry & Chemical Biology, Northeastern University*
Despite advances in protein purification, host cell proteins (HCPs) remain a serious concern for protein therapeutics, as they may affect both product quality and immunogenicity in patients. In this talk, we will discuss our new methodologies toward the affinity capture, enrichment, and characterization of "problematic" HCPs. In more deeply understanding the fundamental chemical nature of HCPs, we enable the development of more targeted solutions for their removal.

**1:55 Presentation to be Announced** UNCHAINED LABS

**2:25 Networking Refreshment Break**

## DETECTION, CHARACTERIZATION, AND CONTROL OF THE PROCESS- AND PRODUCT-RELATED IMPURITIES

**2:40 Antibody Impurity Assessment via Integration of Mobile Affinity Selection Chromatography with Automated Data Analysis**
*Harsha Gunawardena, PhD, Principal Scientist, Mass Spectrometry, Janssen Pharmaceutical Companies of Johnson & Johnson*
we present an integrated approach for the analysis of two critical quality attributes of mAbs, namely titer and relative aggregate content. Integration of sample preparation and molecular recognition-based analyses were achieved in a single step utilizing an isocratically eluted Mobile Affinity Selection Chromatography (MASC) column. MASC circumvents the protein A step, simplifying sample preparation.

**3:10 Protein A ELISA Platform Method Development Comparing Two Commercial Kits**
*Theresa O'Brien, Scientist, Sanofi*
Residual Protein A is a process-related impurity that needs monitoring, due to potential safety considerations. A new Protein A resin was introduced into the process which uses a new commercially available ELISA (kit 1). Challenges during qualification using kit 1 led to the use of a well-established Protein A commercial kit 2. This presentation will focus on the challenges observed transitioning between the two commercial Protein A ELISA kits.

**3:40 Session Break and Transition to Plenary Keynote Session**

## PLENARY KEYNOTE SESSION: SOLVING TODAY'S CHALLENGES

**4:20 Organizer's Remarks**
*Daniel Barry, Senior Conference Director, Cambridge Healthtech Institute*

**4:30 PLENARY KEYNOTE PRESENTATION: READY: Addressing Current Challenges in Biomanufacturing with Reliability, Efficiency, Agility, Data, and (High) Yields**

***Jerry A. Murry, PhD, Senior Vice President, Process Development, Amgen***

The biopharmaceutical sector is currently producing vast amounts of data, a trend set to amplify with smart sensors, PAT, and process automation. This presentation will highlight the significance of a holistic digital strategy, incorporating AI, machine learning, predictive modeling, and data visualization, to spearhead the evolution of biomanufacturing. Emphasizing enhanced efficiency and innovation, this strategy will enable the efficient manufacture of complex biologics with reliability of supply, agility, and differentiation.

**5:10 Plenary Q&A**

**5:30 Welcome Reception in the Exhibit Hall with Poster Viewing**

**6:30 Close of Day**

## TUESDAY, AUGUST 20

**7:30 am Registration and Morning Coffee**

## ACCELERATED STABILITY STUDIES AND PREDICTIVE TOOLS

**7:55 Chairperson's Remarks**

***Pinaki Ranadive, PhD, Senior Scientist, Formulation Development Group, Regeneron Pharmaceuticals***

**8:00 Water Loss from Silicone Tubing and Effect on Protein Concentration during Drug Product Manufacturing**

***Pinaki Ranadive, PhD, Senior Scientist, Formulation Development Group, Regeneron Pharmaceuticals***

Silicone tubing is used in various unit operations during drug product (DP) manufacturing. Hold of protein formulations in semi-permeable silicone tubing over time may have an impact on product quality, particularly protein concentration. In this work, a semi-empirical mechanistic diffusion-based model was developed that predicts the change in protein concentration over various hold times for a given formulation and tubing size, and hence supports DP process development and validation.

**8:30 Rapid Profiling, Fingerprinting, and Speciation of Polymeric Excipients in Biotherapeutic Products**

***Ross Yang, Scientist, Merck Research Labs***

Polymeric excipients—such as polysorbate 20/80 and poloxamer 188, used in formulation of biotherapeutics—share the same building block which is polyethylene oxide. Charge-reduction mass spectrometry coupled with two-dimensional ion density mapping has been used for rapid profiling, fingerprinting, and speciation of polymeric excipients. This approach has proven to be a fast and effective tool for the visualization of polymeric species from the intact structure.

**9:00 Using Protein Language Models to Predict Polyreactivity of Antibodies**

***Michail Vlysidis, PhD, Senior Engineer, AbbVie***

It is crucial to assess antibody polyreactivity early on to minimize potential risks. I will discuss an ensemble model created within AbbVie which can accurately predict outcomes in both the baculovirus particle and bovine serum albumin assays. To train this model, we utilized a vast dataset of sequences, enriched with experimental conditions, obtained through a highly efficient application. The resulting models displayed strong and consistent performance across various antibody types.

**9:30 Presentation to be Announced**

Pfanstiehl

**10:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**10:45 Breakout Discussion Groups**

Breakout Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussions page on the conference website for a complete listing of topics and descriptions.

**IN-PERSON ONLY BREAKOUT: Can We Use a Mass Spec-Only Strategy for HCP Characterization?**

***Thomas Kofoed, PhD, Co-Founder & CEO, Alphalyse, Denmark***

- In which situations will it be relevant to use an MS-only strategy?
- What will it require to use an MS-only strategy
- What are the potential hurdles of using an MS-only strategy?

**11:30 CMC Analytical Comparability Strategies for Biotech and Gene Therapy Products**

***Kevin Zen, PhD, Senior Director, IGM Biosciences***

The comparability study is to assess the effect of manufacturing changes on product quality. In this presentation, I will overview the current thinking of health authorities on the comparability in complex biotech and gene therapy products, highlight the comparability strategies, and share the industry practices to ensure continuous product quality throughout the product lifecycle.

**12:00 pm The Protein Stabilising Capability of Surfactants against Agitation- and Surface-Induced Stresses**

***Michelle P. Zoeller, PhD, Senior Scientist, Liquid Formulation R&D, Merck Life Science KGaA***

The application of surfactants, mainly polysorbates, is a common practice to prevent surface- or agitation-induced protein aggregation in liquid formulation. However, polysorbates, despite their common application, bring along disadvantages, including chemical and enzymatic instability. This presentation will provide an overview of the protein-stabilising capability of surfactants against agitation- and interface-induced stresses, and corresponding assays for its evaluation. Furthermore, a focus is set to alternative surfactants suitable to replace polysorbates.

**12:30 Presentation to be Announced**

CYGNUS TECHNOLOGIES
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**1:00 Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**1:30 Refreshment Break in the Exhibit Hall with Poster Viewing**

**2:10 Chairperson's Remarks**

***Jianmei D. Kochling, PhD, Senior Director, Head of Analytical Development and QC, mRNA Center of Excellence, Sanofi***

**2:15 KEYNOTE PRESENTATION: What We Know and Do Not Know about Analytical Testing for mRNA Characterization**
***Jianmei D. Kochling, PhD, Senior Director, Head of Analytical Development and QC, mRNA Center of Excellence, Sanofi***
Despite the deepened analytical understanding of the mRNA molecule structure and mechanism of action and function of mRNA-LNP, the unique properties of IVT mRNA molecules and the mRNA-LNP complex add challenges to the characterization of the drug substance and drug product. This presentation will illustrate what we have learned from the past few years and what we still need to work on for mRNA-LNP characterization.

**3:15 Characterization for mRNA Therapies**
***Francis Poulin, PhD, Vice President, Analytical Sciences, Sail Biomedicines***
Introducing Sail Biomedicines' platform and discussing various methods for the analysis of circular RNAs. The presentation will identify key challenges in the analytical development of high-quality Endless RNA (eRNA). The discussion will focus on purity evaluation of circular RNAs and a novel AEX-HPLC analytical method used for eRNA.

**3:45 Refreshment Break in the Exhibit Hall with Poster Viewing**

**4:30 Quality Control and Analytical Characterization of mRNA LNP Drug Products in Early Clinical-Phase**
***Eivor Örnskov, PhD, Principal Scientist, AstraZeneca***
The presentation will outline critical quality attributes of mRNA lipid nanoparticle (LNP) drug products, with a focus on early clinical phases. It will also address potential impurities and degradation pathways pertinent to mRNA LNP formulations. A selection of key analytical methods essential for quality control and analytical characterization will be showcased.

**5:00 PANEL DISCUSSION: Analytical Techniques for Characterization of RNA and mRNA Products**
***Moderator: Jianmei D. Kochling, PhD, Senior Director, Head of Analytical Development and QC, mRNA Center of Excellence, Sanofi***
***Panelists:***
***Francis Poulin, PhD, Vice President, Analytical Sciences, Sail Biomedicines***
***Eivor Örnskov, PhD, Principal Scientist, AstraZeneca***
***Khaled Yamout, Analytical Sciences, Quality and Manufacturing, Consultant Y-Chem Consulting, LLC***

**5:30 Close of Rapid Methods to Assess Stability and Impurities in Biologics Conference**

## WEDNESDAY, AUGUST 21

**7:30 am Registration and Morning Coffee**

### HIGH-CONCENTRATION PROTEIN FORMULATIONS

**7:55 Chairperson's Opening Remarks**
*Kanika Sarpal, PhD, Senior Scientist, Biologics Drug Product Development, Sanofi*

**8:00 Understanding Formulation and Process Needs for High-Concentration Protein Therapeutics**
*Kanika Sarpal, PhD, Senior Scientist, Biologics Drug Product Development, Sanofi*
High-concentration protein therapeutics have become more popular as they favor subcutaneous (SC) administration. Successful development of high dose biologics requires adopting certain formulation approaches to overcome technical challenges such as viscosity, solubility, stability, process issues, and delivery limitations. There is no one approach that fits all. This talk will outline some key aspects while designing high concentration protein therapeutics from the formulation and process standpoint.

**8:30 Ongoing Challenges and Considerations to Develop High-Concentration Protein Formulation**
*Jia He, Senior Scientist, Amgen*

**9:00 One-Step Formulation Development of Biologics**
*Slobodanka (Dina) Manceva, Associate Director Drug Product and Technology Development, Teva Branded Pharmaceuticals*
The accelerated timelines in the evaluation of novel drug products and getting 1st to the market, demand a fast formulation development. Here we present one step global formulation development approach that is able to select a formulation based on malty factor interaction in less than 4 months.

**9:30 Anatomy of High-Concentration Biologics**
*Twinkle Christian, MS, Senior Scientist, Amgen, Inc.*
High-concentration biologics are complex to manufacture and deliver with patient centric initiatives. This presentation will focus on the design space with an optimized TPP (target product profile), early engagement of pivotal multidisciplinary stakeholders, interdependency of critical attributes during product development and key patient centric milestones across product development lifecycle of a high-concentration biologic.

**10:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**10:40 KEYNOTE PRESENTATION: Applying Deep Learning to Predict High-Concentration Antibody Viscosity**
*Pin-Kuang Lai, PhD, Assistant Professor, Department of Chemical Engineering and Materials Science, Stevens Institute of Technology*
Highly concentrated antibody solutions are necessary for developing subcutaneous injections but often exhibit high viscosity. We measured a large panel of 229 antibody viscosity to develop predictive models for screening viscosity at high concentrations. DeepViscosity was developed based on artificial neural network models to classify low-viscosity and high-viscosity antibodies at 150 mg/mL. The DeepViscosity model exhibited an accuracy of 87.5% and an AUC score of 90% on 16 independent antibodies.

**11:40 Automated Formulation Development across Modalities**
*Peter Soler, PhD, Senior Research Investigator, Bristol Myers Squibb Co.*
Biologics drug development has experienced rapid growth in recent years. To meet the need biologics formulation development has quickly acquired a set of automation tools and analytical techniques to provide robust drug products for patients. This has motivated the adaptation of our tools to meet the increases in process complexity for the benefit of patients globally.

**12:10 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:40 Refreshment Break in the Exhibit Hall with Poster Viewing**

### NOVEL DRUG DELIVERY TECHNOLOGIES & DEVICES

**1:25 Chairperson's Remarks**
*Sean Bedingfield, PhD, Senior Advisor, Lilly Genetic Medicine, Eli Lilly and Company*

**1:30 AAV Drug Product Local Delivery Administration Device Consideration**
*Xin Jin, PhD, Scientist, Biological Drug Product Development, Sanofi*
Adeno-associated viruses (AAVs) have been widely used as the delivery vehicles for CNS gene therapies. Intra-cisterna magna (ICM) administration was one of the local delivery administrations, which has benefit of widespread transgene delivery in both brain and spinal cord. This presentation summarized the work of an AAV drug product ICM administration device selection and studies for both animal tox study and clinical trial study.

**2:00 RNA Delivery in the Central Nervous System**
*Sean Bedingfield, PhD, Senior Advisor, Lilly Genetic Medicine, Eli Lilly and Company*
The clinical use of small interfering RNA (siRNA) and antisense oligonucleotides has required, in some cases, the implementation of invasive routes of administration such as intrathecal or intraocular injection. However, improved durability is mitigated by clearance of siRNA. We present a microcapsule-based method to extend activity of cholesterol-conjugated siRNA locally. We show that microcapsules protect the siRNAs from being cleared and enable release over 3 months compared to unencapsulated siRNAs.

**2:30 Sponsored Presentation** *(Opportunity Available)*

**3:00 Refreshment Break in the Exhibit Hall with Poster Viewing**

### PLENARY FIRESIDE CHAT: LEADING TO TOMORROW'S ADVANCES

**3:50 Organizer's Remarks**
*Nandini Kashyap, M.Pharm., Senior Director, Conferences and Social Media Strategy, Cambridge Innovation Institute*

**4:00 Genetic Medicines—Transforming the Future of Biotherapeutics**




*Moderator: Ann Lee, PhD, CTO, Prime Medicine, Inc.*
*Panelists:*
*E. Morrey Atkinson, PhD, Executive Vice President, Chief Technical Operations Officer, Head, Biopharmaceutical Sciences and Manufacturing Operations, Vertex Pharmaceuticals Inc.*
*Manmohan Singh, PhD, CTO, Beam Therapeutics*
*Heidi Zhang, PhD, Executive Vice President, Head, Technical Operations, Tune Therapeutics*

**5:00 Networking Reception in the Exhibit Hall with Poster Viewing**

**6:00 Close of Day**

## THURSDAY, AUGUST 22

**7:30 am Registration and Morning Coffee**

### FORMULATION DEVELOPMENT OF CELL AND GENE THERAPIES

**7:55 Chairperson's Remark**
*Bharathi Vellalore, PhD, Senior Scientist, Therapeutics Development and Supply, Janssen Pharmaceuticals*

**8:00 Comparing the Outlook of Developability Assessment of Monoclonal Antibodies to AAV Therapeutics for Successful Lead Candidate Selection from Discovery to Development**
*Yogapriya Murugesan, Scientist I, Gene Therapy & Drug Product Development, Biogen*
Molecular properties that impact developability attributes and outcomes comprises of conformational, chemical, colloidal, and other interactions. These attributes are measured using relevant analytical methods to assess the developability/ manufacturability of the molecule in different formulation. Developability assessment of mAbs has been studied and applying this assessment using the right tools to new modalities such AAV will help streamline capsid selection and candidate selection from discovery to development for new modalities

**8:30 Drug Product Consideration for AAV-Based Gene Therapy Products**
*Paria Moxley, PhD, Scientist, Biologics Drug Product Development & Manufacturing, Sanofi*
Recombinant adeno-associated virus (AAV) has emerged as a promising gene delivery vector for the treatment of various diseases. There are marked differences in buffer selection for formulation development with AAVs and protein therapeutics, which must be considered in the context of product manufacturing, long-term storage, and shipping/handling. This entails screening for buffer pH, ionic strength, and the impact of added surfactants on stability/degradation trends.

**9:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**9:30 Breakout Discussion Groups**
Breakout Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussions page on the conference website for a complete listing of topics and descriptions.

**IN-PERSON ONLY BREAKOUT: Process Development and Manufacturing Considerations for Novel Modalities**
*Bharathi Vellalore, PhD, Senior Scientist, Therapeutics Development and Supply, Janssen Pharmaceuticals*
- Scale-out vs scale-up for allogeneic and autologous cell therapies
- Manufacturing considerations for lentivirus
- Large-scale manufacturing of gene therapies and other novel modalities

**10:30 FEATURED PRESENTATION: Concentrating siRNA by Ultrafiltration for Gene Therapy Applications**
*Ken K. Qian, PhD, Scientific Director, Eli Lilly & Co.*
The present study is focused on developing a fundamental understanding of the factors controlling the ultrafiltration behavior of a siRNA drug product during tangential flow filtration (TFF). A dependence of the filtrate flux on the logarithm of the siRNA concentration was observed, consistent with classical concentration polarization models. Our work demonstrates the importance of both concentration polarization and membrane fouling on the ultrafiltration behavior of highly concentrated solutions of siRNA.

**11:00 Cell Therapy Drug Product Development**
*Bharathi Vellalore, PhD, Senior Scientist, Therapeutics Development and Supply, Janssen Pharmaceuticals*
- Process considerations for manufacturing autologous and allogeneic cell therapy products
- Drug product considerations for hematological malignancies and solid tumor indications
- Clinical vs commercial supply chain needs: Integrated drug product design

**11:30 Sponsored Presentation** *(Opportunity Available)*

**12:00 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:30 Refreshment Break in the Exhibit Hall & Last Chance for Poster Viewing**

### LNPs & NOVEL DELIVERY APPROACHES

**1:05 Chairperson's Remarks**
*Niels Delamotte, Director Analytical Development, Etherna*

**1:10 Analytical Lifecycle Management to expand Analytical Capabilities in support of product/process development.**
*Niels Delamotte, Director Analytical Development, Etherna*
We'll discuss our strategy for overcoming some of the QC challenges and strengthening analytical capabilities in support of mRNA Drug Substance and LNP product and process development. It will delve into the development of some of the traditional analytics used within Quality Control as well as emerging trends in analytical techniques for more in-depth characterization. The goal is to share insights to foster open dialogue to collectively advance the field.

**1:40 Formulation Developability Assessment for Viral Vector Delivery Agents: A Closer Look into Physical and Functional Particle Assessment**
*Ahmet Bekdemir, PhD, Senior Scientist II, Formulation & Analytics, Novartis Institutes for BioMedical Research Inc.*
Maintaining the stability of viral vectors through formulation assessment is essential for cell and gene therapy products. In this presentation, I will describe a study conducted to evaluate the stability of particle characteristics and functional titer for lentiviral vectors under varying buffer, pH, and excipients conditions. Through our screening experiments and comprehensive analytics, I will discuss how stability for these complex modalities is multifaceted and requires careful investigation.

**2:10 Process Development and CMC Considerations for the Development of Prime Editor Lipid Nanoparticles to Correct Disease-Causing Mutations**

***Weiyi Li, PhD, Scientist II, Prime Medicine Inc.***

Prime editing is a next-generation genome editing technology that could theoretically correct up to 90% of known genetic variants associated with human diseases. We have developed a universal lipid nanoparticle (LNP) for the delivery of Prime Editors (PE) to the liver. This presentation will highlight process development and CMC considerations for the development of PE-LNPs and provide selected case studies for PE RNA components and LNP-formulated PE process unit optimization.

**2:40 Networking Refreshment Break and Transition into Town Hall Discussions**

## FACILITATED TOWN HALL DISCUSSIONS

**2:55 Facilitated Town Hall Discussions**

These Town Halls offer delegates the opportunity to participate in interactive discussions on important themes that were explored during the conference. Each Hall will have a host(s) to facilitate the conversation, and all are welcome to participate, share views and best practices and ask questions of colleagues.

**Harnessing ML/AI and Big Data for Biotherapeutic Development**

***Pin-Kuang Lai, PhD, Assistant Professor, Department of Chemical Engineering and Materials Science, Stevens Institute of Technology***

**Cell and Gene Therapy Manufacturing: In-House vs. Outsourced**

***Elben Guimaraes, Senior Manufacturing Manager, Upstream Manufacturing, Ultragenyx Pharmaceutical Inc.***

The decision of handling cell and gene therapy processes in-house or outsourcing them is crucial. This facilitated discussion explores the advantages and challenges of both approaches, analyzing their impact on cost, control, strategic direction, and innovation. Share experiences and best practices for managing internal and external manufacturing, while examining common scenarios faced by sponsors and vendors.

**Digital Transformation & AI in Bioprocess—What, Where, When, and How?**


***Christian Airiau, PhD, Global Head, Data Sciences, CMC, R&D, Sanofi***
***Irene Rombel, PhD, CEO & Co-Founder, BioCurie Inc.***

The bioprocessing industry is undergoing a digital revolution fueled by AI. This interactive session dives into current digital adoption and explores the latest trends in AI applications (AIML). Join the conversation to explore the potential of AI for process optimization and digital twins. Share real-world success stories and discuss ethical considerations along with potential workforce impacts.

**3:55 Close of Summit**

# STREAM #8
# DIGITALIZATION

The Digitalization stream delves into cutting-edge bioprocess innovation, exploring two critical themes: Automating Analytical Development and Digital Transformation & AI in Bioprocess. The first conference tackles transitioning from manual methods to agile, data-driven automation, offering enhanced speed and precision. The second dives deep into harnessing AI and machine learning, empowering researchers to predict, optimize, and accelerate process development and manufacturing. This Stream equips you with the tools and insights to propel your process to the next generation, leaving behind time-consuming traditional methods and ushering in a new era of data-driven bioprocess revolution. Don't miss this opportunity to network, learn, and shape the future of biomanufacturing!

## Conference Programs

**AUGUST 19-20**

Accelerating Analytical Development

View Program »

**AUGUST 21-22**

Digital Transformation and AI in Bioprocess

View Program »

## MONDAY, AUGUST 19

**8:00 am Registration and Morning Coffee**

### OPTIMIZING PLATFORMS AND WORKFLOWS

**9:55 Chairperson's Opening Remarks**
*Rosalind Ang, PhD, Associate Principal Scientist, Merck*

**10:00 Platform Validation for Process Impurities Workflows**
*Rosalind Ang, PhD, Associate Principal Scientist, Merck*
Successful biologic drug characterization demands meticulous identification and control of process impurities. This presentation will explore the development and implementation of a robust platform validation strategy for process impurities. We'll discuss critical parameters, analytical techniques, and best practices for ensuring comprehensive validation. Attendees will gain insights to streamline impurity characterization, enhance product safety, and meet regulatory requirements.

**10:30 Overcoming the Barriers to Further Adoption of MAM**
*Hao Zhang, PhD, Senior Principal Scientist and Team Lead, Pivotal Attribute Sciences, Amgen*
The advances of new therapeutic modalities drive the development of liquid chromatography (LC)-mass spectrometry (MS)-based Multi-Attribute Method (MAM). MAM has successfully demonstrated its capability in replacing some of the traditional chromatographic and electrophoretic testing methods for monitoring product quality attributes for both release and in-process testing. We list several hurdles encountered along the way of MAM adoption and discuss the approaches to overcome them based on the latest development efforts.

**11:00 From Insight to Impact: Prior Knowledge and Streamlined Workflows in Analytical Development**
*Weichen Xu, PhD, Director, Analytical Sciences, Macrogenics*
To expedite new medicines to patients, the biopharmaceutical industry is focusing on platform technologies and prior knowledge. The application in analytical development is not a one-size-fits-all approach, but a dynamic strategy shaped by the unique historical wealth of knowledge tied to each method at each company. Beyond this, streamlining processes plays an integral role in optimizing operational efficiency. This presentation discusses how MacroGenics strategically employs these approaches to accelerate analytical development.

**11:30 Sponsored Presentation** *(Opportunity Available)*

**12:00 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:30 Session Break**

### AUTOMATION AND MINIATURIZATION

**12:50 Chairperson's Remarks**
*Lasse Stach, PhD, Principal Investigator & Leader, Developability Profiling Team, GSK*

**12:55 ML-Enabled Image Analysis to Characterize Formulation Aggregates**
*Theodore Randolph, PhD, Professor, Chemical and Biological Engineering, University of Colorado*
Many drug product manufacturing processes require characterization of microparticulate products and contaminants. Machine learning analyses of flow imaging microscopy datasets can be used for these applications, including monitoring cell health and debris during manufacture of cell-based therapies, detection of particulate matter formed during processing of adjuvanted vaccine suspensions, and exploration of root-causes for protein aggregation. We will discuss advances in unsupervised and supervised machine learning for these analytical tasks.

**1:25 Automation for All: Developing Workflows for Broad Deployment**
*Jon Jurica, PhD, Principal Scientist, Analytical Research and Development, Merck & Co.*
The use of automation provides significant opportunities in biologics analytical development to enable increased efficiency and improved experimental design. At Merck, we have strategically positioned a group of automation experts with an explicit goal to develop user-friendly tools, templates, and designs that are shared with our scientists, including simple bench-top platforms and larger liquid handling systems. We discuss implementation of this strategy that has resulted in an automation-first mindset.

**1:55 Sponsored Presentation** *(Opportunity Available)*

**2:25 Networking Refreshment Break**

**2:40 Development of a Custom-Automated Method for AAV Capsid Titer in Gene Therapy Products**
*Matthew J. Lotti, Senior Research Associate II, Ultragenyx Pharmaceutical, Inc.*
For viral vectors used in gene therapies, monitoring concentration throughout manufacture is vital for product consistency and quality. Using automation to assess AAV capsid titer enhances throughput while reducing assay hands-on time. The following presentation describes the development of an AAV capsid titer assay that combines two forms of automation: automated sample preparation and automated immunoassay and analysis. The resulting assay produces high-throughput, accurate sample results while reducing hands-on time.

**3:10 Scaling Lab Automation: Proactive Semi-Automation in Assay Development for Efficient Transition to Full Automation**
*Kentaro Marchionni, Automation Engineer, Cellino Biotech*
Semi-automation is a proactive approach to assay development that ensures the entire process is aligned with assay requirements and is automation-compatible. Semi-automated assays are inherently designed with miniaturization and optimization considerations, ensuring seamless scalability and efficiency. This approach prevents redevelopment of assays that are incompatible with automation and greatly simplifies the process of transitioning them into fully automated systems.

**3:40 Session Break and Transition to Plenary Keynote Session**

### PLENARY KEYNOTE SESSION: SOLVING TODAY'S CHALLENGES

**4:20 Organizer's Remarks**
*Daniel Barry, Senior Conference Director, Cambridge Healthtech Institute*

**4:30 PLENARY KEYNOTE PRESENTATION: READY: Addressing Current Challenges in Biomanufacturing with Reliability, Efficiency, Agility, Data, and (High) Yields**
*Jerry A. Murry, PhD, Senior Vice President, Process Development, Amgen*
The biopharmaceutical sector is currently producing vast amounts of data, a trend set to amplify with smart sensors, PAT, and process automation. This presentation will highlight the significance of a holistic digital strategy, incorporating AI, machine learning, predictive modeling, and data visualization, to spearhead the evolution of biomanufacturing.

Emphasizing enhanced efficiency and innovation, this strategy will enable the efficient manufacture of complex biologics with reliability of supply, agility, and differentiation.

**5:10 Plenary Q&A**

**5:30 Welcome Reception in the Exhibit Hall with Poster Viewing**

**6:30 Close of Day**

## TUESDAY, AUGUST 20

**7:30 am Registration and Morning Coffee**

## PREDICTIVE MODELING AND MACHINE LEARNING IN BIOPROCESS ANALYTICS

**7:55 Chairperson's Remarks**
*Bo Zhai, PhD, Principal Scientist, Analytical Method Development, Janssen*

**8:00 Higher Throughput Antibody Characterization to Improve Candidate Quality and Enable Machine Learning**
*Lasse Stach, PhD, Principal Investigator & Leader, Developability Profiling Team, GSK*
At the interface between discovery and CMC, the developability team at GSK characterizes lead molecules to identify stable molecules for progression. Making use of significant investment in protein production facilities, we are now collecting biophysical data at a higher throughput and at near formulation strength. This talk will focus on how these rich data are used to improve candidate quality as well as to feed predictive models.

**8:30 *In silico* CQA Identification and Assessment**
*Michael Kim, PhD, Technical Development Senior Principal Scientist, Protein Analytical Chemistry, Genentech*
Protein therapeutics contain heterogenous product variants, often due to post-translational modifications (PTM). A specific PTM's criticality depends on its potential impact to a therapeutic's efficacy and safety, which is traditionally evaluated empirically. With the burgeoning rise in computational power and biological structure elucidation, we explore the use of *in silico* biophysical modeling—specifically thermodynamic integration for relative binding free energies—to inform functional impacts of PTMs.

**9:00 KEYNOTE PRESENTATION: Where Are the Data—Solving One Challenge at a Time for Developing Digital Technologies to Support All Phases of Analytical Method Lifecycle**
*Neeraj Agrawal, PhD, Director, Attribute Science Data Engineering, Amgen*
FAIR data is required to derive maximum value from the recent developments in generative AI, ML, and other digital technologies. Extraction of FAIR data from diverse source systems that are used throughout the lifecycle of analytical methods while maintaining data integrity, as required in the regulated environment, requires substantial investments. This presentation will showcase Amgen's strategy for developing digital technologies to support all phases of analytical method lifecycle.

**9:30 Sponsored Presentation** *(Opportunity Available)*

**10:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**10:45 Breakout Discussion Groups**
Breakout Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussions page on the conference website for a complete listing of topics and descriptions.

**11:30 Making Data Work for You—Transformational Data Analytics Solutions**
*Brian Good, PhD, Senior Research Advisor, Eli Lilly and Company*
As scientists, we have expected electronic data to deliver us, only to find we are subjugated by it. The time has come to realize the unfulfilled promise. New technologies like NoSQL, ontologies, and AI/ML are rushing towards us and have outmoded our current platforms. We will explore how these technologies are changing our laboratories and increasing the value we can bring to our organizations through streamlined information delivery.

**12:00 pm Capture and Assimilation of Historical Analytical and Process Data**
*Christina Vessely, PhD, Senior Consultant, CMC Analytics & Formulation Development, Biologics Consulting Group, Inc.*
The development of biologics generally spans years, and we build on our past experiences as we advance. As we start working on our BLA filing, we find ourselves floating in a sea of data with no clear direction, and often with databases that are only partially searchable. How do we assimilate our big data and how do we assure that future data will be better organized and more searchable?

**12:30 Sponsored Presentation** *(Opportunity Available)*

**1:00 Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**1:30 Refreshment Break in the Exhibit Hall with Poster Viewing**

**2:10 Chairperson's Remarks**
*Chaojie Wang, Scientist, Biologics, Bristol Myers Squibb Co.*

**2:15 SPECIAL PRESENTATION: Building a Roadmap for Implementation of the Multi-Attribute Method in QC**
*Diane McCarthy, PhD, Senior Scientific Director, Global Biologics, US Pharmacopeia*
While the multi-attribute method (MAM) has potential to improve the efficiency and specificity of analytical testing, several challenges remain to implementation in QC. This presentation will provide an overview of considerations and best practices for use of MAM in QC from <1060> Mass Spectrometry-Based Multi-Attribute Method for Therapeutic Proteins. An update on a study of MAM versus conventional methods, funded through a cooperative agreement with FDA, will also be provided.

## NEW STRATEGIES AND TECHNOLOGIES

**2:45 A Systems Biology Approach to Modeling CHO Cell Cultures and Predicting Outcomes**
*Bo Zhai, PhD, Principal Scientist, Analytical Method Development, Janssen*
CHO cell biopharmaceutical production faces challenges due to the demand for high-titer and complex molecules. The genome-scale metabolic model serves as a powerful tool for exploring cellular physiology and predicting cellular behaviors. By integrating omics data and advanced computational

techniques, it guides metabolic engineering strategies for bioprocess optimization. Moreover, the model will guide in-process analytical testing strategies ensuring consistent product quality across all stages of production.

**3:15 Analytical Insights into Innovative Biologics and Biosimilars: Unveiling the Key Differences in Analytical Development**

***Miha Vodnik, PhD, Senior Expert Science & Technology, Novartis***

Analytics represent a fundamental pillar for development of biosimilars and innovative biologics. Although they are both biopharmaceuticals, the analytical strategies diverge in terms of purpose, scope, methods, and timelines. Novartis has years of experience in development of biologics and has recently transitioned into a fully innovative medicines-focused company. This presentation aims to delineate the critical distinctions between biosimilars and innovative biologics, underscoring the scientific and organizational aspects of analytical development.

**3:45 Refreshment Break in the Exhibit Hall with Poster Viewing**

**4:30 Case Study: NGS for Deep Characterization**

***Chaojie Wang, Scientist, Biologics, Bristol Myers Squibb Co.***

Monoclonality is expected for a biologics-producing cell line. Retrospective analysis of clonality using Southern blot raised questions about clonality vs. genetic plasticity of a cell line. Long-read sequencing is an innovative assay for plasmid integration structure analysis. CRISPR/Cas9-targeted Nanopore long-read sequencing provided accurate information on the integration structure, and helped solve the clonality vs. plasticity issue. Using Southern, Sanger sequencing, and NGS as orthogonal assays confirmed the conclusions.

**5:00 Fully Automated Immuno-µPlaque Assay for Live-Attenuated Quadrivalent Dengue Vaccine Development**

***Yi Wang, PhD, Senior Scientist, Vaccine Analytical R&D Merck***

A 96-well plate format immuno-µPlaque assay was developed for a viral potency test to support the development of a live-attenuated quadrivalent dengue vaccine. Full automation of the assay via an integrated robotic system illustrated the potential of high-throughput cell-based analytics in the vaccine development space. A deep learning-based plaque-counting algorithm further accelerates the assay by providing analysts with precise analysis results and robust workflow.

**5:30 Close of Accelerating Analytical Development Conference**

## WEDNESDAY, AUGUST 21

**7:30 am Registration and Morning Coffee**

## DIGITAL AND DATA STRATEGY, INFRASTRUCTURE, AND QUALITY

**7:55 Chairperson's Remarks**
*Mark Duerkop, CEO, Novasign GmbH*
*Angela Li, PhD, Senior Scientist, Sanofi Pasteur*

**8:00 Bringing Data Analysis on Par with Data Generation Speed**
*Christoph Herwig, PhD, former Professor, Bioprocess Engineering, Vienna University of Technology; CPO, Fermify GmbH; Senior Scientific Advisor, Körber Pharma Austria*
No matter if in development or in manufacturing, biopharmaceutical companies swim in data. However, data is not analyzed due to multiple reasons: Missing availability, missing contextualization, different frequency, and different dimensionality. As a result, experiments are not based on previous knowledge, creating an unnecessary waste of resources and costs. This contribution shows how to automatically organize and analyze data at the speed of its generation.

**8:30 UX & Data Quality: Two Sides of the Digital Transformation Coin**
*Madalene Crow, Senior ISA Product Manager, Genentech Inc.*
Case Study presentation to explore the relationship between scientific user experience and high quality data set generation in the context of evolving scientific methods and digital transformation. Digital product innovation guiding principles, a model for informatics product team/scientific user partnership and a summary of lessons learned will be shared.

**9:00 Digitalization of Tech Transfer Strategies: Why and How**
*Niki Wong, PhD, Director Global Tech Operations CMC, Global Tech Operations CMC, AbbVie Operations Singapore Pte Ltd.*
Tech transfer projects have always been stigmatized with tight timelines and limited resources. This presentation would like to tackle this challenge of increasing effectiveness and efficiency of tech transfer challenges by considering lessons learned and what can be done better through digitalization.

**9:30 Presentation to be Announced**



**10:00 Coffee Break in the Exhibit Hall with Poster Viewing**

## STRATEGIES FOR AI/ML APPLICATIONS IN BIOPROCESSING

**10:40 Use of AL/ML in Modeling and Simulation of Upstream and Downstream Bioprocesses for Vaccine Development**
*Angela Li, PhD, Senior Scientist, Sanofi Pasteur*
Hybrid models involving the combination of machine learning (ML) and physical knowledge hold promise to increased efficiency in process development. Case studies will be presented where a hybrid model of microbial cell culture process provides the flexibility and speed needed to bring value at early stages of process development. The presentation will also share the development of a novel hybrid model framework for modeling chromatography processes.

**11:10 Application of AI and Digital Twins for Bioprocessing: Pitfalls and Solution Paths for Accelerated Process Development and Automated Process Control**
*Mark Duerkop, CEO, Novasign GmbH*
In the slowly evolving landscape of bioprocess development and manufacturing, digital bioprocess-twins have emerged as potential accelerators. This presentation will illuminate the essential stages in developing robust process models, encompassing experimental design, customized modeling strategies, smooth scale-up processes, and the real-time application of models for effective monitoring and control. Concrete examples from both upstream and downstream processes will be provided to enhance comprehension of these principles.

**11:40 Perspectives on the Digitalization of the Biomanufacturing Industry**
*Antonio R. Moreira, PhD, Vice Provost, Academic Affairs & Advanced Technology Center, University of Maryland, Baltimore County*
The biopharmaceutical industry is undergoing a major transformation on the heels of the introduction of Pharma 4.0 concepts. The digitalization of the industry is impacting all aspects of biopharma from discovery, research, development, and manufacturing. New products as well as legacy products are exploring how to best employ these tools to optimize benefits. This presentation will discuss current and future perspectives of the digitalization of biopharma.

**12:10 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:40 Refreshment Break in the Exhibit Hall with Poster Viewing**

## KEYNOTE SESSION: THE FUTURE IN DIGITAL BIOMANUFACTURING

**1:25 Chairperson's Remarks**
*Mark Duerkop, CEO, Novasign GmbH*
*Angela Li, PhD, Senior Scientist, Sanofi Pasteur*

**1:30 KEYNOTE PRESENTATION: Global Digital Transformation Program—It's All About Data Consumption**
*Christian Airiau, PhD, Global Head, Data Sciences, CMC, R&D, Sanofi*
Sanofi CMC/Process Development is transforming the way we develop new medicines by driving a data centric approach at the core of our activities.Three use cases are presented to demonstrate how we support Sanofi projects by applying innovative methodologies of quantitative sciences, leveraging empirical, hybrid and mechanistic models to design, optimize and control our processes. We also share our vision for a Digitally mature, AI-enabled process development organization.

**2:00 KEYNOTE PRESENTATION: Applications of Machine Learning in Antibody Discovery, Process Development, Manufacturing, and Formulation: Current Trends, Challenges, and Opportunities**
*Bogdan Gabrys, PhD, Professor of Data Science, Data Science Institute, School of Computer Science, University of Technology Sydney*
While machine learning (ML) has made significant contributions to the biopharmaceutical field, its applications are still in the early stages in the development and manufacturing of biologics, hindering the enormous potential for bioprocesses automation from their development to manufacturing. In this talk we will discuss current applications, the main challenges, and offer insights into the adoption of innovative ML methods in the development of new digital biopharma solutions.

**2:30 Efficiency and Robustness in Process Development for Bio-Production** YOKOGAWA

*Soichiro Shimoda, Manager, Business Design, Yokogawa Electric Corp.*

Yokogawa Electric Corporation is a leading provider of process automation for more than 50 years. Expertise are in technologies for sensing, analyzing, controlling and information management for industrial automation. We would like to share our experience and efforts in the biopharmaceutical industry, such as inline sensing and advanced control algorithms using techniques represented by modeling and machine learning, aiming to realize efficiency and robustness in bio-production.

**3:00 Refreshment Break in the Exhibit Hall with Poster Viewing**

## PLENARY FIRESIDE CHAT: LEADING TO TOMORROW'S ADVANCES

**3:50 Organizer's Remarks**

*Nandini Kashyap, M.Pharm., Senior Director, Conferences and Social Media Strategy, Cambridge Innovation Institute*

**4:00 Genetic Medicines—Transforming the Future of Biotherapeutics**

*Moderator: Ann Lee, PhD, CTO, Prime Medicine, Inc.*
*Panelists:*
*E. Morrey Atkinson, PhD, Executive Vice President, Chief Technical Operations Officer, Head, Biopharmaceutical Sciences and Manufacturing Operations, Vertex Pharmaceuticals Inc.*
*Manmohan Singh, PhD, CTO, Beam Therapeutics*
*Heidi Zhang, PhD, Executive Vice President, Head, Technical Operations, Tune Therapeutics*

**5:00 Networking Reception in the Exhibit Hall with Poster Viewing**

**6:00 Close of Day**

## THURSDAY, AUGUST 22

**7:30 am Registration and Morning Coffee**

## MODELING AND SIMULATION IN UPSTREAM AND DOWNSTREAM PROCESS DEVELOPMENT

**7:55 Chairperson's Remarks**

*Christian Airiau, PhD, Global Head, Data Sciences, CMC, R&D, Sanofi*

**8:00 Evaluating Molecular-Scale, Coarse-Grained Mayer Sampling Simulations for Predicting the Self-Association of Commercial Monoclonal Antibodies**

*Jonathan Janke, PhD, Scientist, Biologic Drug Product Development and Manufacturing, Sanofi*

Screening for CMC protein liabilities is a crucial, although costly, step in mAb drug product development. The diffusion interaction parameter, kD, has been demonstrated to be a highly useful predictor for CMC liabilities, and kD, in conjunction with B22, can be predicted using molecular-scale simulations. After parameterizing coarse-grained simulations, we have determined that these simulations are both robust and efficient for predicting self-interactions of monospecific, commercial mAbs.

**8:30 Closed-Loop Control of Fed-Batch Bioreactors for Monoclonal Antibody Production**

*Anastasia Nikolakopoulou, Investigator—Modeling and Simulation, Pharmaceutical Development, R&D Medicinal Science and Technology, GSK*

In this talk, we discuss model predictive control (MPC) strategies for CHO fed-batch cell culture. MPC strategies have been investigated for their potential to achieve consistent end-of-run titer in the presence of unexpected process disturbances (i.e., iVCC deviations, pH or temperature controller errors). First, we discuss two different modeling frameworks and their integration with MPC. Then, we compare the impact of process disturbances on the process with and without MPC.

**9:00 Coffee Break in the Exhibit Hall with Poster Viewing**

**9:30 Breakout Discussion Groups**

Breakout Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussions page on the conference website for a complete listing of topics and descriptions.

**IN-PERSON ONLY BREAKOUT: Post Model Establishment: Meaningfully Implementing Models in Process Development**

*Terrence Dobrowsky, PhD, Head, Gene Therapy Drug Substance, Biogen*

**IN-PERSON ONLY BREAKOUT: Digital Bioprocessing and Industry 4.0: How Far along Are We?**

*Mark Duerkop, CEO, Novasign GmbH*

This interactive roundtable discussion will cover the following topics:
- Critical evaluation of the current industrial evolution?
- AI vs. mechanistic modeling: what to choose?
- Workflow vs. data: where to invest?
- Outlook—how AI will change the way of bioprocessing in the future?

**10:00 Quantifying Catabolism to Predict and Model the Kinetics of CHO Cell Cultures**

*Sergio Rossell, PhD, Expert Scientist, Upstream Development, GSK*

Mammalian cell lines require complex media. Cells utilize the nutrients available to them as building blocks for biosynthesis, but also as substrates from which they derive the energy to drive biosynthesis and cell maintenance. Here we show how the rates of catabolic reactions can be dissected from the rest of metabolism, and show that catabolism governs the rates of growth and product and byproduct formation in antigen-producing CHO cells.

**10:30 CFD Simulations for Efficient Upscaling of Stem Cell Production in Bioreactors**

*Ramon van Valderen, PhD Candidate, Delft University of Technology*

*Ex-vivo* cultivation of iPSCs for the production of red blood cells is a promising therapeutic alternative to donor-based cell transfusion, yet scale-up of this bioprocess remains challenging. In this work, highly-resolved large-eddy simulations were performed to compare the hydrodynamics of a 125mL shake

flask and 250mL bioreactor for various operating conditions, to help translate shake flask operating conditions to bioreactor operating conditions, which ultimately contributes to faster process development times.

**11:00 Industry Maturity Models as the North Star for Digital Transformation**
***Eugene Tung, PhD, Executive Director, Manufacturing IT, Merck & Co., Inc.***

**11:30 Sponsored Presentation** *(Opportunity Available)*

**12:00 pm Luncheon Presentation** *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

**12:30 Refreshment Break in the Exhibit Hall & Last Chance for Poster Viewing**

## MODELING AND SIMULATION IN UPSTREAM AND DOWNSTREAM PROCESS DEVELOPMENT (CONT.)

**1:05 Chairperson's Remarks**
***Anastasia Nikolakopoulou, Investigator—Modeling and Simulation, Pharmaceutical Development, R&D Medicinal Science and Technology, GSK***

**1:10 A DoE Approach to Identify and Model the Design Space for Worst-Case Upstream Bioprocessing**
***Wilhad H. Reuter, Lead Engineer, Upstream Process Development, Mural Oncology, Inc.***
Worst-case studies are a facet of late-stage process characterization that are used to model the combination of factors that have the least desirable outcome in a manufacturing process. In this case study, both screening and response surface DoEs were executed to identify the highest risk factors on a 14-day fed-batch cell culture process. These models were then leveraged for designating the Upstream Control Strategy AORs prior to PPQ manufacturing.

**1:40 A Novel Digital Twin for Enhancing rAAV Production in Sf9/Baculovirus Cultures**
***Francesco Destro, PhD, Postdoctoral Associate, Chemical Engineering, Center for Biomedical Innovation, MIT***
This work introduces a groundbreaking digital twin designed to enhance the production of recombinant-adeno-associated virus (rAAV) within baculovirus/Sf9 cultures—a platform responsible for producing 50% of commercial rAAV-based gene therapies. A mechanistic model is developed to systematically identify bottlenecks within the intracellular pathway for full rAAV capsid formation in producer cells. After experimental validation, the digital twin indicates genetic modifications and process enhancements aimed at boosting overall platform productivity.

**2:10 Digital Twin Strategy for Continuous Manufacturing of Biologics: Case Study**
***Pedro de Azevedo Delou, Senior Consultant Engineer, Siemens Industry Software***
***Robert Taylor, PhD, Associate Scientist, Bioseparation Sciences, Merck Manufacturing Division***
Through this work, we designed and conducted *in silico* DOE runs, decreasing the number of experiments, material, and the overall program timeline and costs of process development and commercialization phases. Currently, we are initiating our first mechanistic models for some of the operation units, and attempt to generate first feedback controls through integration of tangential flow filtration models as soft sensors for membrane fouling.

**2:40 Networking Refreshment Break and Transition into Town Hall Discussions**

## FACILITATED TOWN HALL DISCUSSIONS

**2:55 Facilitated Town Hall Discussions**
These Town Halls offer delegates the opportunity to participate in interactive discussions on important themes that were explored during the conference. Each Hall will have a host(s) to facilitate the conversation, and all are welcome to participate, share views and best practices and ask questions of colleagues.

**Harnessing ML/AI and Big Data for Biotherapeutic Development**
***Pin-Kuang Lai, PhD, Assistant Professor, Department of Chemical Engineering and Materials Science, Stevens Institute of Technology***

**Cell and Gene Therapy Manufacturing: In-House vs. Outsourced**
***Elben Guimaraes, Senior Manufacturing Manager, Upstream Manufacturing, Ultragenyx Pharmaceutical Inc.***
The decision of handling cell and gene therapy processes in-house or outsourcing them is crucial. This facilitated discussion explores the advantages and challenges of both approaches, analyzing their impact on cost, control, strategic direction, and innovation. Share experiences and best practices for managing internal and external manufacturing, while examining common scenarios faced by sponsors and vendors.

**Digital Transformation & AI in Bioprocess—What, Where, When, and How?**

***Christian Airiau, PhD, Global Head, Data Sciences, CMC, R&D, Sanofi***
***Irene Rombel, PhD, CEO & Co-Founder, BioCurie Inc.***
The bioprocessing industry is undergoing a digital revolution fueled by AI. This interactive session dives into current digital adoption and explores the latest trends in AI applications (AIML). Join the conversation to explore the potential of AI for process optimization and digital twins. Share real-world success stories and discuss ethical considerations along with potential workforce impacts.

**3:55 Close of Summit**

# Sponsorship Programs

**CII offers comprehensive packages that can be customized to your budget and objectives. Sponsorship allows you to achieve your goals before, during, and long after the event. Packages may include presentations, exhibit space, and branding, as well as the use of delegate lists. Signing on early will maximize your exposure to qualified decision-makers and drive traffic to your website in the coming months.**

## Podium Presentations

***Available within Main Agenda!***

Showcase your solutions to a guaranteed, targeted audience through a 15- or 30-minute presentation during a specific program, breakfast, lunch, or a pre-conference workshop. Package includes exhibit space, on-site branding, and access to cooperative marketing efforts by CII. Lunches are delivered to attendees who are already seated in the main session room. Presentations will sell out quickly! Sign on early to secure your talk.

## One-on-One Meetings

CHI will set up 6-8 in-person meetings during the conference, based on your selections from the advance registration list. Our staff will handle invites, confirmations and reminders, and walk the guest over to the meeting area. This package also includes a meeting space at the venue, complimentary main-conference registrations, branding, an 8'x10' exhibit space, and more.

## Invitation-Only Dinner/Hospitality Suite

Select specific delegates from the pre-registration list to attend a private function at an upscale restaurant or a reception at the hotel. From extending the invitations, to venue suggestions, CII will deliver your prospects and help you make the most of this invaluable opportunity.

## Exhibit

Exhibitors will enjoy facilitated networking opportunities with qualified delegates, making it the perfect platform to launch a new product, collect feedback, and generate new leads. Exhibit space sells out quickly, so reserve yours today!

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- Badge Lanyards
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- Padfolios and More…

## For more information regarding exhibits and sponsorship, please contact:

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# 2023 Attendee Demographics


- 79% Biotech
- 5% Services
- 4% Financial
- 3% Academic
- 3% Government
- 2% Healthcare
- 1% CRO
- 1% Societies
- 1% Press


- 72% East Coast
- 19% West Coast
- 9% Midwest

- 86% USA
- 9% Europe
- 3% Asia
- 2% Rest of World


- 35% Scientist/Technologist
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- 2% Assistant
- 1% Professor

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