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

Cambridge Healthtech Institute's

16TH ANNUAL BIOPROCESSING SUMMIT

Time is running out – reserve your spot now!

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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#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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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

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Your partner in life sciences workforce development

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

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

Instructor:

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

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.

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 PhD, Director, Data Science, Lumilytics

Varsha Daswani, PhD, PMP, Senior Director, Analytics and Data Science, 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 changes are a critical component of drug development, impacting both CMC (Chemistry, Manufacturing, and Controls) and process development teams significantly. Robust potency assays are crucial not only for these studies but also for process validation and stability testing. This 1.5-day training seminar offers a comprehensive exploration of regulatory science and biological standardization in biologics. The seminar details the nature of potency, highlights the differences in potency assays across biotech and cell and gene therapy products, and discusses the principles of comparability and their varied applications across these sectors.

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

Instructor:

Kevin Zen, PhD, Senior Director, IGM Biosciences

The chemistry manufacturing and controls (CMC) of biologics is a multidiscipline technical operation of bioprocess, analytics, dosage formulation, and cGMP manufacturing/testing for DS/DP release and stability to treat human diseases. This interactive training course will provide a comprehensive CMC overview of therapeutic biological products. It introduces a variety of therapeutic modalities including recombinant proteins, monoclonal antibodies (Mab), and cell and gene therapy (CGT) in the context of IMPD and IND regulatory filing. Attendees 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. Numerous real-world regulatory queries/comments from health authorities worldwide will be exemplified as case studies during the training course.

Please check our website for an updated agenda.

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 Generation of Fucosyltransferase 8 Knock-out CHO-K1 Host Cell Lines
Tiffany McLamarrah, PhD, Sr Scientist, Mammalian & Cellular Assays, Sanofi

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 From Genetic Design to Bioprocess Performance: Toward Holistic Optimization of Upstream Bioprocesses Across Modalities ASIMOV
Will Johnson, Head of Process Modeling, Asimov
Progress toward holistic optimal design of genetic circuits, cell lines, and bioprocesses across monoclonal antibody, cell and gene therapy, and mRNA therapeutic platforms using a portfolio of mechanistic, data-driven, and hybrid models. Serial application of genetic, cellular, and bioprocess models enables monoclonal antibody titers from 4 to 11 g / L in fed batch culture, and lentiviral functional titers greater than 1x108TU / mL in batch culture.

12:00 pm LUNCHEON PRESENTATION: Speeding up Transient HEK293 and Transient/Stable CHO from 96 well, 24 well, 6 well, 125mL-7L Optimum Growth flasks THOMSON INSTRUMENT COMPANY
Sam Ellis, CEO, Thomson Instrument Co
Conditions for Plasmids, Transient HEK293 and Transient/Stable CHO from 96, 24, 6 well, 125mL-7L Optimum Growth flasks need to be maintained at a small scale. Data will be presented on techniques and technology that allow for getting high amounts of protein in smaller volumes with fast techniques from 1mL-3L. This allows teams to get to IND molecules quickly. All of these techniques are proven technologies for protein production, structural biology, and can lead to successful clinical candidates

12:30 Session Break

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

12:55 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.

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 HT Antibody Quantity & Quality Analytics on Your Plate Reader with the Valita Titer and Valita Aggregation Pure Assays BECKMAN COULTER Life Sciences
Paul Sweeney, Sr Product Dev Scientist, Beckman Coulter Ireland
Cell line & process development are still some of the largest rate-limiting steps in the bioprocessing end of bringing a new drug to market. Ensuring suitable high throughput, automation friendly & cost-effective bio-analytics are in place is critical in preventing process bottlenecks. Here we describe the use of Valita Titer & Valita Aggregation Pure Assays: simple, high-throughput & automation friendly plate-based antibody quantification & quality assays to streamline your antibody workflows.

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
Nayiri Kaissarian, PhD, ORISE Fellow, OTAT DPPT, FDA CBER
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

4:25 Chairperson's Remarks
Tara Jones, PhD, Senior Vice President, Global Drug Product Operations, Moderna

4:30 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 One-to-One Interview, with Audience Q&A
Jerry A. Murry, PhD, Senior Vice President, Process Development, Amgen

5:10 Talk Title to be Announced
Tara Jones, PhD, Senior Vice President, Global Drug Product Operations, Moderna

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 Platform for Process Analytical Methods for mAb Production: Upstream Monitoring, Process and Product Characterization
Jonathan Bones, Principal Investigator, Natl Institute for Bioprocessing Research & Training NIBRT
Biopharmaceutical industry is seeking robust solutions for PAT and process analytical methods to support upstream production. Here, an integrated platform enabled rapid generation of informative data on the manufacturing process behaviour: Key process parameter monitoring was done using 908 Devices MAVERICK in-line Raman spectroscopy, media profiling with REBEL at-line cell culture analyser; and rapid product quality assessment with ZipChip microchip capillary electrophoresis interface coupled to an Orbitrap Exploris MX mass detector (Thermo Scientific).The high frequency and coverage of the data collected throughout the bioprocess is crucial for process modelling. Temporal sampling enabled the visualisation of trends in the behaviour of individual proteoforms present over the duration of the culture including the dynamics of the N-glycans present on the expressed IgG. When combined with media profiling data obtained from REBEL and Raman data from MAVERICK, a deeper insight into process-product interplay was gene

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.

TABLE 1: Advances in Cell Line Engineering
Susan Sharfstein, PhD, Professor, Nanobioscience, Nanoscale Science and Engineering, University of Albany
- Multi-omics approach
- Precision gene editing with CRISPR
- Targeted integration and NGS
- Cell-free systems for production
- AIML in cell line engineering

TABLE 2: Cell Culture Processes: Better Through AI Technology?
Angela Botros, Process Modelling Scientist and Machine Learning Expert, DataHow
- Is there a potential for Upstream cell culture development to advance on the statistical methods and find new solutions?
- Status Quo in Cell Culture
- From CQAs to Process Dynamics
- Mechanistic Modelling vs Data-driven (Machine Learning) Modelling
- Potential of AI-driven Technology for Cell Culture Development
- Accelerating Insights by Simplifying Tasks

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 Talk Title to be Announced

Gilad Doron, Scientist I, AAV Cell Line Development, Resilience

12:45 C.STATION: End-to-End Automation for Generating Stable Cell Lines for the Development of Advanced Therapeutics

John Carroll, Regional Sales Manager Biopharma East Coast, 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.

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 Targeted Model Building for Bioprocessing Applications Using Raman Spectroscopy

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

Nipun Goel, Sr Scientist, Sanofi

This presentation explores computational modeling and mathematical approaches that leverage data-driven insights and quantitative models to enhance our ability to predict and optimize cell culture processes. By bridging experimental biology with computational tools, we can unlock new avenues for improving yield, reproducibility and scalability in cell-based applications.

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, Vaccine CMC Development & Supply, Sanofi

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 Enhancing Bioprocess Monitoring and Understanding by Leveraging Benchtop NMR
Victor Beaumont, Strategic Market Dev. Specialist, Bruker
Gabriella Gerzon, PhD Candidate, York Univ
Despite the recognized efficiency of continuous biopharmaceutical manufacturing, it has yet to be widely adopted because of the inherent complexity of biologics and bioprocesses. Optical spectroscopies are typically employed for process monitoring but lack the level of structure characterization achieved by NMR. This presentation demonstrates the power of benchtop NMR as a well-suited technique to complement existing technologies for enhanced bioprocess monitoring and understanding.
This work was done in the context of the acquisition of an NMR instrument by Sanofi, and in agreement with the company.
Authors: Victor Beaumont*, Christian Fischer*, Matteo Pennestri* *Bruker International

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

STRATEGIES FOR AI/ML APPLICATIONS IN BIOPROCESSING

10:40 Use of AI/ML for Bioprocess Systems Modeling and Applications in Vaccines CMC Development
Angela Li, PhD, Senior Scientist, Vaccine CMC Development & Supply, Sanofi
We discuss the strategy of implementing process modeling for production of complex biotherapeutics and how models powered by ML can be useful to drive data value continuum along the CMC development cycle. Case studies will be presented on the use of hybrid, mechanistic, and data-driven models for upstream and downstream, including potential applications of a novel physics-informed machine learning algorithm.

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 Industry Maturity Models as the North Star for Digital Transformation
Eugene Tung, PhD, Executive Director, Manufacturing IT, Merck & Co., Inc.
As companies in the pharmaceutical manufacturing industry undergo digital transformations, they face numerous questions around the transformation. What are the goals? What are the benefits? What does good look like? In this presentation, we examine digital transformation through the lens of digital plant maturity models, which help companies understand the current state of their plants and prioritize their digital investments as they progress up the maturity curve.

12:10 pm LUNCHEON PRESENTATION: Leveraging Digital Threads for Seamless Data and Process Automation, Traceability, and Reproducibility
Tara Madhyastha , Sr. Solutions Architect , Rescale
Advanced modeling and simulation are increasingly key to speeding up bioprocessing. These tools are powerful, but need to be made easy to use without specialists to generate actual performance gains. Our luncheon will explore best practices for automating simulations, ensuring data provenance, and supporting FAIR principles for digitalization efforts. Learn how Rescale customers are simplifying complexity in R&D and boosting simulation speed and throughput to cut time to discovery.

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

KEYNOTE SESSION: THE FUTURE IN DIGITAL BIOMANUFACTURING

1:25 Chairperson's Remarks
Moo Sun Hong, PhD, Assistant Professor, Department of Chemical and Biological Engineering, Seoul National University

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, Yokogawa Electric Corp.
Shahzad Khan, , Yokogawa Electric Corporation
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.

2:45 **Advancing Bioprocessing with AI-Native MLOps: Case Studies and Prospects** INVERT

Karthik Sekar, PhD, Invert
Invert is a cutting-edge AI-native software platform, purpose-built as an MLOps solution for bioprocessing. This talk will:
Introduce Invert's innovative approach to managing data for bioprocess optimization
Highlight outcomes of collaborations in identifying critical process parameters
Demonstrate our AI-driven experimental design methodologies
Preview upcoming features, including advanced temporal process prediction
Join us to explore how Invert is transforming bioprocessing efficiency and accelerating innovation in the field.

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

PLENARY FIRESIDE CHAT: LEADING TO TOMORROW'S ADVANCES

3:50 **Plenary Introduction**
Daniel Barry, Senior Conference Director, Cambridge Healthtech Institute

3:55 **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 **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.

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** MACHEREY-NAGEL

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.

TABLE 1: Post Model Establishment: Meaningfully Implementing Models in Process Development
Terrence Dobrowsky, PhD, Head, Technology Development and Implementation, Takeda
- What do you consider when deciding on entry points for modeling in process development?
- How do you enable engagement and use of models in development workflows?
- What does '*in silico* first' mean to different stakeholders?
- What are the biggest hurdles when incorporating models directly into process control?

TABLE 2: 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?

10:30 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.

11:00 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.

11:30 In-house vs Off-the-Shelf Solutions for a Digital R&D Ecosystem

Victor Sanchez Tarre, PhD, Director, Data Science, Manufacturing Science & Technology, MeiraGTX

- Factors to be considered when deciding on building an in-house software solution vs. off-the-shelf offerings
- Tools to build a successful digital ecosystem—from specific code packages to ELN and data exploration platforms of choice
- Infrastructure supporting Data Engineering and Data Science functions
- Challenges—assay nomenclature harmonization, standardization of data capture and data processing workflows, and strategies to routinely generate FAIR data

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

SGS

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 - IN PERSON ONLY

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.

Town Hall 1: 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

Town Hall 2: 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.

Town Hall 3: Digital Transformation & AI in Bioprocess Development and Manufacturing

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

Alois Jungbauer, PhD, Professor & Head, Biotechnology, Institute of Bioprocess Science and Engineering, University of Natural Resources and Life Sciences (BOKU)

10:00 Optimization Strategies for Developing Robust Perfusion and Harvest Methods for High Cell Density Mammalian Cultures

Jessica Pedroso, 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 Redefining the Economics of Upstream mAb and Viral Vector Bioprocessing: How Process Intensification Boosts Productivity REPLIGEN

Rachel Legmann, PhD, Senior Director of Technology, Gene Therapy , Repligen Corp

Viral vectors higher complexity compared to monoclonal antibodies creates additional production and purification challenges to overcome. This presentation will present few case studies on how integrated solutions with intensified production, continuous harvest, purification, and process analytical tools simplify and speed up the process, increase lentivirus (LV) and adeno-associated virus (AAV) titer yield, driving down gene therapy drugs cost down by more than 50%, and enable upstream-downstream continuous processing.

11:45 LUNCHEON PRESENTATION: Continuous Processing at Any Scale PAK BioSolutions

Joanna Pezzini, CEO, PAK BioSolutions

PAK BioSolutions is expanding its range beyond the Pilot scale system, which purifies 50-500L cell culture/day. Single-use flow paths & hardware were adapted to create 3 separate systems with capabilities ranging from 2L/day to over 20,000L/day. The broad processing ranges necessitated different fluid handling components, while the control strategies remained largely consistent. Challenges included titrations, instrument accuracy, hardware integration, & varying fluid dynamics.

12:00 pm LUNCHEON PRESENTATION: Delivering a Solution to Meet the Increasing Demand for Sterile Filtration of High Concentration Drug Products cytiva

Rajeshwar Chinnawar, Sr Engineer, Cytiva

Drug formulations are changing, and the share of subcutaneous administration over intravenous infusion is increasing. The subcutaneous administration trend has gained traction and is seen to be the preferred approach both by patient and healthcare providers due to the potential benefits of reduced infusion time and enhanced patient convenience. With the development and manufacture of subcutaneous biologics come challenges in the sterile filtration of challenging feeds that are both highly concentrated (>100 g/L) and viscous (10-30 cP). A larger filter must be used to compensate for decreased capacity and this results in the loss of high value product in hold-up volumes in both the filter membrane and capsule.
In this presentation we will share the current trends and challenges with the manufacture of high concentration drug products, and the solution to help you confidently meet these challenges.

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 Introducing the Next Cycle of Affinity Resin Innovation Purolite

Yair Peres, Field Specialist V, Purolite, An Ecolab Company

Purolite, an Ecolab Company, will present its next-generation high-capacity protein A resin that demonstrates improved stability with low ligand leakage. The resin aims to increase productivity and cost reductions. Building on the success of Praesto™ Jetted A50 HipH, this protein A continues to address aggregation and impurity clearance through a wider pH elution window.

2:25 Networking Refreshment Break

2:40 Green Metrics to Reduce Environmental Impact of Biologics
Felix Dieringer, PhD Student, BOKU University
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

4:25 Chairperson's Remarks
Tara Jones, PhD, Senior Vice President, Global Drug Product Operations, Moderna

4:30 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 One-to-One Interview, with Audience Q&A
Jerry A. Murry, PhD, Senior Vice President, Process Development, Amgen

5:10 Talk Title to be Announced
Tara Jones, PhD, Senior Vice President, Global Drug Product Operations, Moderna

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
Madiha Khurshid, Process Development Senior 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
Robert S. Gronke, Senior Principal Scientist, Technical Development, 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 Breaking the $40 per Gram Barrier for mAbs with Fully-Connected Continuous Manufacturing (FCCM)
Himanshu Gadgil, CEO, Enzene Biosciences Ltd
Traditional biologics manufacturing relies on batch processing, which involves sequential operations and faces significant limitations, such as low productivity, lengthy processing times, and high operational costs due to large infrastructure needs and frequent manual interventions. To overcome these challenges, Enzene has introduced a commercially validated, fully-connected continuous manufacturing platform (FCCM) for protein-based therapeutics. This platform, EnzeneX™, operates continuously from upstream to downstream, enhancing productivity with a smaller facility footprint. It achieves higher cell densities and viabilities, resulting in up to ten times

greater productivity than traditional methods. EnzeneX™ 2.0 aims to reduce mAb production costs to less than $40 per gram, making life-saving medicines more affordable and accessible globally.

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.

TABLE 3: 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?

TABLE 4: 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 Cell Separation: Optimize Critical Process Parameters with Tubular Bowl Centrifugation
Dan Nelson, Director, Product, CARR Biosystems
This session will present case studies to illuminate critical process parameter considerations such as shear, separation efficiency and process speed when developing separation processes including primary clarification, cell retention, wash and concentration. The audience will learn how tubular bowl centrifugation works and how the tunable parameters and scalability enable process efficiency.

1:00 LUNCHEON PRESENTATION: Holographic Microscopy and Machine Learning – Transforming Cellular Assays
Hans-Joachim Muhr, Business Development Manager, METTLER TOLEDO
Cell counting is a critical step in cellular assays, enabling researchers to ensure a consistent number of cells for their experimental analyses. Recent advances in holographic microscopy and machine learning have transformed cellular assays development. This innovative technique provides a label-free and non-invasive approach for identifying and distinguishing healthy, dying, and dead eucaryotic cells. The integration of machine learning techniques allows for automatic cell classification, reducing result variability due to stain intensity, microscope settings, and user-dependent classification. This approach enhances the reliability of viability measurements between samples, while also addressing common issues present in other cellular analytical techniques.We will discuss the potential of holographic microscopy and machine learning in improving the accuracy and consistency of cellular assays.

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

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, Process Development Senior Scientist, Pivotal Drug Substance Technologies, 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 POSTER HIGHLIGHT: Development of an Improved Upstream Process to Accelerate Multi-Modality Drug Discovery Pipeline

Keshab Rijal, PhD, Principal Scientist, Process Development, Amgen, Inc.

Amgen developed an improved upstream process combining a robust new cellular host and a novel cell culture bioprocess. The increased yield from this improved process is relevant in particular for multi-specifics that can be difficult to express, emphasizing the benefit of continuously improving upstream processes. The new CHO host attained robust growth and high cell viability in both traditional fed-batch and perfusion cell culture processes.

5:15 POSTER HIGHLIGHT: Process Intensification with Single Use Technologies and the Impact on Economic and Sustainability Metrics

Matthew Peters, Bioprocess Applications Specialist, Purification and Filtration, Solventum

In this presentation, we will discuss how our modeling shows that advanced single-use technologies deployed at the clarification and polishing unit operations can have a positive impact on key economic and sustainability metrics. We will show how using these technologies can lead to increased total output as well as reduced consumable waste, buffer consumption, lower PMI, and decreased CO_2e intensity.

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 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, 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 Viral Clearance in the Age of Q5A(R2); Case for Non-Infectious Spiking Agents

David Cetlin, Senior Director, R&D, MockV Products, Cygnus Technologies

To determine viral clearance efficacy of biomanufacturing steps, viruses are "spiked" into in-process solutions, processed and analyzed for reduction. Due to the infectivity of these viruses, studies are conducted in BSL-2 facilities. Costs and logistics limit analysis during process development. The revised Q5A(R2) guidelines which govern the biotechnology industry on the topic of viral safety and evaluation provides an update to the regulatory stance on viral safety including the use of "Prior Knowledge". Cygnus Technologies, through the MockV® product line, offers solutions which ease the accumulation of viral clearance data. Supplementing a company's existing prior knowledge with MockV® derived clearance data could effectively reduce the scope and/or need of conducting process-specific live viral validation spiking studies.

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, Senior 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: A Proprietary Alkaline-Stable Protein A Resin for Improved Evaluation, Scale-up and Efficiency in mAb Purification

Simona Serban, Director, Global Life Science Applications, Sunresin New Materials Co. Ltd.

Dr. Simona Serban introduces a proprietary rProtein A ligand and agarose base matrix creating new possibilities for purification of mAbs and Fc-containing biomolecules. The talk will cover:
-Development of an alkaline-stable ligand through fermentation and purification
-Optimization of a highly cross-linked agarose base matrix and ligand coupling
-mAb affinity purification performance
-mAb Purity, Yield and HCP clearance following three chromatography steps

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

1:25 Chairperson's Remarks

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

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 Optimizing Biotherapeutic Purification with Buffer Concentrates - An In-Depth Analysis

Philip Hansel, Associate Scientist IV, Downstream Process Dev, Alexion Pharmaceuticals Inc.

Buffer requirements for large-scale purifications present significant facility storage and resource demands. Buffer concentrates, which minimize these constraints, have been successfully implemented in thirteen 500 L pilot production runs at Alexion, AstraZeneca RDU. An inline dilution system has produced buffers within tight tolerances as measured by offline pH, conductivity, and density measurements. The system has reduced buffer volumes by 70%, preparation time by 66%, and storage space by 50%.

2:30 High Productivity Protein A Membrane Devices Complement Disposable Upstream Technology for a Fully Single-Use Process

William Barrett, Product Specialist, WL Gore & Associates Inc

An intensified and fully single use downstream operation was demonstrated to process a monoclonal antibody cell culture harvest at a manufacturing scale. The results of the study were extrapolated to show the potential for high productivity affinity capture sufficient up to 10 g/L titers at the 2000 L scale.

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

PLENARY FIRESIDE CHAT: LEADING TO TOMORROW'S ADVANCES

3:50 Plenary Introduction

Daniel Barry, Senior Conference Director, Cambridge Healthtech Institute

3:55 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.

TABLE 4: 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

TABLE 5: Alternative Approaches to ProteinA Affinity Chromatography

Jean-Francois P. Hamel, PhD, Lecturer, Chemical Engineering, Massachusetts Institute of Technology

In this round-table discussion, we will discuss the recent advances in non-affinity and mixed-mode chromatography, their format (e.g., packed-bed versus monolith), and their applications in the analytical and process environments, such as for ionic and hydrophilic drugs, antibodies and proteins, alkaloids, oligonucleotides, and peptides. The development of analytical methods and processes for pharmaceuticals will also be discussed under the framework of Quality-by-Design.

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 Navigating Large Biomolecule Purification Challenges with Novel Solutions BIO-RAD

William H. Rushton, MS, Chromatography Support Scientist, Bio Rad Laboratories

Large biomolecule purification presents significant challenges due to their considerable size. Purification of molecules like immunoglobulins is particularly intricate owing to several factors, including their weak Protein A binding sites, limited dynamic binding capacity, and slow diffusion rates. This technical session will explore innovative strategies using design of experiments (DOE) methodologies and case studies on plasma protein and virus purification. Advanced chromatography resins can improve efficiency, reduce processing times, and enhance product purity, bolstering productivity and scalability in the biopharmaceutical sector.

11:45 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 SGS

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 - IN PERSON ONLY

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.

Town Hall 1: 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

Town Hall 2: 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.

Town Hall 3: Digital Transformation & AI in Bioprocess Development and Manufacturing

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

CHALLENGES AND COMPRIMISES WHEN COMMERCIALIZING GENE THERAPIES

9:55 Chairperson's Remarks

James Richardson, PhD, Senior Director, Analytical Development, Interius BioTherapeutics

10:00 KEYNOTE PRESENTATION: Advancing Viral Vector Gene Therapies—Impact of Technical Solutions on Development Timeline, Development Cost, and COGs

Jinpian Diao-Piezunka, PhD, Head, Technical Development, Spark Therapeutics, Inc.

Multiple AAV manufacturing platforms co-exist. While much attention was given to understand each platform's full potential in reaching high product purity and low cost of goods, the true impact of the manufacturing platform decision is largely determined by the specific context of each asset under development. This presentation will discuss the business impact of choosing different AAV manufacturing platforms on development timeline, development cost, and cost of goods in commercial.

10:30 KEYNOTE PRESENTATION: Technical Development and Comparability Strategies for Gene Therapies

Phillip Ramsey, Senior Vice President, Technical Operations, 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.

11:00 PANEL DISCUSSION: GENE THERAPY CMC LEADERS PANEL

Moderator: James Richardson, PhD, Senior Director, Analytical Development, Interius BioTherapeutics

Panelists:

Jinpian Diao-Piezunka, PhD, Head, Technical Development, Spark Therapeutics, Inc.

Phillip Ramsey, Senior Vice President, Technical Operations, Sangamo Therapeutics

James Warren, PhD, Senior Vice President, Global CMC Development, Ultragenyx Pharmaceutical

11:30 Next level AAV and lentivirus characterization with Stunner, Uncle, and Leprechaun

Kevin Lance, Market Director, Gene Therapy, Unchained Labs

Taking AAV and lentivirus characterization to the next level means faster and more thorough answers with minimal method development. Stunner delivers fast, precise, and standard-free capsid titer, empty/full ratio, and aggregation for any AAV you can throw at it. Uncle checks out AAV stability from every angle in a single experiment. Leprechaun dishes out the titer for capsid- and RNA-containing lentivirus from both crude and pure samples.

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

Raphael Gübeli, Vice President, 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

CHARACTERIZING GENE THERAPIES

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 In-line and on-line Monitoring of CQAs for Biologics, Vaccines and Gene Vectors

Dan Some, Sr Principal Development Engineer, Waters | Wyatt Technology

Real-Time Multi-Angle Light Scattering is a key Process Analytical Technology in drug development, especially for complex drugs like gene vectors. It provides instant feedback on quality attributes such as molar mass and particle size, essential for monitoring product identity and purity. RT-MALS is particularly effective for gene vectors like AAV, tracking empty-full ratios and titers. This talk discusses RT-MALS' principles, capabilities, limitations and case studies in bioprocessing.

2:10 Navigating Potency Assay Evolution in Gene Therapy

Huda Naas, Bio-assay Development Associate III , Ascend Advanced Therapies

Huda Naas will explore critical aspects of potency assay evolution in gene therapy, illustrated through an insightful case study highlighting the challenges and innovative solutions in this evolving field. A summary of analytical and quality methods to support assay readiness from early to late phase programs will be displayed. The presentation will include:

Introduction to Ascend
Global sites overview
Analytical capabilities & leveraging of analytics for program success
Potency assay development
Case study on potency assay

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

4:25 Chairperson's Remarks
Tara Jones, PhD, Senior Vice President, Global Drug Product Operations, Moderna

4:30 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 One-to-One Interview, with Audience Q&A
Jerry A. Murry, PhD, Senior Vice President, Process Development, Amgen

5:10 Talk Title to be Announced
Tara Jones, PhD, Senior Vice President, Global Drug Product Operations, Moderna

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

CHARACTERIZING GENE THERAPIES

7:55 Chairperson's Remarks
Xiaohui Lu, PhD, Director, Analytical Development, Ultragenyx Pharmaceutical

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
Residual host-cell DNA is a safety concern in gene therapy products, necessitating analytical tools for evaluating residual DNA mass and size. This presentation will report on development of a linker-ligation/PCR-based method for characterizing residual DNA size distribution, as well as on evaluation of PCR-based methods for quantifying residual host-cell DNA. Using these tools, a performance comparison of commercial nucleases during lentiviral downstream processing will be presented.

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 Viral Clearance Strategies for Gene Therapy Products
Akunna Iheanacho, Chief Operating Officer, Texcell North America Inc
An overview of recent regulatory revisions, viral clearance study strategies, and considerations that are unique to gene therapy products.

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: Formulation Strategies for Gene Therapies
Kruti Soni, PhD, Scientist, Technical Development, Biogen

IN-PERSON ONLY BREAKOUT: Gene Therapy Analytics
Aishwarya Bapat, PhD, Scientist, Process Development, Moderna

POTENCY ASSAYS, IN-PROCESS TESTING

11:25 Chairperson's Remarks
Kruti Soni, PhD, Scientist, Technical Development, Biogen

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 Optimizing Viral Vector and Plasmid Production through Real-Time Monitoring and Control with Single-Use Sensors

Nick Troise, Technical Support and Innovation Manager , PendoTECH (Mettler toledo)

Real-time, in-process analytics is a critical challenge for the efficient development and production of gene therapies. Single use sensors are a powerful analytical tool that can help address this challenge by optimizing the yield and quality of viral vector and plasmid production. In this presentation, we will review PendoTECH single use sensing technologies that enable the precise monitoring and control of key parameters such as pressure, turbidity, and conductivity.

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.

3:15 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

Xiaozhi Ren, PhD, Director, Plasmid and Cell Line Development, Nvelop Therapeutics

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

Switching from adherent to suspension manufacturing platform during late-stage clinical development requires rigorous risk analysis and a comprehensive comparability study. Here, we used a risk-based approach to investigate the impact of several key factors on the critical quality attributes of our AAV gene therapy product. We evaluated the effects of cell lines, media types, enhancers, and harvest conditions based on suspension process to ensure pre- and post-changes are similar.

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 3PBIOVIAN

Timothy Cinq-Mars, Head of Business Development, 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 sbi SCIENTIFIC BIOPROCESSING

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: Key Considerations in Designing a Production Facility for Gene Therapy Reagents teknova:

Stephen Gunstream, CEO, Teknova

Nicky Young, Senior Director, Sterility Assurance, Teknova

Designing a production facility for gene therapy reagents requires meticulous planning to meet the quality and customization demands. Few facilities support the flexibility required nor meet the GMP standards for the small-scale manufacture of made-to-order products where sterility, process flow, and layout are critical. Learn how Teknova built their new, modular ISO 13485-certified facility to meet the demands of GMP-grade reagents for gene therapy development and commercialization.

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

1:25 Chairperson's Remarks

David McNally, Director, Process Development, MassBiologics

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 Leveraging MVDA to Predict rAAV Titer Production of New Novel Capsids and Associated Scale Up

Andrew Schrock, Engineer, Voyager Therapeutics

rAAV technology for gene therapy is rapidly improving, increasing the demand for high yield manufacturing processing techniques. These processing strategies require monitoring of a large number of factors that are all interconnected and impact the overall performance of a run. Multivariate data analysis (MVDA) is a mathematical tool that can be leveraged to generate predictive models of desired outputs such as upstream titer yield.

2:30 BioRMB Platform - a Column-free Process Intensification for Continuous Purification of AAV and mRNA

Oleg Shinkazh, CEO, Chromatan Inc

The BioRMB™ is a column-free and steady-state purification platform that is specifically tailored for sensitive modalities such as gene therapies, vaccines, mRNA, and complex antibodies. In this work we will present the general principles of operation for the BioRMB™, as well as showcase AAV and mRNA capture and viral removal studies. The platform shows >80% reduction in resin volume vs. batch columns, higher recovery and lower HCP.

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

PLENARY FIRESIDE CHAT: LEADING TO TOMORROW'S ADVANCES

3:50 Plenary Introduction

Daniel Barry, Senior Conference Director, Cambridge Healthtech Institute

3:55 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 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 2.7×10^8 TCID50 mL^{-1} in a 3L batch process and 1.1×10^9 TCID50 mL^{-1} in a perfusion culture for HSV-1 and competitive titers for other viruses.

8:30 Process Development and Manufacturing of Novel Large-Capacity Recombinant Parvovirus Gene Therapy Vectors

Shu-Hao Liou, PhD, Principal Scientist, Process Development, Carbon Biosciences

Carbon Biosciences is leveraging the genetic diversity of non-AAV parvoviruses to develop novel vectors with increased capacity, minimal seroprevalence, specific tropism and liver-detargeting properties. Carbon has demonstrated successful vectorization of capsids with capacity of 5.5kb and a robust, scalable manufacturing process resulting in high productivity and yield. Carbon platform technology aims to alleviate many of the challenges with current gene therapy and provides optionality for diseases requiring larger genetic constructs.

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.

As recombinant AAVs (rAAVs) gain prominence as gene delivery vehicles, rAAV production has evolved from research-oriented processes to scalable industrialized operations. Decades' experience of biologics process development framework has sped up this evolution. However,

more nuanced understanding of rAAV CQAs, product heterogeneity and product degradation is critical for process design. The talk will illustrate key strategies to enable development of robust downstream manufacturing process that deliver quality product consistently.

11:30 Streamline AAV Titer Analysis with Automated Immunoassay Technology

GYROS PROTEIN Technologies

Maria Gianneli, Product Manger, Gyros Protein Technologies

By adopting advanced analytical tools, the industry can achieve greater reproducibility, improved productivity, and faster time to results. In the quest to advance AAV vector production, the development of automated AAV capsid titer assays represents a significant leap forward in analytical methodologies. The Gyrolab® technology, recognized for automation, precision and short turn-around, is adopted by several biopharma companies for AAV titer testing. In this talk a case study from a biopharma customer will illustrate the evaluation of the platform with aim to accelerate and improve the process development in AAV viral vector production.

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

SGS

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 - IN PERSON ONLY

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.

Town Hall 1: 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

Town Hall 2: 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.

Town Hall 3: Digital Transformation & AI in Bioprocess Development and Manufacturing

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, Chief Regulatory Scientist, Cellx Inc

10:00 KEYNOTE PRESENTATION: 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

The Cell Manipulation Core Facility (CMCF) at Dana Farber Cancer Institute (DFCI) in Boston, MA, works with academic investigators and biotech companies to implement GMP manufacturing of cellular products for early-phase (often first-in-human) IRB and FDA approved clinical trials that will enroll patients at DFCI, MGB/BWH, BCH, and other in/out of state institutions. CMCF currently supports over 15 different FDA IDs and implements 4-5 new manufacturing procedures every year.

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 NIST Flow Cytometry Standards Consortium Enables Quantitative and Comparable Measurements for Cell and Gene Therapies

Lili Wang, PhD, NIST Fellow, 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.

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 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 Mass Photometry instruments for in process analytics of Viral and LNP Gene Therapy Vectors

Matt Ranaghan, Senior Applications Scientist, Refeyn Inc.

Mass photometry is a revolutionary technology designed for quick and accurate assessment of biomolecule purity, stability, and heterogeneity. We present here the applications of two instruments designed for gene editing vectors: the SamuxMP for AAV, and the new KaritroMP for characterization of adenovirus (AdV), lentivirus (LVV), and lipid nanoparticles (LNPs). With experimental times of minutes per sample, these instruments remove bottlenecks for in-process analytics that are conventionally encountered with slower methods like analytical ultracentrifugation (AUC).

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

Cell Therapy CMC and Analytics

Improving Product and Process Quality Across All Stages of Development

AUGUST 19-20
All Times EDT

PLENARY KEYNOTE SESSION: SOLVING TODAY'S CHALLENGES

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

4:25 Chairperson's Remarks
Tara Jones, PhD, Senior Vice President, Global Drug Product Operations, Moderna

4:30 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 One-to-One Interview, with Audience Q&A
Jerry A. Murry, PhD, Senior Vice President, Process Development, Amgen

5:10 Talk Title to be Announced
Tara Jones, PhD, Senior Vice President, Global Drug Product Operations, Moderna

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, Principal CMC 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.

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.

BREAKOUT DISCUSSION: Potency Assays for Cell and Gene Therapies
Diana Colleluori, PhD, MBA, Principal CMC Consultant, CMC Analytical, Biologics Consulting Group

BREAKOUT DISCUSSION: Developing Successful Cell Therapies
Scott R. Burger, Principal, Advanced Cell & Gene Therapy LLC

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.

12:00 pm 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.

12:30 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.

1:00 LUNCHEON PRESENTATION: Overcoming Analytical Challenges and Safeguarding Product Quality with Rapid Sterility and Mycoplasma Detection Techniques

Speaker to be Announced, Thermo Fisher Scientific

Biotherapies, particularly cell therapy products, necessitate rigorous sterility and mycoplasma testing for patient safety. However, devising an analytical strategy for both sterility and mycoplasma testing can be daunting. The key is identifying analytical testing methods that can not only be utilized at the early stages of development but also subsequently scaled to address the challenges of biotherapeutic production. This presentation will delve into the challenges faced by manufacturers and discuss the advantages of adopting rapid sterility and mycoplasma detection techniques. By enabling the early detection of potential contamination in the production process, these techniques can help save time and resources while instilling confidence in the final product. For Research Use Only. Not for use in diagnostic procedures.

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 Points to Consider for Cellular Therapy

Akihiro Shimosaka, PhD, Chairman, Asian Cellular Therapy Organization (ACTO)

Autologous therapy is not for marketing but only for the single patient using patient cells. We need new concepts to regulate autologous cell therapy. New regulation in Japan and Taiwan started covering advanced therapy and US FDA approved autologous therapy for multiple myeloma last year. Japan's regulation has two pass ways: commercial product license application via PMDA or advanced therapy application via MOHLW.

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 Process Development on Cell Therapy Products

Yuechen Zhu, PhD, Staff 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

POINT-OF-CARE MANUFACTURING: 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 Quality-by-Design: Accelerating Cell Therapy Manufacturing
Joe Wong, Director, Project/Program Mgmt., Ascential Medical & Life Sciences
Cell therapy is advancing rapidly, but manufacturing has not kept up, leading to challenges in commercialization speed, cost, and quality management. We'll discuss Quality-by-Design (QbD) principles for Cell therapy to guide the complex development of manufacturing processes, ensuring high-quality, repeatable results. Key topics include QC challenges in cell therapy manufacturing, 4 steps to an end-to-end QbD process, and enabling technologies driving innovation in quality management.

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

11:10 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.

11:40 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.

12:10 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 Obstacles for Xenotransplantation
Knut Niss, PhD, President and Founder, Know The Edge Consulting

2:30 Streamline Cell Therapy Manufacturing with Scalable, GMP-compliant Electroporation

James Brady, Sr VP Tech Applications & Customer Support, MaxCyte Inc

As therapeutic developers use increasingly complex editing strategies in difficult cell types, achieving efficient delivery of transgenes and genome editing reagents without impacting cell viability or function is becoming more challenging. During this presentation, we present data showcasing MaxCyte's clinically validated electroporation platform for the efficient delivery of CRISPR-based editing reagents, transposons, and other molecules to T cells, NK cells, and macrophages, among others, while avoiding many of the pitfalls associated with other methods.

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

PLENARY FIRESIDE CHAT: LEADING TO TOMORROW'S ADVANCES

3:50 Plenary Introduction

Daniel Barry, Senior Conference Director, Cambridge Healthtech Institute

3:55 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

FDA UPDATES, DEVELOPING QUALITY TARET PRODUCT PROFILE

7:55 Chairperson's Opening Remarks

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

8:00 US Regulatory Update 2024: New Guidance Documents From FDA OTP

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

This presentation will summarize CGT-related FDA guidance released over the last year. These include CGT-specific guidance on potency assurance strategies, development of CAR T cell and gene-edited products, safety testing of *ex vivo* expanded allogeneic cells, and use of human-and animal-derived materials in manufacturing CGTs and tissue-engineered products. FDA's new platform technology designation program and final rule on BLAs and Master Files will also be discussed.

8:30 Development of the Quality Target Product Profile (QTPP) in Cell Therapy

Yemin Xu, PhD, Director, CMC Regulatory, Global Development, ProKidney Corp.

The quality target product profile (QTPP) guides cell therapy development for optimal safety and efficacy. This presentation explores the early implementation of a QTPP, considering product understanding, manufacturing experience, and overall control strategy. The presentation will further elucidate how a dynamic QTPP can inform strategic decision-making throughout development, ultimately mitigating potential risks.

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.

BREAKOUT DISCUSSION: Process Development for TILs

Kathryn L Aron, PhD, Director, Process Development, Obsidian Therapeutics, Inc.

BREAKOUT DISCUSSION: Promises and Challenges of Bioprocessing for PSC-Derived NK Cell Therapies

Allen Qiang Feng, PhD, Founder and CSO, HebeCell Corp.

PROCESS DEVELOPMENT FOR IPSC AND NK 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 Solving the Scale-up Bottleneck in iPSC Derived Cell Therapies Using a Novel CTF Perfusion Technology

Hongliang Zong, VP, Neukio Biotherapeutics

Shengquan Huang, Application Scientist, ALIT Biotech

iPSC derived cell therapy has the potential to treat broader patient population at a faster turnaround time for a variety of disease areas. However, it is challenging to achieve desired production scale using traditional technology, resulting in high cost and delays.
In this presentation, we will discuss how we significantly improved the cell density of iNK cells using a 15L stir tank bioreactor coupled with a novel CTF

perfusion system. Cell viability was maintained at >95% during intensified culture. Applications and results in several other iPSC derived cell types will also be discussed.

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 POSTER PRESENTATION: Multifaceted Application of the Cue Cell Processing System for Automated and Closed Manufacturing of CD34+ Hematopoietic Stem and Progenitor Cells (HSPCs)

Margaret Y. Zhang, Senior Research Associate II, Process Development, Beam Therapeutics

Autologous therapies for HSPCs require manufacturing processes that are high-throughput, scalable, and high-yielding. The Cue is a fully closed and automated platform device that has enabled high accuracy and temperature-controlled cell washing, concentration, formulation, and filling for small volumes. Here we show the successful application of the Cue for the preparation of CD34+ cell selection, harvest, final formulation, and filling drug product for HSPC manufacturing at Beam Therapeutics.

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

FACILITATED TOWN HALL DISCUSSIONS

2:55 Facilitated Town Hall Discussions - IN PERSON ONLY

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.

Town Hall 1: 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

Town Hall 2: 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.

Town Hall 3: Digital Transformation & AI in Bioprocess Development and Manufacturing

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

ADVANCING NUCLEIC ACID THERAPIES: REGULATIONS, QUALITY, PREDICTION AND MODELING

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
Minghua Liu, Consultant, CMC Biologics, Eliquent Life Sciences
mRNA 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 and interactions with the Agency, as well as guidance published.

10:30 DNA Starting Material Quality Limits: There is No 'One Size Fits All'
Lawrence C. Thompson, PhD, Associate Research Fellow, Analytical R&D, Pfizer Inc.
- What is a "DNA Starting Material"?
- Defining a DNA starting material analytical control strategy
- The impact of nicked DNA starting material on the quality of rAAV vs mRNA drug substance

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 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.

12:00 pm 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, Cell & Gene Therapy, Parexel

12:55 Biophysical Properties 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 Monolithic Columns for mRNA-LNP Therapeutics and Vaccines SARTORIUS
Andreja Gramc Livk, Head of Process Analytics, Sartorius BIA Separations
Lipid nanoparticles (LNPs) have emerged as the foremost non-viral carriers for therapeutics and vaccines due to their ability of encapsulating various payloads. They show limited adverse effects, are straightforward to formulate and are easier scaled than other viral delivery systems.
Post formulation, LNPs require downstream processing to ensure the formulation is applicable for *in vivo* applications and adherent to the desired specifications. Therefore, comprehensive, high resolution analytical tools for process monitoring and quality control (QC) of LNPs are critical for ensuring the patients' safety and product efficacy.
The presentation will outline diverse applications of monolithic column chromatographic analytics for monitoring critical quality attributes (CQAs) during mRNA LNP production, purification, and release. The monolithic column applications will include:
Two-dimensional chromatography tool for the analysis of LNPs with no sample pre-treatment. This approach enables determination of critical quality attributes, such as encapsulation efficiency, nucleic acid quantification and size distribution.
mRNA integrity analysis using ion pair-reverse phase approach.
Lipid composition analysis.
Using those, you can streamline your mRNA-LNP optimization and manufacturing, setting optimal critical process parameters.

2:25 Networking Refreshment Break

2:40 Advancing mRNA Process Development and CMC Strategies for Prime Editing from Pre-Clinical to IND
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 Drug Product Process Development
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

4:25 Chairperson's Remarks
Tara Jones, PhD, Senior Vice President, Global Drug Product Operations, Moderna

4:30 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 **One-to-One Interview, with Audience Q&A**
Jerry A. Murry, PhD, Senior Vice President, Process Development, Amgen

5:10 **Talk Title to be Announced**
Tara Jones, PhD, Senior Vice President, Global Drug Product Operations, Moderna

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 Remarks**
Niels Delamotte, Director Analytical Development, Etherna

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 **Aligning CMC and Regulatory Strategies for Gene Therapy Development**
Rajiv Gangurde, PhD, Vice President, Technical Operations, Cell & Gene Therapy, Parexel
With several FDA-approved products and >1600 ongoing clinical trials in the USA, gene therapy has emerged as a modality to challenge a wide variety of diseases. CMC has become front-and-center to the development of gene therapies. This talk covers key elements for developing phase-appropriate CMC strategies, including the critical role of early FDA interactions in developing a robust reg-CMC strategy, keeping up with evolving regulatory guidance, and balancing resources and timelines.

9:30 **Assessing Critical Quality Attributes (CQAs) of mRNA-LNPs: A Case Study on the Influence of Supercoiled DNA**
Ye Gu, PhD, Co-Founder & CTO, Crystal Bio

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.

mRNA Production Platform: Global Engineering Challenges and Opportunities
Amit Kumar, PhD, Global Engineering- Site Lead Biomanufacturing Facility Design and Capital Project, Moderna
- Facility Design Essentials: Key considerations for designing flexible, modular facilities.
- Global Process Standardization: Strategies for maintaining consistency and quality across multiple sites.
- Automation and Digitalization: Implementing Industry 4.0 solutions for streamlined production.
- Regulatory Compliance: Navigating global regulatory landscapes and ensuring GMP compliance.
- Technology Transfer: Best practices for seamless scale-up and technology transfer.

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 **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 panomebio

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

Eivor Örnskov, PhD, Principal Scientist, Pharmaceutical Sciences, 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

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

sbi SCIENTIFIC BIOPROCESSING

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: Blast through Biologics Formulation Studies with the Right Characterization Tools

UNCHAINED LABS

Andre Mueller, Marketing Manager, Biologics, Unchained Labs

High-concentration biologics are more and more popular and controlling their viscosity is a critical task, adding one more layer of complexity to formulation development. Unchained Labs' mission is to provide integrated solutions for finding out about quantity, quality, stability, and viscosity, while requiring small volumes and offering high throughputs. Join my talk to learn about our tailored solutions that help you blast through characterizing formulations of proteins, ADCs, and other biologics.

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

Enzo

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 Thermodynamics in Biopharmaceuticals: Ultra-High Concentration mAb Formulations
James K. Kranz, PhD, Vice President & Head, Chemistry Manufacturing & Controls, Compass Therapeutics Inc.

Thermodynamic assessment of formulations can provide key information on self-association behavior in crowded protein solutions that can predict long-term stability trends with respect to colloidal stability of candidate formulations.

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

FORMULATRIX

PLENARY FIRESIDE CHAT: LEADING TO TOMORROW'S ADVANCES

3:50 Plenary Introduction
Daniel Barry, Senior Conference Director, Cambridge Healthtech Institute

3:55 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 **Enjoy Lunch on your Own**

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

LNPs & NOVEL DELIVERY APPROACHES

1:05 **Chairperson's Remark**

Weiyi Li, PhD, Scientist II, Prime Medicine Inc.

1:10 **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.

1:40 **Formulation Developability Assessment for Lentivirus Vectors: 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 **Q & A with Speakers**

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

FACILITATED TOWN HALL DISCUSSIONS

2:55 Facilitated Town Hall Discussions - IN PERSON ONLY

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.

Town Hall 1: 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

Town Hall 2: 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.

Town Hall 3: Digital Transformation & AI in Bioprocess Development and Manufacturing


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, 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 Enjoy Lunch on Your Own

AUTOMATION AND MINIATURIZATION

12:50 pm 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, Director, Analytical Research and Development, Merck & Co., Inc.
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 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.

2:25 Networking Refreshment Break

2:40 Scaling Lab Automation: Proactive Semi-Automation in Assay Development for Efficient Transition to Full Automation
Michael Cheng. 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.

3:10 3D Printed Portable Fluorescence Microscope for Automated Single-Cell Enumeration for Advanced Biomanufacturing Applications
Umer Hassan, PhD, Assistant Professor, Electrical & Computer Engineering, Rutgers University
Cell enumeration is vital to determine the appropriate dosage of a cell-based therapy manufacturing process. Gold standard instruments require high capital costs, maintenance, and significant sample processing for its utility. Here, I will present our recently developed portable, 3D printed, florescence microscope capable of imaging and enumerating cells in a completely automated way at the point-of-care.

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

4:25 Chairperson's Remarks
Tara Jones, PhD, Senior Vice President, Global Drug Product Operations, Moderna

4:30 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 **One-to-One Interview, with Audience Q&A**
Jerry A. Murry, PhD, Senior Vice President, Process Development, Amgen

5:10 **Talk Title to be Announced**
Tara Jones, PhD, Senior Vice President, Global Drug Product Operations, Moderna

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 **Novel Method for Automated Aseptic Sampling from a Bioreactor**
Craig Love, Principal Scientist, IDEX Health & Science LLC
Automated aseptic sampling solutions typically require bulky hardware components on the tank, have a high cost of expansion, and are not well suited for small sample volumes. In this presentation, we will introduce a novel approach for automated sampling from multiple bioreactors that addresses these limitations. Our proprietary non-porous aseptic membrane sampling technology provides a compact footprint that is readily expandable to multiple bioreactors, consumes less than 100 µL of sample with automated degassing, and is capable of sub-two minute sampling intervals.

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.

TABLE 10: Digitizing Historical Bioprocess Data: Challenges and Solutions
Christina Vessely, PhD, Senior Consultant, CMC Analytics & Formulation Development, Biologics Consulting Group, Inc.

TABLE 11: Turn Workflow Challenges into Data Science Projects
Varsha Daswani, PhD, PMP, Senior Director, Analytics and Data Science, Lumilytics

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 **Ensemble Modeling for the Prediction of Large-Molecule Protein Structures**
Varsha Daswani, PhD, PMP, Senior Director, Analytics and Data Science, Lumilytics
The use of computational methods to predict three-dimensional protein structures from a primary sequence is a dynamic area of research with significant implications for drug discovery. While large molecule proteins are vital in drug discovery and development, accurately predicting their complex structures is challenging. Ensemble modeling, which combines multiple predictive models, shows promise in improving the accuracy and reliability of protein structure predictions, in the absence of experimental data.

12:30 **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?

1:00 LUNCHEON PRESENTATION: Increase Productivity and Reduce Costs with the Maurice icIEF400 Cartridge bio-techne

Peter Johnson, Field Application Scientist & Mgr, Bio Techne

The icIEF technology and instruments have become the leading method for characterizing the charge heterogeneity of biomolecules. The Maurice™ and MauriceFlex™ platforms have led the industry for this application globally. We're thrilled to announce a new Maurice cartridge for charge hereterogeneity analysis that increases the total number of injections from 200 to 400 and the batch limit from 25 to 40.

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

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

Li Jing, PhD, Principal Scientist, USP

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 panomebio

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
Benjamin Laccetti, PhD, Senior Scientist, Process Development, 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 Optimizing Bioprocess Development Decisions with Next-Generation Analytical Techniques SCIEX
Fang Wang, Sr Tech Product Mgr, SCIEX
Detailed and accurate information about a molecule's characteristics is critical for production. This data allows scientists to make informed decisions, pushing the strongest drug candidates forward, while maintaining a competitive pace. Recent advances in analytical techniques have enhanced the speed and reliability of data collection, making it easier to gather critical insights. Here, we explore case studies, demonstrating how SCIEX solutions support the development of biopharmaceuticals from routine analysis to deep characterization of PQAs.

10:00 Coffee Break in the Exhibit Hall with Poster Viewing sbi SCIENTIFIC BIOPROCESSING

CHARACTERIZATION OF mRNA, OLIGO, AND ANTISENSE THERAPEUTICS

10:40 Biophysical Characterization for Antisense Oligos
William Yueheng Zhang, PhD, Senior 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, Genentech
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 Enzo

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 HaLCon Protein Analyzer: A Study on At-Line Titer Analysis in Biopharmaceutical Applications REDSHIFTBio
Yuxiang Henry Zhao, Scientist, Bristol Myers Squibb
With the increasing demand for intensified processes in biopharmaceutical manufacturing, there is a growing requirement for a rapid and precise analytical method to measure product yield. This talk presents a case study on the HaLCon protein analyzer, a compact Liquid Chromatography (LC)

system equipped with a Protein A affinity column. The study investigates the capabilities, performance, and potential applications of HaLCon in GMP facilities.

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

FORMULATRIX

PLENARY FIRESIDE CHAT: LEADING TO TOMORROW'S ADVANCES

3:50 Plenary Introduction
Daniel Barry, Senior Conference Director, Cambridge Healthtech Institute

3:55 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
Sarah Muse, PhD, Senior Scientist, Sanofi

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 Integration of Orthogonal Methods for Enhanced Product Understanding
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

MACHEREY-NAGEL

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.

TABLE 9: Advancing Multi-Specific Molecules: Innovations and Challenges in Analytical Characterization
Hirsh Nanda, PhD, Director, Analytical Sciences, Janssen

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 Characterization Nucleic Acid within AAV Vectors by Long-Read Sequencing
Serena Dollive, PhD, Senior Scientist, Analytical Development, Oxford Biomedica
I will present newly developed methods for characterizing AAV vector via PacBio long-read sequencing. Our tools quantitate residual impurities as well as vector subspecies (ie full vs partial) all within one assay, allowing deeper, more comprehensive understanding of vector batch content.

11:30 Enjoy Lunch on Your Own

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

SGS

NEW MS APPLICATIONS AND TECHNOLOGIES

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

1:10 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.

1:40 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.

2:10 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.

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

FACILITATED TOWN HALL DISCUSSIONS

2:55 Facilitated Town Hall Discussions - IN PERSON ONLY

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.

Town Hall 1: 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

Town Hall 2: 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.

Town Hall 3: Digital Transformation & AI in Bioprocess Development and Manufacturing


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 »

Rapid Methods to Assess Stability and Impurities in Biologics

Hot Topics, Case Studies, and Strategies and Technologies for Detection, Analysis and Control

AUGUST 19-20
All Times EDT

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 AAE-MS: A Powerful Method to Assess HCP ELISA Fit for Purpose

Jared Isaac, Assoc Dir Chromatography, Cygnus Technologies

Antibody Affinity Extraction (AAE) is a powerful orthogonal approach to determine if a Host Cell Protein (HCP) ELISA is fit for purpose. AAE is referenced as Immunoaffinity Chromatography in USP 1132 and has been used in over 300 projects to perform antibody coverage analysis for regulatory submissions. This presentation will focus on several case studies demonstrating utility of AAE-MS method in identification of immunoreactive HCPs to establish HCP Antibody coverage, HCPs enriched during process changes, and HCPs co-purified with DS.

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 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.

12:00 pm 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 Toward the Molecular-Level Understanding of "Problematic" Host Cell Protein Impurities in Bioprocessing

Michael Dolan, Senior Staff Engineer, Biotherapeutics Process Development, 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 SELECTED POSTER PRESENTATION: High-Throughput Activity Assay for Prime Editor mRNA

Amanda Jay, Assoc Engineer II, Plate Based Assays & Lab Automation, Prime Medicine Inc

As gene editing technologies evolve, analytical and QC methods need to be created to determine the functional activity of different gene-editing components. This assay was created to quantify the relative activity of prime-editing mRNAs. By introducing an edit into the luciferase gene of a luciferase-expressing cell line, the activity of target mRNAs were determined by their ability to correct the base-pair insertion and restore function to the luciferase gene.

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

4:25 Chairperson's Remarks
Tara Jones, PhD, Senior Vice President, Global Drug Product Operations, Moderna

4:30 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 One-to-One Interview, with Audience Q&A
Jerry A. Murry, PhD, Senior Vice President, Process Development, Amgen

5:10 Talk Title to be Announced
Tara Jones, PhD, Senior Vice President, Global Drug Product Operations, Moderna

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 Biologics Drug Product Manufacturing
Pinaki Ranadive, PhD, Senior Scientist, Formulation Development Group, Regeneron Pharmaceuticals
Silicone tubing is used in various unit operations during biologics drug product (DP) manufacturing. Hold of protein formulations in semi-permeable silicone tubing over time may impact product quality, particularly protein concentration. We developed a semi-empirical mechanistic diffusion-based model that predicts protein concentration change over hold time for a given formulation type and tubing size. Overall, our study suggests the significance of monitoring water loss from silicone tubing during DP manufacturing.

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 Polysorbate Degradation Challenges and Risk Mitigation Strategies
Pfanstiehl
SUDHAKAR VORUGANTI, Director, Business Development, Pfanstiehl Inc
PS80 is a non-ionic surfactant routinely used in biotherapeutic formulations. PS80 prevents protein aggregates and protects proteins from denaturation from various stresses. However, there are challenges associated with PS80 as it is prone to degradation via oxidative or hydrolytic pathways. There are several root causes for PS80 degradation via oxidative pathway including transition metal contamination such as Fe and Cu. In this presentation we will discuss about mitigating risks with PS80 degradation using Pfanstiehl high purity, low endotoxin, low metal excipients.

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
Can Araman, PhD, Senior Manager, Protein Formulation Laboratory, Merck 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 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, Pharmaceutical Sciences, 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

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

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 Remark

Weiyi Li, PhD, Scientist II, Prime Medicine Inc.

1:10 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.

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 Presentation to be Announced

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.

Town Hall 1: Harnessing ML/AI and Big Data for Biotherapeutic Development

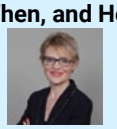
Pin-Kuang Lai, PhD, Assistant Professor, Department of Chemical Engineering and Materials Science, Stevens Institute of Technology

Town Hall 2: 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.

Town Hall 3: 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

4:25 Chairperson's Remarks
Tara Jones, PhD, Senior Vice President, Global Drug Product Operations, Moderna

4:30 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 One-to-One Interview, with Audience Q&A
Jerry A. Murry, PhD, Senior Vice President, Process Development, Amgen

5:10 Talk Title to be Announced
Tara Jones, PhD, Senior Vice President, Global Drug Product Operations, Moderna

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 Presentation to be Announced

IDEX HEALTH & SCIENCE

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.

BREAKOUT DISCUSSION: Turn Workflow Challenges into Data Science Projects
Varsha Daswani, PhD, PMP, Senior Director, Analytics and Data Science, Lumilytics

BREAKOUT DISCUSSION: Digitizing Historical Bioprocess Data: Challenges and Solutions
Christina Vessely, PhD, Senior Consultant, CMC Analytics & Formulation Development, Biologics Consulting Group, Inc.

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: Increase Productivity and Reduce Costs with the Maurice icIEF400 Cartridge

bio-techne

Peter Johnson
The icIEF technology and instruments have become the leading method for characterizing the charge heterogeneity of biomolecules. The Maurice™ and MauriceFlex™ platforms have led the industry for this application globally. We're thrilled to announce a new Maurice cartridge for charge hereterogeneity analysis that increases the total number of injections from 200 to 400 and the batch limit from 25 to 40. This talk will present data generated using both routine standards and customer test molecules that show like-for-like performance between the new icIEF 400 cartridge and the regular Maurice cIEF cartridge. Additionally, this talk will demonstrate comparable data between the two cartridges on both Maurice and MauriceFlex instruments from multiple test sites and different analysts. Customer beta-site test data will also be shared to demonstrate customer satisfaction. Such data comparability enables a seamless transition from the regular Maurice cIEF cartridge to the new icIEF400 cartridge, with a favorable cost per injection. With the discontinuation of the iCE3 instruments in 2029, this new cartridge eases the transition to the Maurice platforms by providing higher batch and sample numbers for high throughput applications.
Peter Johnson, PhD, Shehab Barakat, PhD, Teva Collaborator, Tim Gieger, PhD 1ProteinSimple, A Bio-Techne Brand, San Jose, CA

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

Li Jing, PhD, Principal Scientist, USP

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, Vaccine CMC Development & Supply, Sanofi

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 Enhancing Bioprocess Monitoring and Understanding by Leveraging Benchtop NMR
Speaker to be Announced, Bruker BioSpin AG
Despite the recognized efficiency of continuous biopharmaceutical manufacturing, it has yet to be widely adopted because of the inherent complexity of biologics and bioprocesses. Optical spectroscopies are typically employed for process monitoring but lack the level of structure characterization achieved by NMR. This presentation demonstrates the power of benchtop NMR as a well-suited technique to complement existing technologies for enhanced bioprocess monitoring and understanding.
This work was done in the context of the acquisition of an NMR instrument by Sanofi, and in agreement with the company.
Authors: Victor Beaumont*, Christian Fischer*, Matteo Pennestri* *Bruker International

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

STRATEGIES FOR AI/ML APPLICATIONS IN BIOPROCESSING

10:40 Integrating Machine Learning (ML) for Bioprocess Systems Modelling and Use Cases in Vaccines CMC Development
Angela Li, PhD, Senior Scientist, Vaccine CMC Development & Supply, Sanofi
Applications where we see the value of predictive and quantitative models in a CMC development space will be discussed. Case studies will be presented on how process models (hybrid, mechanistic and ML) of different unit operations, productivity, and quality attributes may be used to accelerate development. Moreover, insights on the development and potential applications of a novel physics-informed machine learning chromatography model framework will also be shared.

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 Industry Maturity Models as the North Star for Digital Transformation
Eugene Tung, PhD, Executive Director, Manufacturing IT, Merck & Co., Inc.
As companies in the pharmaceutical manufacturing industry undergo digital transformations, they face numerous questions around the transformation. What are the goals? What are the benefits? What does good look like? In this presentation, we examine digital transformation through the lens of digital plant maturity models, which help companies understand the current state of their plants and prioritize their digital investments as they progress up the maturity curve.

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
Moo Sun Hong, PhD, Assistant Professor, Department of Chemical and Biological Engineering, Seoul National University

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.

Shahzad Khan, Yokogawa Electric Corporation

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.

2:45 Advancing Bioprocessing with AI-Native MLOps: Case Studies and Prospects INVERT

Karthik Sekar, PhD, Staff Data Scientist, Invert

Invert is a cutting-edge AI-native software platform, purpose-built as an MLOps solution for bioprocessing. This talk will:
Introduce Invert's innovative approach to managing data for bioprocess optimization
Highlight outcomes of collaborations in identifying critical process parameters
Demonstrate our AI-driven experimental design methodologies
Preview upcoming features, including advanced temporal process prediction
Join us to explore how Invert is transforming bioprocessing efficiency and accelerating innovation in the field.

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 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.

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, Technology Development and Implementation, Takeda

- What do you consider when deciding on entry points for modeling in process development?
- How do you enable engagement and use of models in development workflows?
- What does '*in silico* first' mean to different stakeholders?
- What are the biggest hurdles when incorporating models directly into process control?

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?

IN-PERSON ONLY BREAKOUT: In-house vs Off-the-Shelf Solutions for a Digital R&D Ecosystem
Victor Sanchez Tarre, PhD, Dir Data Science, Mfg Science & Technology, MeiraGTX
- Factors to be considered when deciding on building an in-house software solution vs. off-the-shelf offerings
- Tools to build a successful digital ecosystem—from specific code packages to ELN and data exploration platforms of choice
- Infrastructure supporting Data Engineering and Data Science functions
- Challenges—assay nomenclature harmonization, standardization of data capture and data processing workflows, and strategies to routinely generate FAIR data

10:30 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.

11:00 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.

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.

Town Hall 1: 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

Town Hall 2: 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.

Town Hall 3: 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!

Additional Sponsorship & Branding Opportunities Include:

- Exhibit Hall Networking Reception
- Conference Tote Bags
- Product Launch Promo
- Footprint Trails
- Literature Distribution (Tote Bag Insert or Chair Drop)
- Badge Lanyards
- Conference Materials Advertisement
- Padfolios and More…

For more information regarding exhibits and sponsorship, please contact:

Companies A-K

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Companies L-Z

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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
- 16% Executive
- 24% Sales & Marketing
- 14% Director
- 8% Manager
- 2% Assistant
- 1% Professor

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Hotel and Travel

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Discounted Room Rate: $275 s/d
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To reserve your hotel accommodations and for additional information please visit
Bioprocessingsummit.com/hotel-travel

Top Reasons to Stay at the Sheraton Boston

- No commute, since meeting takes place at hotel!
- Steps away from the Prudential Center & Copley Place with shopping and restaurants
- Local attractions including Newbury Street, Eataly, Boston's South End, and Boston Public Gardens within walking distance

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Pricing & Registration

16TH ANNUAL
BIOPROCESSING SUMMIT
August 19-22, 2024 | Boston, MA & Virtual

STANDARD PACKAGE

Includes access to all conferences and/or training seminars (4 days) & networking events. Plus, on-demand access. You are allowed to move between conference tracks to attend presentations taking place at the same time, excluding the Talent in Biopharma workshop and Bioprocessing: Venture, Innovation & Partnering conference.

	COMMERCIAL	ACADEMIC-GOVERNMENT-HOSPITAL, INVESTMENT/BANKING
Advance Registration Rates until July 12	$3,099	$1,499
Standard Pricing after July 12	$3,299	$1,599

BASIC PACKAGE

Includes access to ONE conference or training seminar (2 days) & networking events. Plus, on-demand access. You are allowed to move between conference tracks to attend presentations taking place at the same time, excluding the Talent in Biopharma workshop and Bioprocessing: Venture, Innovation & Partnering conference.

Advance Registration Rates until July 12	$2,099	$1,099
Standard Pricing after July 12	$2,299	$1,199

WORKSHOP ONLY PRICING

Includes access to the Talent in Biopharma workshop and all the Bioprocessing Summit exhibit hall networking events.

Talent in Biopharma Leadership Workshop	$695	$495

GROUP DISCOUNTS

Have your colleagues or entire team attend! Purchase a full-price registration, and participants from the same organization will receive a 20% discount when registering through the Group Registration page. For more information on group discounts, contact Uma Patel at 781-972-5447.

LEARN MORE »

ALUMNI DISCOUNT

Cambridge Healthtech Institute appreciates your participation at our events. As a result of the great loyalty you have shown us, we are pleased to extend to you the exclusive opportunity to save an additional 20% off the registration rate.

LEARN MORE »

POSTER DISCOUNT

($50 Off) Poster materials are due by June 28, 2024. Once your registration has been fully processed, we will send an email containing a unique link and instructions for submitting your abstract and other materials. If you do not receive your link within 5 business days, please contact jring@healthtech.com. CHI reserves the right to publish your poster content in various marketing materials and products.

LEARN MORE »

FOR ADDITIONAL REGISTRATION OPTIONS, VISIT OUR EVENT WEBSITE:
BioprocessingSummit.com

Please use keycode **BPD EF** when registering!