THE 15TH ANNUAL BIOPROCESSING SUMMIT

August 14-17, 2023 | Boston, MA
Sheraton Boston + Virtual

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

Join 1500+ Global Participants
Register Today

2023 PROGRAMS

- STREAM #1 **UPSTREAM PROCESSING**
- STREAM #2 **DOWNSTREAM PROCESSING**
- STREAM #3 **GENE THERAPY**
- STREAM #4 **CELL THERAPY**
- STREAM #5 **ANALYTICAL & QUALITY**
- STREAM #6 **STABILITY & FORMULATION**
- STREAM #7 **VACCINE & mRNA THERAPIES**
- STREAM #8 **BIOMANUFACTURING & DIGITALIZATION**

PLENARY KEYNOTE SPEAKERS

Glen R Bolton, PhD
Executive Director, Late Stage Bioprocess Development, Amgen Inc.

Rachel Salzman, DVM
Founder, The Stop ALD Foundation & Executive Vice President, Portfolio, External Affairs & Development, Alcyone Therapeutics

Konstantin B. Konstantinov, PhD
CTO, Codiak Biosciences

Richard D. Braatz, PhD
Professor, Chemical Engineering, Massachusetts Institute of Technology

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BioprocessingSummit.com

Celebrating its 15th year,

The Bioprocessing Summit is a premier global forum for industry leaders to share the latest developments in bioprocess R&D, scale-up, quality, and analytics. This year's programming comprises more in-depth content than ever before, showcasing 8 topic-focused streams, 14 conference tracks, plus training seminars, research posters, and so much more.

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PLENARY KEYNOTE SESSIONS

MONDAY, AUGUST 14, 2023 | 4:20-5:30 PM

SOLVING TODAY'S CHALLENGES

Current Challenges in Bioprocessing

Glen R Bolton, PhD, Executive Director, Late Stage Bioprocess Development, Amgen Inc.

Commercializing Gene Therapies – The Combined Power of Patient Advocacy and Cost-Effective Manufacturing

Rachel Salzman, DVM, Founder, The Stop ALD Foundation & Executive Vice President, Portfolio, External Affairs & Development, Alcyone Therapeutics

WEDNESDAY, AUGUST 16, 2023 | 3:50-5:00 PM

LEADING TO TOMORROW'S ADVANCES

Current and Future Trends in Biomanufacturing of New Modalities

Konstantin B. Konstantinov, PhD, CTO, Codiak Biosciences

The Digitalization of Biomanufacturing

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

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

BIOPROCESSING TECH

Venture, Innovation, and Partnering Conference

August 16, 2023
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
CEO, Landmark Bio

Learn More at Bioprocessingsummit.com/Investor »

WORKSHOP

AUGUST 14

**In-person only. *Separate registration required.*

Talent Acquisition and Retention Strategies for Biopharma

The exponential growth and complexity of the biopharmaceutical industry combined with the growing shortfall of experience and talent is now recognized as a leading threat and roadblock to operational sustainability and profitability, often ahead of technology.

Increased competition to recruit and retain talent requires that biopharmaceutical companies adjust current talent acquisition strategies to support continued business and operational functionality.

CHI, in partnership with Evaluating Biopharma, invites you to attend and participate in the inaugural ***Talent Acquisition and Retention Strategies for Biopharma*** fireside discussions and networking event taking place, Monday August 14, 2023 in Boston (part of the 15th Annual Bioprocessing Summit).

AGENDA:

2:00–2:10 pm	Welcome Keynote
2:10–2:30 pm	Fireside Chat #1: Attracting and Acquiring Talent – Leveraging Your Market Brand and Business USPs
2:30–2:50 pm	Fireside Chat #2: Assessing and Validating Talent – Ensuring Candidates Are Really the Right Fit
2:50–3:00 pm	Coffee/Refreshment Networking Break
3:00–3:20 pm	Fireside Chat #3: Onboarding and Retaining Talent– Best Practices to Fast-Track and Keep Talent
3:20–4:30 pm	Coffee/Refreshment Networking Break
4:30–5:30 pm	Access to Bioprocessing Summit Plenary Sessions
5:30–6:30 pm	Invitation to Bioprocessing Summit Welcome Reception

For more details on the workshop, please contact:

Brian Caine
Phone: (+1) 908-809-0946
Email: bcaine@healthtech.com

Learn More »

CONFERENCE-AT-A-GLANCE

2023 Programs

	AUGUST 14-15	AUGUST 16-17
Stream #1 UPSTREAM PROCESSING	Cell Line Development & Cell Culture Optimization	Smart Biomanufacturing & Digitalization
Stream #2 DOWNSTREAM PROCESSING	Intensified & Continuous Processing	Advances in Purification & 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 ANALYTICAL & QUALITY	Host Cell Proteins	Accelerating Analytical Development
Stream #6 STABILITY & FORMULATION	Rapid Methods to Assess Stability and Impurities in Biologics	Formulation and Delivery of High-Concentration Proteins and New Modalities
Stream #7 VACCINE & mRNA THERAPIES	Vaccine Development and Manufacturing	mRNA-Based Therapies
Stream #8 BIOMANUFACTURING & DIGITALIZATION	Intensified & Continuous Processing	Smart Biomanufacturing & Digitalization
Training SEMINARS By Cambridge Healthtech Institute	Introduction to Bioprocessing Integrating AI and Data Science in the Product Life Cycle Introduction to Viral Vectors and Gene Therapy	Introduction to CMC for Biotech, Cell & Gene Therapy Products Potency Assays and Comparability for Cell and Gene Therapies
WORKSHOP	Talent Acquisition and Retention Strategies for Biopharma	
BIOPROCESSING TECH VENTURE, INNOVATION, AND PARTNERING CONFERENCE		Bioprocessing Tech Venture, Innovation, and 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 14 10:00 am–3:30 pm
Tuesday, August 15 8:00 am–1:00 pm

TS1: Introduction to Bioprocessing

Instructors:

Sheila Magil, PhD, Independent Consultant

Frank Riske, PhD, Managing Director, BioProcess Technology Group, BDO USA, LLP

CHI's Introduction to Bioprocessing training seminar offers a comprehensive survey of the steps needed to produce today's complex biopharmaceuticals, from early development through commercial. The seminar begins with a brief introduction to biologic drugs and the aspects of protein science that drive the intricate progression of analytical and process steps that follow. We then step through the stages of bioprocessing, beginning with the development of cell lines and ending at scaling-up for commercial production. The seminar also explores emerging process technologies, facility design considerations, and the regulatory and quality standards that govern our industry throughout development. The important roles of analytical methods at all stages of development as well as formulation and stability assessments in developing and gaining approval for a biopharmaceutical are also examined. This 1.5-day class is directed to attendees working in any aspect of the industry, including scientific, technical, business, marketing, or support functions, who would benefit from a detailed overview of this field.

TS2: Integrating AI and Data Science in the Product Life Cycle

Instructors:

Christoph Herwig, PhD, Founder, Lisalis GmbH, former Professor, Bioprocess Engineering, TU Wien

Sherwin Jayashinghe, Technical Sales Engineer, Koerber Pharma Software

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

TS3: Introduction to Viral Vectors and Gene Therapy

Instructors:

Scott Cross, Senior Principal, Dark Horse Consulting Group

Jacob Staudhammer, Principal, Dark Horse Consulting Group

Catherine Colandro, PhD, Senior Consultant, Dark Horse Consulting

Targeting disease at its origin, gene therapies offer the promise of a one-time treatment and have transformed how we treat some diseases. These medicines are complex biologics requiring advanced manufacturing methods and highly skilled operators. This training session provide an expansive introduction to gene therapy, the manufacture of these complex biologics, the facilities, equipment and personnel needed to produce them; and the analytical and quality aspects surrounding them.

Wednesday, August 16 8:00 am–3:00 pm
Thursday, August 17 8:00 am–12:00 pm

TS4: Potency Assays and Comparability for Cell and Gene Therapies

Instructor:

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

The evaluation of potency plays a key role in defining the quality of cellular and gene therapy products. CHI's Training Seminar, Potency Assays and Comparability for Cell and Gene Therapy, provides an insight into the expectations and challenges in development of potency assays specific for cell and gene therapies; several real-life experiences from the industry are presented as illustrations, including the impact of comparability assessment following process change.

TS5: 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 requirement. 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

In biopharmaceutical production, the need to achieve higher productivity while maintaining quality consistency and reducing cost is the holy grail. The industry has made great progress in upstream processing in the past decade, led by improved host cell engineering, higher expression cell lines, optimized cell culture, and perfusion/intensified processing. The next decade will see smarter biomanufacturing strategies coming to the forefront, incorporating new platforms and technologies such as NGS, targeted integration, synthetic biology, data management, automation, modeling, digital twins, PAT, and more. The Upstream Processing conferences will dive into the many exciting trends and innovations driving the next wave of upstream development.

Conference Programs

AUGUST 14-15

Cell Line Development & Cell Culture Optimization

View Program »

AUGUST 16-17

Smart Biomanufacturing & Digitalization

View Program »

MONDAY, AUGUST 14

8:00 am Registration and Morning Coffee

NEW PLATFORMS AND APPROACHES IN CELL LINE DEVELOPMENT

9:55 Chairperson's Remarks
Jianfa Ou, PhD, Senior Scientist, Bristol Myers Squibb Co.

10:00 KEYNOTE PRESENTATION: Automation in Cell Line Development
Lina Chakrabarti, PhD, Associate Principal Scientist, AstraZeneca
Automating cell line development (CLD) and cell culture workflow enables (1) early enrichment of high producer and high-quality clones, (2) increased process consistency and screening throughput, (3) enhanced resource efficiency, (4) generation of reliable and reproducible results, and (5) improved data traceability and integrity. Hence, establishment of an automated platform in CLD offers multi-faceted advantages for developing desirable cell lines to be used for biomanufacturing.

10:30 Pfizer's Targeted Integration Platform Enables Development of Robust and Stable Cell Lines on Accelerated Timelines
Laura Zielewicz, PhD, Senior Scientist, Pfizer Inc.
Speed to regtox and clinical trials has become a common paradigm in the pharmaceutical industry. Pfizer's targeted integration CHO host provides the platform for developing robust cell lines on accelerated timelines. The platform utilizes an engineered dual-landing pad and multiple-copy vectors to develop cell lines with predictable and stable genotype, growth characteristics, and production of standard mAbs, multi-specifics, and fusion proteins, which is critical to enable acceleration.

11:00 Sequence Variants Determination via NGS for Cell Line Screening
Grace Yi-Wei Huang, PhD, Scientist, IGM Biosciences, Inc.
Proteomic methods and Sanger sequencing have been used in cell line development for over a decade, but the advent of next-generation sequencing (NGS) has revolutionized the screening and characterization of cell lines for biologics production. IGM Biosciences uses NGS to identify sequence variations and evaluate cell line genotypes relating to their growth and productivity. We will explore the benefits of utilizing these sequencing technologies when selecting lead clones.

11:30 Integrating Synthetic Biology and Computer-Aided Design to Advance Biologics Production in CHO Cells ASIMOV
Scott Estes, PhD, Head of Cell Line Development, Asimov
In this talk, we present a CHO platform that moves beyond the one-size-fits-all paradigm by tailoring vectors for each molecule to optimize expression. Our system combines a GS knockout CHO host, transposase, genetic libraries, and computational tools. These tools enable development of high-expression lines, fine-tuning of chain expression, and ML-based optimization. We present case studies highlighting the impact of these tools to optimize expression for both standard monoclonal and bispecific antibodies.

12:00 pm LUNCHEON PRESENTATION: Picodroplets for Cell Line Engineering: a Novel Automation Approach sphere FLUIDICS
Frank F. Craig, PhD, MBA., Chief Executive Officer, Sphere Fluidics Ltd.
The development process for cell lines is complex and laborious, with increasing expectations for supporting in-process data. We will show how microfluidic-enabled picodroplets deliver integrated, user-friendly, automated workflows where millions of individual cells are assessed daily, and the best single cells selected - in an environment that maintains high cell viability and outgrowth. We will introduce Cyto-Mine®, a platform that enables a step-change in speed and scale of working.

12:30 Session Break

MEDIA OPTIMIZATION

12:50 Chairperson's Remarks
Laura Zielewicz, PhD, Senior Scientist, Pfizer Inc.

12:55 Media Optimization for Robust CHO Cell Culture Process
Jianfa Ou, PhD, Senior Scientist, Bristol Myers Squibb Co.
Process intensification, including implementation of highly concentrated media, is widely pursued to improve process yield and reduce footprint for monoclonal antibody production. Media preparation parameters contribute significantly to media quality, cell culture performance, and productivity. Thus, it is important to understand and develop proper media preparation procedure for robust CHO cell culture process. Here we evaluated the key parameters on media stability by cell culture and different process analytical technologies.

1:25 Leveraging AstraZeneca's Proprietary Media and Feeds to Improve Yield for a Commercial CHO-Derived Therapeutic Protein
Jessica Kenney, Senior Process Engineer, Biologics Drug Substance Tech Transfer, Alexion, AstraZeneca Rare Disease
This talk will give an overview of the development of a next-generation upstream manufacturing process for a CHO-derived therapeutic protein to significantly improve yield, reduce cost of goods, and ultimately serve more patients with rare diseases. Newly accessible AstraZeneca proprietary media and feeds were leveraged to achieve this goal, with modifications to the platform feeding strategy leading to an increase in titer of ~33%.

1:55 A Turnkey Automation Solution for Highly Efficient CLD for Biotherapeutics CYTENA A BICO COMPANY
John Carroll, Eastern Regional Manager Biopharma, CYTENA
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 can be tailored and configured with the best-in-class instruments and software for monoclonal antibody development, viral vector production, and cell therapy. Join us to explore its transformative impact and discover the future of CLD.

2:10 Overcoming the Challenges of Product Concentration Monitoring at a CDMO REDSHIFTBio
Thaddeus Webster, R&D Supervisor, Lonza
Conventional methods of titer analysis require trained analysts and hours of runtime, delaying results for trending and forward processing decisions. The HaLCon [RedShiftBio] system provides near real-time assessment of product concentration for Fc-containing proteins. Daily monitoring of a bispecific antibody in a fed-batch process and product sieving throughout a perfusion process were demonstrated using HaLCon. This approach allows for accelerated timelines in a manner that should be scalable to manufacturing.

2:25 Networking Refreshment Break

LEVERAGING DOE AND QBD PRINCIPLES TO OPTIMIZE CELL CULTURE PROCESSES

2:40 Achieving High Titer in a Non-Platform CHO Process when Converting to an Internal Medium Platform

Thomas Hayes, Senior Scientist, Cell Culture Development, Sanofi

A non-platform CHO process using proprietary commercial medium was internalized, and initial results showed a 3-fold decrease in productivity. We identified key medium components and process parameters that significantly increased cell-specific productivity, and after optimization a 5-fold increase in titer was achieved in an internal medium platform. The final optimized process was scaled from AMBR250 and benchtop bioreactors to pilot-scale single-use bioreactors to demonstrate process and scale-up robustness.

3:10 Leveraging DoE Modeling to Optimize Cell Culture Performance Attributes of a Null Cell Pool for Developing a Process-Specific HCP Reagent

Wilhad H. Reuter, Lead Engineer, Upstream Process Development, Alkermes, Inc.

This case-study will address the methodologies for cell culture optimization to generate a process-specific HCP reagent, recommended for late-stage product characterization. A combination of screening DoE and OFAT experimentation was implemented to identify optimal process conditions in the ambr15 miniature bioreactor system. These conditions were then implemented in a 2 L single-use bioreactor system to show comparable growth attributes and HCP production to that of the original producer cell line.

3:40 Session Break and Transition to Plenary Keynote Session

PLENARY KEYNOTE: SOLVING TODAY'S CHALLENGES

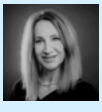
4:20 Chairperson's Remarks

Susan D'Costa, PhD, CTO, Genezen

4:30 Overcoming the Challenges of Bioprocesses: The Future of Biomanufacturing

Glen R. Bolton, PhD, Executive Director, Late Stage Bioprocess Development, Amgen, Inc.

Novel therapies and technologies are emerging to meet the needs of patients; however, the manufacturing of biopharmaceuticals remains a complex and challenging process. As demand for biopharmaceuticals grows, the industry faces new challenges in terms of scalability, cost, and process robustness. The implementation of innovative technologies to improve process efficiency and the importance of process control and data analytics in ensuring process robustness are key levers to meet these challenges.



5:00 Commercializing Gene Therapies—The Combined Power of Patient Advocacy and Cost-Effective Manufacturing

Rachel Salzman, DVM, Founder, The Stop ALD Foundation; Global Head, Corporate Strategy, Armatus Bio

This presentation will examine the development of an FDA-approved gene therapy where patient advocacy played a critical role resulting in the first ever clinical use of a lentiviral vector. Although manufacturing continues to represent a significant challenge throughout the entire R&D journey, there are opportunities for advocacy and manufacturing communities to seek alignment and combine their collective powers to achieve the common goal of increasing patient access to transformative medicines.

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

Nitto Avecia

6:30 Close of Day

TUESDAY, AUGUST 15

7:30 am Registration and Morning Coffee

GENOME ENGINEERING

7:55 Chairperson's Remarks

Ping Liu, PhD, Associate Director & Head of Cell Line Development, REGENXBIO, Inc.

8:00 Genome Engineering and Cell Line Development to Facilitate Drug Development

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

Developing cellular models of disease have the potential to facilitate and/or accelerate the drug discovery and development. At REGENXBIO, we are evaluating the use of genome engineering and cell line development approaches, to generate mammalian disease cell line models that have utility in the screening of drug candidates. These models should facilitate the robust advancement of gene therapy products from the lab bench to the clinic.

8:30 MAD7 Nuclease Activity in CHO Cells

Geneva Alok, PhD, Process Development Scientist, Amgen

Manufacturing cell lines can benefit from host cell engineering to improve performance and product quality. MAD7 is an engineered class 2 type V-A CRISPR-Cas nuclease (Cas12a/Cpf1) shown to function in a variety of systems, recently including CHO cells. MAD7 can successfully facilitate site-specific indels and knock-ins in CHO, making it a useful tool for engineering in cell line development.

9:00 Population Dynamics, Phenotypic Heterogeneity, and Age: Shifting Expression Patterns in Stable and Unstable Clonally-Derived CHO Populations

Theodore Peters, PhD, Senior Scientist, Cell Line Development, Seagen

CHO cell lines have significant phenotypic variability though derived from a single cell progenitor. This variability may lead to or be indicative of the propensity of a cell line to exhibit production instability over time. Here we characterize RNA expression from stable and unstable cell lines using single-cell RNA sequencing. Our work shows that clonally derived cell lines are a complex metapopulation of cells whose make-up changes significantly with age.

9:30 Strategies for Cell Line and Process Development that Support Current and Next-Generation Products and Processes

FUJIFILM Diosynth biotechnologies

Leon Pybus, Associate Director, Process Strategy & Development, FUJIFILM Diosynth Biotechnologies

mAbs produced in fed-batch mode by CHO cell lines have a low risk profile and platform approaches to development, manufacturing technology, and infrastructure allow for rapid IND submission. Next-generation products (e.g., bispecifics) and processes (e.g., perfusion) can stretch this mAb platform paradigm and necessitate increased development. This requires a revaluation of mAb platform components to enable rapid development of current and next generation products and processes.

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

ILC DOVER

10:45 Breakout Discussion Groups

Breakout discussions provide an opportunity to discuss a focused topic with peers from around the world in an open, collegial setting. Select from the list of topics available and join the moderated discussion to share ideas, gain insights, establish collaborations or commiserate about persistent challenges. Please visit the breakout discussions page on the conference website for a complete listing of topics and descriptions.

IN-PERSON ONLY BREAKOUT: The Indispensability of Gene Editing in Drug Discovery and Development

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

- How relevant are isogenic cell lines in drug discovery and development today?
- What are the factors to consider in choosing a cell line for isogenic cell line development?
- What are some strategies to evaluate long term stability of clones?
- What new technologies can we employ to predict clone stability?

IN-PERSON ONLY BREAKOUT: New Platforms and Approaches in Cell Line Development

Lina Chakrabarti, PhD, Associate Principal Scientist, AstraZeneca

- Tackling novel molecular formats
- Aligning clone screening with next generation manufacturing platform
- Advancement in single cell cloning

11:30 Synthetic Genetic Parts and Engineering Systems for Biologics Production

Renata Caikauskaite, PhD, Senior DNA Engineer, SynGenSys Ltd.

Synthetic biology offers a new paradigm for genetic vector design where it is possible to engineer a host cell factory in a product or cell type specific manner via combinatorial tuning of discrete cellular synthetic processes. Utilizing a platform of genome-scale mining and informatic tools, we generate libraries of engineered/synthetic parts with user-defined functionality that can boost biologic manufacturability and specificity via context-specific control of primary cellular processes.

12:00 pm Impact of Sf-rhabdoviral Contaminants of Insect Cell Lines on Biosafety Profile of the Baculovirus-Insect Cell System

Donald L. Jarvis, PhD, Professor, Molecular Biology, University of Wyoming

The insect cell lines widely used as hosts in the baculovirus-insect cell system are contaminated with adventitous viruses. We assessed the infectivity of Sf-rhabdoviruses for mammalian cell lines and immunocompromised mice to determine their impact on the biosafety profile of this biologics manufacturing platform.

12:30 Valita Titer: Rapid & High-Throughput Quantification of Multiple IgG Formats for Cell Line & Process Development

BECKMAN COULTER Life Sciences

Mathura Raman, Senior Scientist, Bristol Myers Squibb

As the increasing use of high-throughput and automation solutions in cell line development (CLD) & process development (PD) has increased both the speed & sample output of these workflows, ensuring suitable bio-analytics are in place is critical in preventing process bottlenecks. Here we describe the use of ValitaTiter, a simple, high-throughput & automation friendly plate-based antibody quantification assay, and its benefits in supporting high-throughput CLD & PD antibody workflows.

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

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

panomebio

UPSTREAM PRODUCTION CHALLENGES FOR NOVEL MODALITIES

2:10 Chairperson's Remarks

Saurabh Sen, PhD, Associate Director, Cell Line Development, Genomic Medicine Unit CMC, Sanofi

2:15 Addressing the Challenges for Production and Analysis of AAV Vectors

Helen Young, PhD, Manager, Synthetic & Mammalian Upstream, CPI

Developing high-yielding, scalable, and commercially viable processes for AAV manufacture, with associated analytical methods for assessing the CQAs, presents challenges. Here we present work being performed at CPI to address some of the upstream challenges, including development of an intensified N-1 seed train step to reduce inoculum requirements. Further, we discuss some of the approaches we have been working on for full and empty capsid determination, including mass photometry.

2:45 Advancing AAV Transient Expression Platform by Cell Line Engineering, Host Cell Engineering, and Plasmid Optimization

Ping Liu, PhD, Associate Director & Head of Cell Line Development, REGENXBIO, Inc.

To improve AAV transient titers in HEK293 system, REGENXBIO had made significant efforts in host cell line development and engineering. In addition, we generated new plasmid expression systems by several rounds of plasmid optimization. Combining the new cell lines and plasmids, we increased our overall transient yield significantly while improving the product quality.

3:15 Sf9 Cell Line Engineering for AAV Production

Shengjiang Shawn Liu, PhD, President & CEO, Avirmax

Approvals of Glybera, Hemgenix, and Roctavian demonstrated a high success rate of Sf9 cell-derived rAAV vectors. Productivity variation with over-passaging and Sf rhabdovirus (Sf-RhV) contamination may be main issues affecting use of Sf-RhV contaminated Sf9 cells. Sf-RhV containing products may potentially cause inflammation or immunogenicity if administered. To this end, we developed several Sf9 clones free of Sf-RhV, consistently producing rAAV of all tested serotypes with high yield and activity.

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

SYNTHGENE

4:30 It All Starts with One Vial: Cell Banking to Enable GMP Manufacturing

Charu Garg, PhD, Senior Scientist, Process Cell Sciences, Merck

Using a CDMO for GMP cell banking is mainstream in the biopharmaceutical industry. There are potential challenges to successfully manage the tech transfer process. Key factors to generate a successful GMP cell bank include 1) sourcing appropriate raw materials, 2) identifying the correct equipment and its setting, and 3) defining growth parameters, freezing condition. Application of a systematic approach can help ensure the successful generation of a GMP cell bank.

5:00 Fed-Batch Fermentation AMBR250 Model Establishment and Its Utilization in Vaccine Development

Zhiguo Liu, PhD, Associate Principal Scientist, Merck

Advantageous in its high throughput and process automation degree, the AMBR250 platform is gaining industrial adaptation. Here we report a successful 'fit-for-purpose' AMBR250 scale-down model case-study for a vaccine bioprocess. This gas-transfer-guided model development not only demonstrates representative process attributes, but also produced statistically equivalent productivity of target product, enabling a faster process evaluation platform to match an accelerated project timeline.

5:30 Close of Cell Line Development & Cell Culture Optimization Conference

WEDNESDAY, AUGUST 16

7:30 am Registration and Morning Coffee

KEYNOTE SESSION: DIGITAL INNOVATIONS DRIVING BIOTHERAPEUTICS DEVELOPMENT

7:55 Chairperson's Remarks
Jun Huang, PhD, Senior Director, Preclinical Manufacturing and Process Development, Regeneron Pharmaceuticals Inc.
Cenk Undey, PhD, Vice President & Global Head, PTD Data & Digital, Roche/Genentech

8:00 Digital Innovation in Transforming Molecules of Today into the Medicines of Tomorrow
Cenk Undey, PhD, Vice President & Global Head, PTD Data & Digital, Roche/Genentech
We generate significant amount of data during development and manufacturing of biopharmaceutical therapeutic proteins. Managing data and applying predictive/prescriptive analytics including artificial intelligence and *in silico* modeling tools right from the start throughout the product development lifecycle into manufacturing is critical for seamless data flow, robust design, and accelerating the development activities. Digital innovation paired with digital mindset is a significant enabler bringing life-changing medicines to patients.

8:30 The Golden Thread: Digital Powered Seamless Therapeutic Translation and Industrialization
Shanti Chari, Assistant Vice President, Digital Technologies and Innovation, Landmark Bio
From early discovery to preclinical, through clinical development, regulatory review and approval, manufacturing, and quality control, the golden (digital) thread will connect all disparate data sources to provide the basis for a digital, paperless shop floor at Landmark Bio. The automation of internal and external tech transfer will be foundational for seamless therapeutic translation and industrialization, and expose more information across business systems, including those used by external partners.

9:00 Preparing for the Smart Factories of the Future: CDER's Journey
Thomas F. O'Connor, PhD, Deputy Director, Office of Pharmaceutical Quality, CDER, FDA
The advanced technologies and manufacturing approaches needed to enable smart factories may have the potential to deliver higher output, increased manufacturing safety, improved quality, better value, increased agility, additional flexibility, and reduced waste. The presentation will share CDER's experience engaging with manufacturers on digital technologies through the Emerging Technology Team. CDER's initiatives on evaluating the existing regulatory framework in the context of digital technologies and AI will also be discussed.

9:30 Demonstration of an End-to-End Integrated and Continuous Bench-scale mAb Production Process
James Angelo, R&D Manager, BioContinuum Process Technologies, MilliporeSigma
In this work, a fully continuous process was executed using a 15L perfusion mAb production integrated with continuous downstream unit operations. A novel digital monitoring strategy was deployed to ensure appropriate oversight of the process and any key challenges resultant from the continuous process were investigated. Over five days of operation, including three contiguous days at steady-state, more than 75 gAb of bulk drug substance were produced at concentrations greater than 170 g/L. Targets for product quality (less than 1% HMW) and removal of process related impurities (below LOQ of 3 ng/mL for residual HCP) were achieved. Overall, the demonstration was highly informative as to where efforts should be directed in the areas of process development and control/monitoring strategies.

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

AI/ML & DIGITALIZATION – IMPACT AND OUTLOOK FOR BIOLOGICS MANUFACTURING

10:40 Digitalization – Another Technology?
Oliver Hesse, Head, Biotech Data Science & Digitalization, Bayer U.S. LLC
Digitalization is one of the buzzwords that is on every organization's goals. The talk will discuss what digitalization is and what it is not.

11:10 Hybrid Model and Machine Learning Enable Efficient Knowledge Generation for Bioprocess Development of mAbs and New Modalities
Michael Sokolov, PhD, Lecturer, ETH Zurich
In this presentation, we show how advanced machine learning and hybrid modeling approaches can be exploited to significantly improve process understanding, performance, and automated operation as digital twins. All presentations will be centered on industrial implementation examples for mAb, cell & gene therapy, and mRNA processes with numerous big pharma and CDMO partners allowing to quantify efficiency gains in process development.

11:40 PANEL DISCUSSION: The Short-Term and Long-Term Outlook of AI-Centered Technologies and Digital Integration in Biomanufacturing
Moderator: Michael Sokolov, PhD, Lecturer, ETH Zurich
- Which technologies are used today and how?
- What value is expected from these technologies?
- What are the main challenges to consistently drive value from data?
- How can business cases be defined leading to success stories?

Panelists:
Oliver Hesse, Head, Biotech Data Science & Digitalization, Bayer U.S. LLC
Jun Huang, PhD, Senior Director, Preclinical Manufacturing and Process Development, Regeneron Pharmaceuticals Inc.
Thomas F. O'Connor, PhD, Deputy Director, Office of Pharmaceutical Quality, CDER, FDA
Cenk Undey, PhD, Vice President & Global Head, PTD Data & Digital, Roche/Genentech
Shanti Chari, Assistant Vice President, Digital Technologies and Innovation, Landmark Bio

12:10 pm Enjoy Lunch on Your Own

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

DATA INFRASTRUCTURE & MANAGEMENT

1:25 Chairperson's Remarks
Jun Huang, PhD, Senior Director, Preclinical Manufacturing and Process Development, Regeneron Pharmaceuticals Inc.
Cenk Undey, PhD, Vice President & Global Head, PTD Data & Digital, Roche/ Genentech

1:30 Advancing Digital and Data Infrastructure for Bioprocess Development
Jun Huang, PhD, Senior Director, Preclinical Manufacturing and Process Development, Regeneron Pharmaceuticals Inc.
Implementing a framework for unified IT/OT infrastructure and data management is foundational to digital process development. This framework of connected systems, data and analytics can be harnessed to allow end-to-end process visibility, improve comparability and predictability across scales and products. A holistic & pragmatic strategy will be discussed to advance data architecture, governance, integration, sharing and analytics consumption in support of our core mission to bring new medicines to patients.

2:00 A Digital Transformation Journey in Process Development – Building Automated Data Flows, from Equipment to eLN to Advanced Analytics
Christian Airiau, PhD, Global Head, Data Sciences Biologics Development, Sanofi
Sanofi CMC/Process Development is implementing a comprehensive Digital Transformation program to improve our productivity and reduce our development timelines. We are deploying a standardized digital workflow across our development sites globally (target: 2,000 users) and building automated data access to ensure scientists can document, visualize, and analyze their experimental work. We will discuss the progress, successes, and pain-points encountered by the development teams as we progress towards our digital ambition.

2:30 Lab-as-Code: Digitalizing Your Bioprocess Starts with Digitizing All of Your Instrument Data
Nathan Clark, Founder, Commercial|Product, Ganymede
Bioprocessing workflows are incredibly complex, not just in protocol, but also in data. Effective digitalization of these end-to-end processes requires rich and robust instrument and device integration. We'll explain why comprehensive data digitization has to precede any successful efforts at digital transformation. Join us as we review successful practices in this space, and discuss Ganymede's Lab-as-Code paradigm for easily and rapidly integrating your entire bioprocess.

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

PLENARY KEYNOTE: LEADING TO TOMORROW'S ADVANCES

3:50 Chairperson's Remarks
Ran Zheng, CEO, Landmark Bio

4:00 Current and Future Trends in Biomanufacturing of New Modalities
Konstantin B. Konstantinov, PhD, CTO, Ring Therapeutics
Using exosomes as an example, this presentation examines the current and future trends in biomanufacturing, and the technologies needed to manufacture emerging modalities at scale. Traditional biomanufacturing methods do not provide the industrialized, commercially scalable, highly efficient and reproducible manufacturing process essential for this new class of biotherapeutics—so we built it from the ground up.

4:30 The Digitalization of Biomanufacturing
Richard D. Braatz, PhD, Edwin R. Gilliland Professor, Chemical Engineering, Massachusetts Institute of Technology
A testbed is described for the end-to-end integrated and continuous manufacturing of monoclonal antibodies, which consists of parallel bioreactors, simulated moving bed chromatography systems, viral inactivation, and an autosampling system. Experimental results are compared with a digital twin. The increased consistency in the glycosylation profile of the monoclonal antibodies being produced is quantified when going from batch to semi-batch to perfusion mode, and when moving from start-up to quasi-steady conditions.

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

6:00 Close of Day

THURSDAY, AUGUST 17

7:30 am Registration and Morning Coffee

DIGITAL TWIN AND DATA SCIENCE

7:55 Chairperson's Remarks
Christoph Herwig, PhD, Founder, Lisalis GmbH, former Professor, Bioprocess Engineering, TU Wien
Oliver Hesse, Head, Biotech Data Science & Digitalization, Bayer U.S. LLC

8:00 Holistic Experimental Design and Deployment Strategies of Digital Twins for Accelerating Bioprocess Life-Cycling
Christoph Herwig, PhD, Founder, Lisalis GmbH, former Professor, Bioprocess Engineering, TU Wien
Acceleration of commercialization of biologics, including the filing of a robust control strategy, is of utmost importance for biosimilars up to new modalities. Digital twins capture CMC knowledge and allow multiple deployments. We will show how end-to-end digital twins can help save 50% of experimental effort by incorporating drug substance specification when designing unit operations and how real-time application allows for prediction and control on process performance.

8:30 Application of Physics-Informed Neural Networks in Real-Time Cell Culture Bioreactor Modeling
Huiyi Cao, PhD, Senior Scientist, Pfizer Inc.
Shu Yang, PhD, Senior Scientist, Pfizer Inc.
Monitoring and control of viable cell density, metabolite concentration, and titer is critical for optimizing the development and manufacturing of cell cultures. A real-time bioreactor model has been developed using the novel modeling approach, physics-informed neural networks. This framework combines the power of AI with the robustness of mechanistic laws to reliably predict key product attributes. A proof-of-concept of this model has been implemented and tested in a bench-scale bioreactor.

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

9:30 Breakout Discussion Groups
Breakout discussions provide an opportunity to discuss a focused topic with peers from around the world in an open, collegial setting. Select from the list of topics available and join the moderated discussion to share ideas, gain

insights, establish collaborations or commiserate about persistent challenges. Please visit the breakout discussions page on the conference website for a complete listing of topics and descriptions.

IN-PERSON ONLY BREAKOUT: Modeling Liquid-Liquid Mixing in Drug Product Process Development

Robert Kuo, PhD, Assoc Principal Scientist, Sterile & Specialty Products, Merck

- Critical inputs for creating a representative model
- Extracting actionable data and insights from simulations
- Validating results and impacting the process development outcome

IN-PERSON ONLY BREAKOUT: Steps Towards A Fullly Automated Bioprocess

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

- Process integration a prerequisite for automation
- What are the barriers for full automation?
- Which sensors do we need?
- Benefits of automation

10:30 Monitoring, Modeling, and Controlling the Basis for Automated and Autonomous Biomanufacturing

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

The long-term vision of biomanufacturing is autonomous bioprocessing. To achieve this state, it is necessary to automate bioprocesses, which require sensors to control a manufacturing system. Currently, for a lot of quality/process parameters sensors are not available and soft sensors and self-learning algorithms must be applied. The state-of-the-art of monitoring and control of bioprocesses will be provided and to which extent integrated continuous biomanufacturing necessitates autonomous bioprocessing.

11:00 Process Modeling for Ultrafiltration and Formulation

Poonam Phalak, PhD, Associate Director & Process Modeling Lead, GSK

Model-based approaches in the biopharma industry have the potential to accelerate decision-making, optimize the product to market time, and reduce costs. *In silico* representation of manufacturing processes is becoming easier thanks to analytical tools, process modeling software, and machine learning algorithms. In this contribution, we demonstrate the use of model-based approaches for decision-making for ultrafiltration and formulation processes.

11:30 Sponsored Presentation *(Opportunity Available)*

12:00 pm Enjoy Lunch on Your Own

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

PAT, APC AND AUTOMATION

1:05 Chairperson's Remarks

Antonio G. Cardillo, PhD, Scientific Lead Associate Director, TRD-DS Global Innovation Centre, GSK Vaccines

Reza Kamyar, PhD, Director of AI and Advanced Control Solutions, Global Technology & Engineering, Pfizer Inc.

1:10 PAT Deployment in GSK Vaccine from R&D to Manufacturing

Antonio G. Cardillo, PhD, Scientific Lead Associate Director, TRD-DS Global Innovation Centre, GSK Vaccines

Biopharmaceutical industry traditionally relies on pharmaceutical manufacturing practices to monitor processes and release products. The use of Process Analytical Technologies (PAT) can improve the process monitoring and control and at the same time increasing the process understanding and modelling capabilities. This talk examines the possible PAT application to vaccine processes using a phase- and technology-appropriate approach to reach a fully implemented in-line monitoring (ILM).

1:40 PAT and Automation for Robust Upstream Stem Cell Processing

Jens Traenkle, PhD, Head, PAT & Automation, Product Supply, Pharmaceuticals, Bayer AG

In this talk, we will present our recent developments in PAT and automation for transferring manual adherent iPSC cultivation processes to highly automated and fully closed cultivation systems. New PAT methods allow for rapid in-process testing of parameters specific to these new modalities and in combination with our robotized cell cultivation platform, the transition from a laboratory process to a closed and data-driven industrialized process is enabled.

2:10 Enabling Global Operations with Realtime Sensing: A Case Study in Digital Product Management

Cylia Chen, Director, Business Performance, Amgen

Brian McBreen, Director, Digital Product Management – Sensing, Amgen

In this session, you will learn about an important digital transformation effort at Amgen focused on delivering insights to senior leadership. The presentation will focus on a key use case for the global operations function (process development, manufacturing, quality, supply chain, etc.) along with the practice of digital product management. Learn how this approach improves agility as well as the nature of challenges that arise.

2:40 Networking Refreshment Break

2:55 Smart Process Analytics for the Prediction of Critical Quality Attributes in End-to-End Batch Manufacturing of Monoclonal Antibodies

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

For many modern biopharmaceutical processes, manufacturers develop data-driven models using data analytics/machine learning methods. The challenge is how to select the best methods for a specific dataset to construct the most accurate and reliable model. This presentation describes the application of smart process data analytics software to industrial end-to-end biomanufacturing datasets for monoclonal antibody production to automate the determination of the best DA/ML tools for model construction and process understanding.

3:25 Accelerated Raman Development for Implementation at Large-Scale

Kurtis Denny, Engineer I, Cell Culture Development, Biogen

Raman spectroscopy has been utilized for many different applications in cell culture bioprocesses, however, the adoption of PAT into commercial environments has been slow. A toolbox methodology will be shown with the aim of reducing time to implement Raman spectroscopy applications in cell culture processes.

3:55 Close of Summit

STREAM #2
DOWNSTREAM PROCESSING

The need for technological innovations is critical in downstream processing not only to meet the demands of higher upstream titer, but also to face the challenges of new modalities and complex molecules coming down the pipeline. Companies are looking toward strategies such as continuous and intensified processing, filter chemistry, scale-down modeling, digitalization, and automation to improve yield and productivity. Meanwhile, downstream scientists are upping their game in developing processes for complex and emerging biologics, while exploring advances in tools, materials and technologies for next-generation purification platforms. The Downstream Processing conferences will bring you on a voyage to discover these innovations driving the next wave of downstream development.

Conference Programs

AUGUST 14-15

Intensified & Continuous Processing

View Program »

AUGUST 16-17

Advances in Purification & Recovery

View Program »

MONDAY, AUGUST 14

8:00 am Registration and Morning Coffee

PERFUSION AND PROCESS INTENSIFICATION APPROACHES

9:55 Chairperson's Opening Remarks
Andrew Sinclair, MSc, CEng, FIChemE, FREng, President & Founder, BioPharm Services Ltd.

10:00 KEYNOTE PRESENTATION: Which Program Is Not Accelerated? Increasing Efficiencies in Process Development for Speed, Quality, and Safety
Gisela M. Ferreira, PhD, Director, AstraZeneca
The talk will exemplify some examples of business and scientific strategies that support program acceleration. Specifically, the exploration of column reuse for clinical production will be described as chromatography resins are largely underutilized during process development. While the study describes the use of three antibodies, the data supporting the proof of concept for resin reuse is demonstrated.

10:30 Implementation of N-1 Perfusion in Production of Biologics
Rok Brisar, PhD, Head of Tactical Manufacturing, Novartis
The production of recombinant proteins using mammalian cell culture has become an increasingly important process in the biopharmaceutical industry. The N-1 perfusion method has emerged as a promising approach for improving protein yields and reducing production costs. The aim of this discussion is to explore the advantages and disadvantages of this method compared to traditional batch and fed-batch processes.

11:00 Scaled-Down Models of N-1 Perfusion Enable Screening and Development of Intensified Upstream Fed-Batch Processes
Justin T. Huckaby, PhD, Process Development Scientist, Upstream Process Development, Shattuck Labs, Inc.
A high-seed density upstream fed-batch process was developed through the implementation of N-1 perfusion scale-down models in shake flasks and bench-scale bioreactors. A greater than 50% increase in harvest titer yields with comparable product quality was achieved as proof-of-concept for a bifunctional fusion protein using this intensified seed train process. By adding a few days to the seed train duration, we achieved significant gain in harvest titer while reducing COGS.

11:30 Integrating Synthetic Biology and Computer-Aided Design to Advance Biologics Production in CHO Cells
Scott Estes, PhD, Head of Cell Line Development, Asimov
In this talk, we present a CHO platform that moves beyond the one-size-fits-all paradigm by tailoring vectors for each molecule to optimize expression. Our system combines a GS knockout CHO host, transposase, genetic libraries, and computational tools. These tools enable development of high-expression lines, fine-tuning of chain expression, and ML-based optimization. We present case studies highlighting the impact of these tools to optimize expression for both standard monoclonal and bispecific antibodies.

12:00 pm LUNCHEON PRESENTATION: Accelerating CGT Process Development with Fully Integrated Perfusion Cell Culture
Yu Liu, PhD, Product Manager, Research and Development, ALIT LifeTech Inc
A novel perfusion technology and an integrated bioreactor are introduced to address major challenges in process development of cell & gene therapies. The perfusion technology provides efficient media exchange with low shear force, can be adopted for various cell types. The integrated bioreactor enables intensified cell culture with up to 10^8 cells/mL, captures comprehensive process data to guide process optimization and scale-up. Applications in HEK293, T-Cell, iPSC will be discussed.

12:30 Session Break

MAXIMIZING YIELD AND IMPROVING PERFUSION PERFORMANCE

12:50 Chairperson's Remarks
Gisela M. Ferreira, PhD, Director, AstraZeneca

12:55 Optimisation of Commercial-Scale Intensified Cell Culture
Andrew Sinclair, MSc, CEng, FIChemE, FREng, President & Founder, BioPharm Services Ltd.
Scaling up a bioprocess for manufacturing is complex and the impact of cell culture parameters influence manufacturing modalities. BioSolve Process incorporating Multi-objective Bayesian Optimization is used to analyse the complex design space to help identify optimal solutions. This case study identifies optimal configurations in terms of Fed Batch, Perfusion, or Intensified Fed Batch. The outcomes of the optimisation studies identify those factors that maximise economic, and sustainable benefits.

1:25 Maximizing Yield of Perfusion Cell Culture Processes: Evaluation and Scale-Up of Continuous Bleed Recycling
Christoph Herwig, PhD, Founder, Lisalis GmbH, former Professor, Bioprocess Engineering, TU Wien
Bleed recycling is an innovative method to enhance yield in steady-state perfusion processes by concentrating process bleed, selectively removing biomass, and recycling the liquid fraction. This saves significant product otherwise wasted. Inclined gravity settling was compared to acoustic separation as bleed recycling technologies. With similar efficiency and no negative impact on cell viability, nutrient levels, or product quality, it emerged as preferred technology due to its reduced complexity and scalability.

1:55 Removal of problematic host cell proteins by the new 3M™ AEX technology "3M™ Polisher ST" post protein A column
Mauhamad Baarine, Bioprocessing Application Specialist, PhD, Biopharmaceutical purification, Separation and Purification Sciences Division, 3m
The single-use 3M™ Polisher ST, launched in 2021, is an anion exchange device that uses a novel guanidinium chemistry. The guanidinium functional group decorating the 3M™ Polisher ST functional membrane is unique. Unlike other commercially-available media, it interacts with negatively-charged carboxylic acid residues of proteins, not only electrostatically, but through 2 in-plane H-bonding interactions. This gives 3M™ Polisher ST high contaminant removal capacity including HCPs over wide ranges of fluid conditions!

2:25 Networking Refreshment Break

2:40 Evaluating Filter Chemistry as a Lever for Improving Perfusion Performance
Alexandria Triozzi, Engineer I, Biogen
Sieving efficiency decline caused by foulants in therapeutic biologics production using continuous manufacturing (CM) is a significant challenge. Membrane fouling may lead to filter failure and eventually batch failure. Additionally, it is undesirable to pass large quantities of foulants through the filter as it may complicate the downstream purification process. Here, we evaluated three commercially-available, commonly-utilized membrane chemistries from multiple manufacturers and compared their sieving efficiency performance.

3:10 Evaluation of a Single-Use Small-Scale Continuous Centrifuge as a Scale-Down Model for Future Manufacturing Continuous Disc Centrifuge
Hirenkumar Panchal, Research Investigator, Incyte Corp.
Lack of a proper scale-down model makes the implementation of continuous centrifugation usually a try-and-error operation directly at large-scale. In this study, with the intention to develop a proper scale- down model, we side-by-side compared a single-use pilot-scale centrifuge to a bench-top centrifuge. Turbidity, lactate dehydrogenase (LDH), and host cell protein were all evaluated for comparison.

3:40 Session Break and Transition to Plenary Keynote Session

PLENARY KEYNOTE: SOLVING TODAY'S CHALLENGES

4:20 Chairperson's Remarks
Susan D'Costa, PhD, CTO, Genezen

4:30 Overcoming the Challenges of Bioprocesses: The Future of Biomanufacturing
Glen R. Bolton, PhD, Executive Director, Late Stage Bioprocess Development, Amgen, Inc.
Novel therapies and technologies are emerging to meet the needs of patients; however, the manufacturing of biopharmaceuticals remains a complex and challenging process. As demand for biopharmaceuticals grows, the industry faces new challenges in terms of scalability, cost, and process robustness. The implementation of innovative technologies to improve process efficiency and the importance of process control and data analytics in ensuring process robustness are key levers to meet these challenges.

5:00 Commercializing Gene Therapies—The Combined Power of Patient Advocacy and Cost-Effective Manufacturing
Rachel Salzman, DVM, Founder, The Stop ALD Foundation; Global Head, Corporate Strategy, Armatus Bio
This presentation will examine the development of an FDA-approved gene therapy where patient advocacy played a critical role resulting in the first ever clinical use of a lentiviral vector. Although manufacturing continues to represent a significant challenge throughout the entire R&D journey, there are opportunities for advocacy and manufacturing communities to seek alignment and combine their collective powers to achieve the common goal of increasing patient access to transformative medicines.

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

6:30 Close of Day

TUESDAY, AUGUST 15

7:30 am Registration and Morning Coffee

DIGITALIZATION AND MECHANISTIC MODELING FOR CONTINUOUS PROCESSING

7:55 Chairperson's Remarks
Stefan R. Schmidt, PhD, MBA, CEO, evitria AG

8:00 End-to-End Mechanistic Models of Integrated and Continuous Biomanufacturing Processes
Nehal Patel, Downstream Bioprocessing Practice Director, Digital Industries Process Automation Software, Siemens
Robert Taylor, PhD, Associate Scientist, Bioseparation Sciences, Merck Manufacturing Division
We will describe examples of how Siemens customers are building and applying dynamic end-to-end mechanistic models of integrated and continuous biomanufacturing processes (ICB) to determine the impact of expected disturbances, deviations, and uncertainties on product quality. We will show practical examples where these models can generate value by performing tasks that are not possible experimentally due to the prohibitive material requirements and complexity of building end-to-end processes in the lab.

8:30 Moving towards Advanced Automation of Continuous Processing
Sean Ruane, PhD, Senior Data Scientist, CPI
In the Integrated Continuous Biomanufacturing project, CPI and its partners have produced an end-to-end continuous mAb production and purification system that demonstrates the possibilities of Advanced Process Control, where CQAs are measured in real-time and controlled in a flexible process. The system also utilises a flexible digital architecture to enable model-based control while maintaining robustness, and a novel flow-balancing architecture to greatly simplify continuous processing.

9:00 A Spiking-Augmentation Method to Improve the Prediction Performance of FTIR-Titer Model on New Molecules
Yuxiang Zhao, PhD, Scientist, Bristol Myers Squibb Co.
Intensified and continuous processes require fast and robust methods for in-line titer monitoring. FTIR and chemometric-based multivariate modeling are promising tools for real time titer monitoring. This presentation demonstrates an adaptive modeling strategy: the model was initially built using a calibration set of available CB samples and then updated by augmenting spiking samples of the new molecules to the calibration set to improve the model robustness.

9:30 Talk Title to be Announced
Ugur Gulmen, Research Associate II, Resilience

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

10:45 Breakout Discussion Groups
Breakout discussions provide an opportunity to discuss a focused topic with peers from around the world in an open, collegial setting. Select from the list of topics available and join the moderated discussion to share ideas, gain insights, establish collaborations or commiserate about persistent challenges. Please visit the breakout discussions page on the conference website for a complete listing of topics and descriptions.

IN-PERSON ONLY BREAKOUT: Bioreactor Design and Optimization for Continuous Bioprocessing
Jean-Francois P. Hamel, PhD, Lecturer, Chemical Engineering, Massachusetts Institute of Technology
• Assessing microbial and animal cell benchtop bioreactors, designed for fed-batch or continuous applications (e.g., perfusion) and scale-down studies.
• Choosing microfluidics and benchtop bioreactors (traditional and single use) for screening, optimization and process development.

IN-PERSON ONLY BREAKOUT: Transient Expression vs Stable (Pool) Cell Lines
Stefan R. Schmidt, PhD, MBA, CEO, evitria AG
• When to choose what mammalian expression system?
• Effort, expression level, timelines, typical purposes, cost, scale, quality, development lifecycle
• Pros and cons for the different approaches

TOWARD COMMERCIAL-SCALE AND SUSTAINABLE BIOMANUFACTURING

11:30 Intensification Strategies: Moving from Lab-Scale to Clinical- and Commercial-Scale

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

Processes can be intensified at all scales and at all dimensions. However, that requires implementing approaches to achieve "more, with less efforts, faster" already at the beginning. This presentation gives a comprehensive overview on strategies how to integrate process intensification through the whole product life cycle and when you switch scales and facilities. The opportunities from early development to continuous process improvements will be summarized in this talk.

12:00 pm Sustainable Biologics Manufacturing – Current State and Future Outlook

Sri Madabhushi, PhD, Associate Principal Scientist & Associate Director, Merck

Sustainability of biologics manufacturing processes is critical in ensuring the efficient production of these life-saving therapies in a resource constrained world. This presentation will provide an overview of the current state of biologics sustainability for different modalities and discuss the findings from process mass intensity (PMI) and life cycle assessments (LCA). The work highlights the need for a comprehensive metric(s) that will drive innovations in sustainability of biologics manufacturing.

12:30 Adopting Digital Transformation and Machine Learning in a CDMO Startup Wheeler Bio

Deepika Verma, Ph.D., Associate Director of Data Science and Digitalization, Wheeler Bio, Inc.

Wheeler Bio is a biomanufacturing pioneer embracing Pharma 4.0 model to create and deliver speed and efficiency in drug development and clinical manufacturing. The talk will cover Wheeler Bio's strategy for creating digital infrastructure, automating data acquisition and integration, and using data science tools to ultimately achieve the goal of building a mature digital twin for rapid development of commercial-ready manufacturing processes.

1:00 LUNCHEON PRESENTATION: Examples of In-Line Harvest Characterization through Particle Analysis, Spectroscopy, and Biophysical Sensors METTLER TOLEDO

Tyler Gable, PhD, Market Development Manager, METTLER TOLEDO

Acidic precipitation of nucleic acids in cell culture broth was evaluated to improve filtration and pre-chromatography impurity clearance. Alternative methods of HCP clearance and filtration outcomes with different flocculation reagents were also evaluated. In each process, offline particle analysis and filtration throughput studies alone were insufficient to fully characterize the processes. Inline particle characterization was utilized to understand clearance mechanisms and optimize harvest.

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

DOWNSTREAM PROCESS INTENSIFICATION

2:10 Chairperson's Remarks

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

2:15 Ultrafiltration of Adeno-Associated Virus Clarified Cell Lysate for Downstream Process Intensification

Christopher Yehl, PhD, Scientist, Downstream Process Development, Spark Therapeutics, Inc.

Affinity Chromatography operational time is directly related to affinity load volume. Implementing an ultrafiltration step to concentrate AAV Clarified Cell Lysate (CCL) prior to Affinity loading can reduce overall operational time, maintain product quality, reduce cost of goods, and simplify the manufacturing procedure. Three commercially available membranes were evaluated over a range of conditions to show proof of concept, reproducibility, scalability, maintained or improved product quality and high product recovery.

2:45 Development of a Simplified Scaled-Down Model for Characterization of a Multi-Column Continuous Protein A Operation

Lauren D. Powers, Senior Scientist, Merck

Continuous manufacturing for mAbs, involving multi-column capture, has demonstrably improved productivity. Process characterization of multi-column ProA, requires substantially large volumes of material, long run duration to achieve steady state, and operational complexity of a closed, sterile system. This talk will explore the opportunities of utilizing a single column as a scale-down model for continuous chromatography process characterization of a multi-column capture step, and share the lessons learned using this approach.

3:15 Optimizing Continuous Chromatography through MPC and EKF: A Novel Approach to Address Resin Aging

Touraj Eslami, PhD, Automation Engineer, Downstream Processing, Institute of Bioprocess Science and Engineering, University of Natural Resources & Life Sciences

The aging of chromatography columns impacts process economics intensively, including productivity, resin utilization, and buffer consumption. Our online optimization approach employs a residence time gradient during the loading step to balance these demands. This approach can forecast optimal conditions to maximize productivity and resin utilization. Results showed a savings of up to 43% in buffer consumption and increased productivity and resin utilization beyond the feasible range with classic chromatography.

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

INTENSIFIED PROCESSES FOR NOVEL & EMERGING BIOLOGICS

4:30 Utilizing Retrovirus-like Particles (RVLP) to Evaluate Viral Clearance for Multiple Modes of Separation CYGNUS TECHNOLOGIES part of Maravai LifeSciences

David Cetlin, Senior Director, MockV Products, Cygnus Technologies

A highly concentrated and purified stock solution of CHO-derived Retrovirus Like Particles (RVLP's) has been used as a BSL-1 compatible spiking agent for viral clearance studies. In this presentation we will compare the Log Reduction Values derived from RVLP's vs XMuLV over multiple modes of separation, including; Protein A, virus filtration, CEX, AEX and Mixed Mode chromatography.

5:00 PANEL DISCUSSION: Intensified Processing for Novel Modalities – mRNAs, AAVs, EVs, and More: Hype vs. Reality

Moderator: Philip Probert, PhD, Technology Lead, CPI, United Kingdom

Novel modalities have the unrealised potential to revolutionise the treatment of disease. Access to these therapies, however, is limited by the high cost of goods of these products related to scale-up and yield challenges. Process intensification provides a solution to these issues – in this panel the current state-of-the-art, limitations, and future perspectives will be discussed.

Panelists:

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

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

Christopher Yehl, PhD, Scientist, Downstream Process Development, Spark Therapeutics, Inc.

Helen Young, PhD, Manager, Synthetic & Mammalian Upstream, CPI

5:30 Close of Intensified & Continuous Processing Conference

WEDNESDAY, AUGUST 16

7:30 am Registration and Morning Coffee

DOWNSTREAM PROCESS INNOVATIONS FOR COMPLEX & EMERGING BIOLOGICS

7:55 Chairperson's Remarks

Ronald Bates, PhD, Vice President, CMC Process Development & Technology Operations, Immunovant

8:00 KEYNOTE PRESENTATION: Platform Approaches for a Diverse Pipeline: Engineering Ways of Working for Robust Separations

Kevin P. Brower, PhD, Global Head, Purification Process Development, Sanofi

Modality diversity and variable separation challenges have become increasingly prevalent in the portfolios in biotechnology. Despite this complexity, timeline and resource pressures remain. In this presentation, we describe Sanofi's purification platform approaches, including separation science, establishment of work packages, and application of novel engineering technologies in high-throughput and integrated continuous biomanufacturing to meet the challenge of our diverse pipeline of mAbs, multi-specifics, antibody-drug conjugates, and therapeutic proteins.

8:30 Downstream Processing of Allogeneic iNK and iT Cell Therapy

Christopher Deborde, PhD, Process Development Engineer, Century Therapeutics

Allogeneic iPSC-derived cell therapies have shown encouraging preclinical and clinical promise. These therapies have the potential to treat a wide range of indications and be readily available to patients. As therapeutic technologies progress, the requirement to support clinical-to-commercial scale production becomes more critical. To that end, this presentation will highlight the downstream processing, purification, and drug product formulation strategies that meet the requirements of both patients and cell therapy manufacturers.

9:00 Identifying and Remediating Root Cause of Particle Formation in Drug Product Presentations

Ronald Bates, PhD, Vice President, CMC Process Development & Technology Operations, Immunovant

This paper will detail elements of an investigation into the formation of particles in a drug product formulation. The paper will focus on the drug substance components of the investigation.

9:30 SIMPLIFYING BIOPROCESSING: Why Automated Bag Filling is a Major Win in DSP

Mike Marciniak, Senior Director, Sales, North America, Single Use Support

How do you get your purified bulk to its final formulation and fill site safely and efficiently? Automated filling technologies facilitate fluid management significantly for manufacturers in the downstream bioprocessing of high-valued bulk drug substances, cell therapies, viral vectors or mRNA-assisted therapies. It's time to simplify fluid transfers.

9:45 Purification Strategies for Viral Gene Therapy Vectors

William Rushton, Process Chromatography Scientist, Process Chromatography, Bio-Rad Laboratories

Recombinant adeno-associated viruses (rAAV) are among the most promising vectors for long-term gene transduction. These viruses have a high degree of safety, making them ideal for gene therapy applications. Significant progress has been made in improving rAAV vector production and purification. In this study, different chromatography workflow solutions were explored to purify rAAV8. Experimental conditions and data will be presented on using anion exchange and mixed-mode chromatography to purify rAAV8.

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

10:40 Capillary-Channeled Polymer Fibers: A Singular Platform for the Purification of Diverse Vector Types

R. Kenneth Marcus, PhD, Professor, Chemistry, Biosystems Research Complex, Clemson University

This laboratory has developed a novel family of polymer fiber stationary phases to affect the isolation and purification of exosomes using a hydrophobic interaction chromatography elution scheme. Here we will demonstrate the breadth of applicability of the approach, extending it to further sources of exosomes including bovine milk and plants. The same method has now been applied to lentiviruses and adeno-associated viruses (AAVs) and lipid nanoparticle (LNP) vectors as well.

11:10 Computational Modeling of Protein A Resin Slurry in a Mixing Tank

Chadakarn Sirasitthichoke, PhD, Process Engineer, MS&T Process Analytics and Engineering, Bristol Myers Squibb Co.

Protein separation is an important purification step in a biopharmaceutical downstream process. Prior to column packing, homogenous resin slurry is necessity for optimal column packing and effective chromatographic purification. Agitation speed, packing buffer, and resin solid percentage are factors to achieve the homogeneity. Computational Fluid Dynamics (CFD) is used as a simulation tool to guide those operating parameters for slurry suspension and thus providing a more consistent column packing process.

11:40 Overcoming Supply Chain Challenges for Various Filters Used in Commercial Biologics Downstream Manufacturing

Elizabeth Pontius, BSc, Associate Engineer/Scientist, MSAT, Bristol Myers Squibb Co.

Filtration is an essential component in biologics downstream manufacturing. The COVID pandemic has caused a global supply shortage in filters used in various downstream processing steps. To ensure manufacturing continuity and uninterrupted delivery to patients, innovative solutions are needed to overcome these challenges. Alternative filter evaluations and sizing studies have been performed to mitigate stockout risks for several filters used in commercial biologics manufacturing.

12:10 pm LUNCHEON PRESENTATION: Process Intensification of Affinity Step of Antibody Purification through Screening of Resins and Additives

Calvin Cheah, Scientist, R&D, Research - Bioprocessing Applications, Avantor

Downstream purification of antibodies encounters issue of optimization and scale-up when different complex molecules are considered for multiple chromatographic steps. In this session, we will review common challenges faced during the affinity step of DSP and discuss the troubleshooting practices and methodologies to optimize the process through screening of different resins and additives. The case studies will include different types of antibodies including fragment antibodies where the process is initially developed in the lab scale and 100X scale up was achieved successfully.

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

DOWNSTREAM PROCESS INNOVATIONS FOR COMPLEX & EMERGING BIOLOGICS (CONT.)

1:25 Chairperson's Remarks
Ronald Bates, PhD, Vice President, CMC Process Development & Technology Operations, Immunovant

1:30 Developing High-Productivity Mixed-Mode CHT Chromatography Purification Step for Complex Biologics
Patrick Staaf, Associate Scientist, Late Stage Process Development, Bristol Myers Squibb
This study details development and provides insight on the alternative approach to using mixed-mode CHT chromatography with complex biologics within the downstream purification process.

2:00 Evaluating the Impacts of Dual Salts and Organic Modifiers on Purification of Antisense Oligonucleotide via Anion Exchange Chromatography
Armin Delavari, PhD, Scientist II, Technical Development, Biogen
Antisense oligonucleotides are short single-strand modified RNA/DNA sequences that are designed to treat genetic disorders by eliminating target mRNAs via specific binding. The safety and efficacy of these therapeutics can be affected by their purities, and therefore, a robust purification process is required. In this study, we explored novel approaches to enhance impurity clearance using anion exchange chromatography polishing step using dual salt systems and organic modifiers.

2:30 Advances in Protein A Chromatography Resins
Aaron Moulin, PhD, Field Application Scientist, Bioprocessing, Purolite, An Ecolab Company
Jetting technology is a continuous emulsification technology by which all Praesto® chromatography resins are produced. This proprietary technology results in resins with a narrow, almost uniform particle size distribution, with excellent mass transfer properties. Herein, we present advances which utilize Jetting technology, including process intensification models and a novel Protein A resin designed specifically for elution of Fc-containing molecules at higher pH levels.

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

PLENARY KEYNOTE: LEADING TO TOMORROW'S ADVANCES

3:50 Chairperson's Remarks
Ran Zheng, CEO, Landmark Bio

4:00 Current and Future Trends in Biomanufacturing of New Modalities
Konstantin B. Konstantinov, PhD, CTO, Ring Therapeutics
Using exosomes as an example, this presentation examines the current and future trends in biomanufacturing, and the technologies needed to manufacture emerging modalities at scale. Traditional biomanufacturing methods do not provide the industrialized, commercially scalable, highly efficient and reproducible manufacturing process essential for this new class of biotherapeutics—so we built it from the ground up.

4:30 The Digitalization of Biomanufacturing
Richard D. Braatz, PhD, Edwin R. Gilliland Professor, Chemical Engineering, Massachusetts Institute of Technology
A testbed is described for the end-to-end integrated and continuous manufacturing of monoclonal antibodies, which consists of parallel bioreactors, simulated moving bed chromatography systems, viral inactivation, and an autosampling system. Experimental results are compared with a digital twin. The increased consistency in the glycosylation profile of the monoclonal antibodies being produced is quantified when going from batch to semi-batch to perfusion mode, and when moving from start-up to quasi-steady conditions.

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

6:00 Close of Day

THURSDAY, AUGUST 17

7:30 am Registration and Morning Coffee

ADVANCES IN TOOLS, MATERIALS, AND TECHNOLOGIES FOR NEXT-GEN PURIFICATION PLATFORMS

7:55 Chairperson's Remarks
Kevin P. Brower, PhD, Global Head, Purification Process Development, Sanofi

8:00 High-Resolution 3D Imaging to Visualize and Characterize 3D-Printed Chromatography Columns
Thomas F. Johnson, PhD, Senior Research Fellow, Biochemical Engineering, University College London
3D design and printing bioprocessing structures enable the chemical and physical characteristics of downstream media to be specifically tailored to the product of interest, particularly important due to the emerging popularity of advanced modalities. In this study we apply high resolution imaging to view multiple length scales analogous to packed bed chromatography that informs the design of next-generation DSP materials.

8:30 POSTER HIGHTLIGHT: Buffer Concentrates for Biotherapeutic Pilot Economization
Philip Hansel, Assoc Scientist III, 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 seven 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 50%, preparation time by 66%, and storage space by 50%.

8:45 POSTER HIGHLIGHT: A Combined Inducible Mammalian Expression System and Novel Affinity Tag Provides a Powerful Tool for the Productivity of Difficult-to-Express Proteins
Megan Batson, Scientist, Large Molecule Research, Sanofi
Mammalian expression of recombinant proteins with the desired quality and post translational attributes can be extremely challenging due to low expression and poor purity. To address this challenge, we at Sanofi implemented a two-armed approach to optimize our expression and purification methods for difficult-to-express proteins. The Expi293F Inducible system combined with an alternative multipurpose affinity tag proved to be a great tool for mg-scale production of difficult-to-express proteins.

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

9:30 Evaluating Biopharma - Fireside Chat and Networking Session

Subject matter experts sit down 1:1 with our moderator to discuss and share their personal bioprocessing experiences, insights, and advice. The real "pay it forward" atmosphere provides biopharma leaders with unique opportunities to leverage and apply their expertise to make better technology, process, and business decisions, and, ultimately, to accelerate success. Dedicated networking within the session allows all attendees to follow up and dive deeper into conversation.

9:30 FIRESIDE CHAT Rethinking Separation Processes to Support a Diverse Pipeline

Kevin P. Brower, PhD, Global Head, Purification Process Development, Sanofi

9:30 FIRESIDE CHAT Presentation to be Announced

Mary Ruberry, Revelate Communications

9:50 FIRESIDE CHAT Considerations when Implementing a Downstream Intensification Strategy

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

9:50 FIRESIDE CHAT Presentation to be Announced

Mary Ruberry, Revelate Communications

10:10 Networking Session

Full room networking provides all attendees opportunity to engage with speakers and fellow attendees. Ask questions, discuss ideas, walk away with ideas to improve process success.

10:30 Viral Clearance Capability of Biotechnology Product Manufacturing Process: IND Viral Clearance Database Case Study

Opeyemi Ajayi, PhD, Scientist II, CDER/OPQ/OBP, FDA

Clearance of endogenous and adventitious viruses is an important consideration for any mammalian cell-derived biotechnology product. Process changes in the investigational phase may impact clearance capabilities depending on the applied unit operations and parameters. Data from an in-house database created to evaluate these process parameters and their impact on clearance will be presented.

11:00 Leveraging Risk-Based Approaches, Process Knowledge, and Appropriate Strategies for the Design of Process-Relevant Impurity Clearance Challenge Studies

Raj Prabu Vijayakumar Saraswathi, Principal Scientist, Biologics Process Dev, Alkermes Inc

This talk focuses on designing a process relevant impurity clearance challenge studies. We used risk-based approach to identify process-related impurities that warranted impurity clearance challenge studies, and applied three different strategies to design the studies: upstream worst case material, column overloading approach and by-pass approach. Data generated will be used to set acceptance criteria wihtin the process control strategy.

11:30 Talk Title to be Announced

Himanshu Gadgil, PhD, Chief Executive Officer, Enzene Biosciences Ltd.

12:00 pm LUNCHEON PRESENTATION: Enabling Single-Use Manufacturing with High Productivity Protein A Membrane Up to 2000L Scale

William Barrett, PhD, Product Specialist, PharmBIO, W.L. Gore and Associates, Inc.

Presenting results from incorporating a scalable rapid cycle GMP supported Protein A affinity membrane from single use downstream operation as well as the processing of a monoclonal antibody cell culture harvest showing high productivity at the affinity capture step. Purification protocols show capability to clear up to 10 g/L titers at 2000 L scale. Quality attributes established with incumbent resin purification were met. Consistent scaling from lab scale columns was demonstrated.

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

OPTIMIZING DOWNSTREAM PROCESSING FOR AAVs

1:05 Chairperson's Remarks

Meisam Bakhshayeshi, PhD, Senior Director and Head of CMC, Intergalactic Therapeutics

1:10 Advanced AEX Platform for AAV Enrichment

Yonatan Abune, Senior Engineer, Process Development, Myeloid Therapeutics

In this investigation, we employed AEX chromatography to simultaneously enrich full AAV capsid and eliminate endotoxin from the final drug product. By optimizing the column conditions, we achieved a high level of AAV capsid purity, as well as a significant reduction in endotoxin levels. This strategy offers a promising solution for the production of safe and effective AAV-based therapeutics.

1:40 Lessons Learned: A Case Study in the Downstream Optimization of an AAV5 Production Process

Ashton Lavoie, PhD, Associate Director, Downstream Process Development, BridgeBio Gene Therapy

Manufacturing strategies and progress towards platform processes for adeno-associated virus (AAV) production have seen substantial advancement in support of the impressive clinical success for this modality. This presentation will provide a case study for the development of a robust, high yield downstream process for the production of AAV serotype 5. Key findings will be discussed in addition to pitfalls and challenges in this development work.

2:10 Improved Host Cell Protein Reduction through Affinity Chromatography in Adeno-Associated Virus Purification Process

Wenjun Di, PhD, Scientist I, Ultragenyx Pharmaceutical

In recombinant adeno-associated virus (rAAV) purification process, host cell proteins (HCPs) were reduced in multiple unit operations to ensure sufficient clearance in drug substance. Here, we demonstrated that additives could be introduced in affinity chromatography step to further reduce HCPs while maintaining rAAV genome recovery.

2:40 Networking Refreshment Break

2:55 Altering the Adsorption Dynamics of Empty Capsids on Anion Exchangers for the Enrichment of Full rAAV Particles

Ronald Jenkins, PhD, Senior Director, Passage Bio

3:25 Studying AAV Capsid Aggregation in Complex Matrix of Clarified Lysate

Yulia Ivanova, PhD, Principal Scientist, Bioprocess R&D, Pfizer Inc.

Recovery out of harvest is the least understood step in downstream purification of AAV vectors. Complexity of lysed cell culture coupled to relatively low protein concentration of AAV product makes it very difficult to analytically investigate this process space. Here we evaluate the ability of dynamic light scattering (DLS) to serve as analytical characterization tool that would allow AAV capsid aggregation investigation in a complex matrix of clarified harvest.

3:55 Plasmid Purification Process Development for Gene Therapy Applications

Jacob C. Cardinal, Associate Scientist III, Biogen

This talk may cover topics including selection of appropriate purification methods, process optimization, and quality control measures to ensure high-quality plasmid DNA for gene therapy applications.

4:25 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, viral and non-viral-based. Topics include product and process characterization, potency assays, comparability, emerging analytical technologies, impurities, quality control, comparability, process development, purification, formulation, scale-up, and commercial manufacturing.

Conference Programs

AUGUST 14-15

Gene Therapy CMC and Analytics

View Program »

AUGUST 16-17

Gene Therapy Manufacturing

View Program »

MONDAY, AUGUST 14

8:00 am Registration and Morning Coffee

BRINGING GENE THERAPIES TO MARKET

9:55 Chairperson's Remarks
Susan D'Costa, PhD, CTO, Genezen

10:00 KEYNOTE PRESENTATION: Commercializing Gene Therapies
Vesselin Mitaksov, PhD, Associate Research Fellow, Global Biologics, Pfizer Inc.
The presentation will focus on Pfizer's experience with late-stage rAAV product development in preparation for commercialization. Case studies presented will highlight aspects of rAAV control strategy development and eventual implementation for commercial manufacture. Examples will include challenges and solutions associated with product and process understanding to identify and control product critical quality attributes and to inform a comprehensive comparability assessment to support process changes throughout rAAV product development history.

10:30 PANEL DISCUSSION: Preparing for Launch and Late-Stage Development
Moderator: Susan D'Costa, PhD, CTO, Genezen
Panelists:
Lyndi Rice, PhD, Head, Gene Therapy Analytical Technologies, BioMarin
Mark Galbraith, PhD, Vice President, Analytical Sciences, Affinia Therapeutics
Svetlana Bergelson, PhD, Senior Director, Technology Development, Biogen
Vesselin Mitaksov, PhD, Associate Research Fellow, Global Biologics, Pfizer Inc.
Santoshkumar L. Khatwani, PhD, Director, Analytical Development, Sangamo Therapeutics

11:30 Real-Time Analytics from Really Tiny AAV & Lentivirus Volumes with Stunner & Leprechaun
Ross Walton, PhD, Sr. Applications Scientist, Unchained Labs
Checking in on your production processes requires low volume analytics that give rapid answers on what your next move should be. Stunner delivers near real-time data on capsid titer, empty/full ratio, and aggregation for AAVs. Leprechaun helps solve your lentiviral riddle by dishing out the lentiviral titer and percentage of capsid-containing virus from crude and pure samples, while providing information on viral aggregation, soluble p24 and non-viral EV contaminants.

12:00 pm LUNCHEON PRESENTATION: In-line Instrumentation Challenges in Viral Vector Purification
Joe Hewitt, Principal, EnVision Instruments LLC, EnVision Instruments LLC
Viral vector purification presents some unique challenges. In many cases, legacy in-line instrumentation
that was well suited for traditional mAb processes fails to meet the requirements of commercial viral vector
production. Recent technical advances offer new options for the most challenging applications.

12:30 Session Break

DEVELOPING FUNCTIONAL AND POTENCY ASSAYS

12:50 Chairperson's Remarks
Mark Galbraith, PhD, Vice President, Analytical Sciences, Affinia Therapeutics

12:55 Gene Therapy Potency Methods: Method Lifecycle Management and Optimization
Lyndi Rice, PhD, Head, Gene Therapy Analytical Technologies, BioMarin
Potency methods remain a significant challenge for gene therapy programs and can lead to program delays. Best practices for potency method development, transfers, and validation will be discussed, including proven analytical strategies of lifecycle management for potency methods. Case studies demonstrating method comparability considerations and method optimization for validated methods will also be covered.

1:25 Potency Method Development and Bridging Strategies
Ping Carlson, PhD, Director, Bioassay, Passage Bio
Potency is the Critical Quality Attribute (CQA) for Gene Therapy (GTx) manufacturing most related to the Mechanism of Action (MoA) of GTx. This presentation will discuss strategies for potency method development, phase-appropriate validation, method bridging, and life cycle management.

1:55 Rapid Characterization of Adeno-Associated Virus Samples: Full & Empty Capsids, Viral Proteins & ssDNA
Anubhav Tripathi, PhD, Professor, Engineering & Medical Sciences, Brown University
Adeno-Associated Virus (AAV) has shown great potential in gene therapy. There exists the need for fast, high-throughput characterization systems using low volumes to determine the sample composition of full/empty capsids, size, and Viral Protein and DNA concentration. Using an innovative microfluidics approach, we estimate the % of full capsids of AAV8 samples with 5% average deviation, offering an effective and high throughput way to evaluate the quality and purity of AAVs.

2:25 Networking Refreshment Break

2:40 Development and Optimization of a Functional Potency Assay for Late-Stage AAV Gene Therapy
Debashree Basu, PhD, Senior Scientist, Analytical Development, Ultragenyx Pharmaceuticals
Potency is a critical quality attribute and potency assays are an important regulatory requirement of late-stage AAV gene therapy. A functional potency assay quantitatively measures a Mechanism-of-Action (MOA)-based biological function. In this presentation, development and optimization of a quantitative, MOA-based *in vitro* functional potency assay for an AAV gene therapy product will be discussed. Challenges in cell line development and strategies for their resolution will be explored as well.

3:10 Cell Assay Development for Gene Therapy
Rajeev Boregowda, PhD, Associate Director, Bioassay and Molecular Analytical Development, Genomic Medicine CMC. Sanofi
While cell and gene therapies offer hope for non-curable diseases; developing analytical methods for these treatments is a complicated and resource-demanding process. Due to complexities around the MOA of gene therapies, more than one *in vitro* method is required to truly represent the MOA and be suitable to implement product conformance testing, stability testing, and comparability studies.

3:40 Session Break and Transition to Plenary Keynote Session

PLENARY KEYNOTE: SOLVING TODAY'S CHALLENGES

4:20 Chairperson's Remarks
Susan D'Costa, PhD, CTO, Genezen

4:30 Overcoming the Challenges of Bioprocesses: The Future of Biomanufacturing
Glen R. Bolton, PhD, Executive Director, Late Stage Bioprocess Development, Amgen, Inc.
Novel therapies and technologies are emerging to meet the needs of patients; however, the manufacturing of biopharmaceuticals remains a complex and challenging process. As demand for biopharmaceuticals grows, the industry faces new challenges in terms of scalability, cost, and process robustness. The implementation of innovative technologies to improve process efficiency and the importance of process control and data analytics in ensuring process robustness are key levers to meet these challenges.

5:00 Commercializing Gene Therapies—The Combined Power of Patient Advocacy and Cost-Effective Manufacturing
Rachel Salzman, DVM, Founder, The Stop ALD Foundation; Global Head, Corporate Strategy, Armatus Bio
This presentation will examine the development of an FDA-approved gene therapy where patient advocacy played a critical role resulting in the first ever clinical use of a lentiviral vector. Although manufacturing continues to represent a significant challenge throughout the entire R&D journey, there are opportunities for advocacy and manufacturing communities to seek alignment and combine their collective powers to achieve the common goal of increasing patient access to transformative medicines.

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

6:30 Close of Day

TUESDAY, AUGUST 15

7:30 am Registration and Morning Coffee

PRODUCT-RELATED IMPURITIES

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

8:00 Development of USP Standards to Support Gene Therapy Products
Anthony Blaszczyk, PhD, Senior Scientist, Global Biologics, US Pharmacopeia
Establishing relevant and applicable standards that apply to gene therapy is essential to maintaining safe and effective therapeutics, but the complexity and diversity of gene therapy products present unique challenges. USP is working with stakeholders and scientific experts to address challenges of standardizing materials and methods. This presentation will focus on the development of documentary and physical standards to support analysis of gene therapy products, process residuals, and raw materials.

8:30 Impurity Analysis of Gene Therapy Products
Santoshkumar L. Khatwani, PhD, Director, Analytical Development, Sangamo Therapeutics
This presentation will focus on the importance of demonstrating impurities clearance from the gene therapy products. In addition, some of the analytics necessary to evaluate impurities will be discussed. Topics include: importance of evaluating impurities in gene therapy products; type of impurities; analytics for measuring impurities; and demonstrating process performance for impurity clearance.

9:00 Analytical Toolbox for Characterizing Empty, Partial, and Full AAV Capsids and Linking Genome to Function
Aisleen McColl-Carboni, PhD, Senior Director, Analytical Development, Oxford Biomedica Solutions, Inc.
Manufacturing of AAV vectors is known to produce three types of capsids: empty, partial, and full. To specifically determine the impact of partial and empty capsid impurities on potency, an AAV preparation with a high amount of empty and partial capsids was separated into full, partial, and empty capsid populations and subjected to full analytical characterization. Capsid, genome, and potency data will be presented.

9:30 Is Viral Clearance Necessary for Gene Therapy Products?
Akunna Iheanacho, PhD, Chief Scientific Officer, Texcell - North America, Inc.
An important quality attribute of gene therapies is viral safety, and viral clearance studies are part of a multifaceted approach to ensure the safety of gene therapy products. This presentation will discuss updates to ICH Q5A and possible implications for viral clearance study design, as well as highlight strategies for integrating virus reduction capacity into gene therapy manufacturing processes.

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

10:45 Breakout Discussion Groups
Breakout discussions provide an opportunity to discuss a focused topic with peers from around the world in an open, collegial setting. Select from the list of topics available and join the moderated discussion to share ideas, gain insights, establish collaborations or commiserate about persistent challenges. Please visit the breakout discussions page on the conference website for a complete listing of topics and descriptions.

IN-PERSON ONLY BREAKOUT: Formulation, Stability, Delivery, and Forced Degradation Studies
Kruti Soni, PhD, Scientist, Technical Development, Biogen

ANALYTICAL TOOLBOX FOR EMPTY/FULL CAPSIDS

11:30 Analytical Development Toolbox for Characterization of Empty, Full, and Partial Capsids
Shreya Ahuja, Senior Principal Scientist, Analytical Department, Prevail Therapeutics
The development of AAV drugs involves assessing critical quality attributes using analytical methodologies. Assessment and quantification of empty/full ratios have proven to be challenging across the industry. Analytical ultracentrifugation is a gold-standard technique that has been used for years but it has its limitations. We have explored SEC-MALS, Mass Photometry, IEX, Capsid/vg ratio, and A260/280 to mitigate the drawbacks of analytical ultracentrifugation and have expanded our AAV characterization panel.

12:00 pm Analytics – Characterization of AAV Drug Product
Xiaozhu Sue Duan, Associate Director, Analytical Development, Astellas Gene Therapies
Stressed AAV drug product was analyzed in several stability indicating assays. Results indicated that *in vitro* assay was much more sensitive accessing degradation than *in vivo* assay. Among the stress conditions, a series of studies of photodegradation of white fluorescent light (WFL) were performed. The loss of potency was associated with total WFL exposure and was independent of the intensity.

12:30 Gene and Cell therapy CMC and Analytics
Patima Cherukuri, CSO, Genezen
Gene and Cell Therapy products present unique challenges in CMC (Chemistry, Manufacturing, and Controls), requiring innovative adaptations for successful development and licensure. This presentation discusses a few critical areas and challenges during the evolution of a CGT program.

1:00 LUNCHEON PRESENTATION: Accelerating Cell & Gene Therapy Development with Next-Generation Analytical Tools
Peter Johnson, Manager, Field Applications Scientist, Field Applications, Bio-Techne
Revolutionary cell and gene therapies offer significant promise to treat life-threatening diseases. However, getting these therapies to market quickly and efficiently is challenging. Rapid and accurate testing of critical quality attributes of viral vectors is necessary but can be impeded by old-school analytics like SDS-PAGE. Come learn how next-generation analytical solutions from Bio-Techne are designed to remove these analytical bottlenecks to get therapeutics to patients sooner.

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

ENSURING PRODUCT QUALITY

2:10 Chairperson's Remarks
Santoshkumar L. Khatwani, PhD, Director, Analytical Development, Sangamo Therapeutics

2:15 Characterization of Critical Raw Material Impact on rAAV Product Quality within the Context of a Transfection-Based HEK Production Process
Dainan Mao, PhD, Senior Scientist, Upstream Processing, Ultragenyx Pharmaceutical
Transient transfection using HEK293 is common for producing rAAV. During the process development supporting multiple Phase III products, upstream parameters and raw material were fully characterized using quality-by-design approach. Critical raw materials including polyethylenimine and plasmids were identified due to potential impact on productivity and quality. Comprehensive characterization including multiple DOEs has resulted in establishing control on these critical RMs to ensure consistent manufacturing of high-quality product.

2:45 Understanding the Impact of Manufacturing Process Changes on an Integrating AAV Vector Using In-Depth Analytical Characterization
Lauren M. Drouin, PhD, Director, Analytical Development, Genomic Medicine, Alexion, AstraZeneca Rare Disease
The manufacturing process for LB-001, an AAV gene editing vector designed to treat methylmalonic academia, was optimized to streamline the purification process and increase final product yield. An analytical comparability assessment was performed to evaluate the AAV gene editing material pre- and post-manufacturing process changes. Here we show that the process changes improved product quality and provide supporting analytical data.

ANALYTICS FOR FORMULATION

3:15 Formulation Activities from an Analytical Perspective
Jonathan Hill, Senior Scientist, Analytical Development, Solid Biosciences, Inc.
Formulation activities can range from a simple screen to confirm performance of an optimized matrix to a full-scale development of a new buffer system. A constant across the spectrum of formulation activities is the need for analytics to evaluate the effectiveness of the test matrices to confer stability while avoiding deleterious effects. This presentation will highlight available analytical techniques while exploring the effects of forced degradation and storage stability conditions.

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

LINKING PTMs TO STRUCTURE/FUNCTION

4:30 Mass Spectrometry for Post-Translational Modifications (PTM) Analysis
Yiling Bi, PhD, Senior Scientist, Sangamo Therapeutics
Post-translational modifications (PTMs) of biologics can occur during the production and storage stages. Due to the chemical modification changes, the potential impact on drug safety, quality, and efficacy needs to be closely monitored. In this presentation, we will focus on PTM analysis of AAV-based gene therapy drug products and in-process samples using mass spectrometry (MS) approach, and discuss the functional implications as well.

5:00 NGS Technology Application in Genomic Medicine Unit of Sanofi
Wei Zhang, PhD, Senior Scientist, Sanofi
A multi-attribute NGS assay for DNA identity and residual DNA contamination was developed using a PCR free library prep method. This method improved overall genome coverage depth, the accuracy of relative quantification of residual DNA, and variant calling confidence. This presentation will discuss the development of the method, provide case studies of use, as well as provide a high-level method validation strategy.

5:30 Close of Gene Therapy CMC and Analytics Conference

WEDNESDAY, AUGUST 16

7:30 am Registration and Morning Coffee

OPTIMIZING PROCESS DEVELOPMENT

7:55 Chairperson's Opening Remarks
Johannes C.M. Van Der Loo, PhD, Director Clinical Vector Core, Perelman Center for Cellular & Molecular Therapeutics, Children's Hospital of Philadelphia

8:00 KEYNOTE PRESENTATION End-to-End Development of a Suspension Transient Transfection Gene Therapy Production Platform
Terrence Dobrowsky, PhD, Head, Gene Therapy Drug Substance, Biogen
Biogen is developing a suspension transient transfection production system to support programs within our Gene Therapy pipeline from pre-clinical to commercial manufacturing. This presentation will review successful development strategies for establishing critical biological raw materials, upstream systems, and downstream processes, as well as their scale-up to reliably generate high volumes of drug substance.

8:30 The Latest Advancements in Adeno-Associated Virus (AAV) Production and Purification at 1000L
Nitin Garg, Senior Director, Gene Therapy, Tech Operations, PTC Therapeutics
Here, we report the optimization of rAAV production in suspension HEK293 cells in SFM media via triple transfection approach. Titers >2e11 VG/ml were observed at harvest. Furthermore, optimization enabled several AAV serotypes & transgenes to exhibit a 3-4 fold titer increase. A scalable enrichment process was established to achieve >50% full capsids at DS. Development (10L), pilot (200L), and production (1000L) were used to determine the process's scalability and robustness.

9:00 Multi-Factor Optimization and Modeling Approach to Improve rAAV Titer for Early Clinical-Stage Gene Therapy Programs
Shaoying Wang, PhD, Senior Scientist, Upstream Process Development, Passage Bio
In this study, multiple factors were optimized through a design of experiments (DoE) approach using flatware to improve the yield of early clinical-stage programs using HEK293 adherent cell. Conditions optimized in flatware were scaled into the iCellis Nano fixed bed bioreactor to test optimized conditions against a validated scale-down model. Further, the weighting of each factor in the model is compared across clinical trial programs with different serotypes.

9:30 Use of an Open, Automated Immunoassay Platform for Accelerated Analysis of Process Impurities and Process Contaminants
GYROS PROTEIN Technologies
Ellen Lee, PhD, Field Application Specialist, Sales, Gyros Protein Technologies
In process development and manufacturing, the need for accurate and efficient impurity and contaminant analysis is high. With the Gyrolab platform, process development, and manufacturing professionals gain a powerful tool to expedite impurity and contaminant analysis. The open format promotes flexibility and adaptability, allowing researchers and developers to customize and optimize assays based on their specific needs. The Gyrolab platform has enabled accelerated impurity and contaminant analysis in gene therapy workflows.

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

INCREASING TITERS, REDUCING TIMELINES

10:40 Comparison of Traditional and Novel AAV Production Processes
Matthew Roach, Associate Director, AAV Production, BridgeBio
There is an industry-wide need for improvement in upstream yield across adeno-associated virus production to meet eventual clinical and commercial need. Despite this, there is still a lack of information around the cellular processes and pathways involved in the manufacture of AAV. This presentation will describe our efforts to improve the yield of our upstream processes while simultaneously determining what cellular processes contribute to this increase in yield.

11:10 Process Development Strategies for Increasing the Genome Titer and Improving the Percentage of Full Capsids of AAV6
Bojiao Yin, PhD, Director, Vector Process Development & Manufacturing, ElevateBio
In this presentation, we will describe strategies applied in upstream and downstream process development to increase the vector genome (vg) titer and achieve high full particle enrichment of AAV6 particles using a two-chromatography step approach. With these improvements, the final AAV6 products showed higher concentration (>1E13 vg/ml) and better quality (70% full particle content) with reduced DNA and host cell protein (HCP) impurities.

11:40 Development and Optimization of a Modified-Batch Process for AAV Gene Therapy Using High-Throughput AMBR250-ATF Perfusion System
Wei Xue, PhD, Senior Scientist, Process Development, Ultragenyx Pharmaceutical
Accessibility and affordability remains one of the biggest challenges in AAV manufacturing, calling for a high-yielding, robust, scalable, and cost-efficient process. At Ultragenyx, we employ a HeLa producer cell/helper-virus based infection process for AAV production, which costs less and scales more readily than a transfection-based process. Furthermore, we developed a modified batch process with perfusion to boost productivity to > 5E11 GC/mL in up to 250L SUB.

12:10 pm LUNCHEON PRESENTATION: Standardizing AAV Downstream Processing
teknova:
Michel Cannieux, PhD, Senior Director of Product Management, Teknova
A lack of reagent and process standardization is holding back the development of gene therapies that can save lives. Here we will present three innovative technologies designed to advance AAV downstream processing: an AEX Buffer Screening Kit optimized for the critical parameters influencing full capsid enrichment (including pH, elution salts, excipients, surfactants, and stabilizers), a novel affinity buffer designed to maximize AAV capsid recovery post lysis, and an innovative reporter assay to monitor infectious capsids at any stage of purification.

12:40 Refreshment Break in the Exhibit Hall with Poster Viewing
ORFLO Personal Cell Analysis

INDUSTRIALIZING GENE THERAPIES, NEW APPROACHES

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

1:30 PANEL DISCUSSION: Industrializing Gene Therapies into Commercially-Viable Products

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

Panelists:

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

Stephen Soltys, PhD, Chief Manufacturing Officer, Primera Genotech

Nathalie Clément, PhD, Vice President, Vector Development, Translational Gene Therapies, Siren Biotechnology

Ashutosh Gupta, PhD, Former, Head, Vector Production, Takeda

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

2:30 Talk Title to be Announced

Speaker to be Announced

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

PLENARY KEYNOTE: LEADING TO TOMORROW'S ADVANCES

3:50 Chairperson's Remarks

Ran Zheng, CEO, Landmark Bio

4:00 Current and Future Trends in Biomanufacturing of New Modalities

Konstantin B. Konstantinov, PhD, CTO, Ring Therapeutics

Using exosomes as an example, this presentation examines the current and future trends in biomanufacturing, and the technologies needed to manufacture emerging modalities at scale. Traditional biomanufacturing methods do not provide the industrialized, commercially scalable, highly efficient and reproducible manufacturing process essential for this new class of biotherapeutics—so we built it from the ground up.

4:30 The Digitalization of Biomanufacturing

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

A testbed is described for the end-to-end integrated and continuous manufacturing of monoclonal antibodies, which consists of parallel bioreactors, simulated moving bed chromatography systems, viral inactivation, and an autosampling system. Experimental results are compared with a digital twin. The increased consistency in the glycosylation profile of the monoclonal antibodies being produced is quantified when going from batch to semi-batch to perfusion mode, and when moving from start-up to quasi-steady conditions.

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

6:00 Close of Day

THURSDAY, AUGUST 17

7:30 am Registration and Morning Coffee

LENTIVIRUS, AAV PROCESS DEVELOPMENT AND QUALITY

7:55 Chairperson's Remarks

Nathalie Clément, PhD, Vice President, Vector Development, Translational Gene Therapies, Siren Biotechnology

8:00 Overcoming the Challenges of Biomanufacturing Lentiviral Vector

Martin Loignon, PhD, Team Leader, Cell Engineering, National Research Council Canada

The demand for lentiviral vectors (LVs) for R&D and engineering cell therapies stems from their efficacy to deliver genes into targeted cells. Current LVs' production bioprocesses vary widely, significantly impacting quantities, quality, and costs. We have used a holistic approach to address challenges of upstream and downstream bioprocesses to increase titers and recovery.

8:30 Presentation to be Announced

Stanley Chung, PhD, Engineer II, Voyager Therapeutics

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

9:30 Evaluating Biopharma - Fireside Chats and Networking Session

Subject matter experts sit down 1:1 with our moderator to discuss and share their personal bioprocessing experiences, insights, and advice. The real "pay it forward" atmosphere provides biopharma leaders with unique opportunities to leverage and apply their expertise to make better technology, process, and business decisions, and, ultimately, to accelerate success. Dedicated networking within the session allows all attendees to follow up and dive deeper into conversation.

9:30 Identifying and Addressing Potency Method Challenges in Gene Therapy

Lyndi Rice, PhD, Head, Gene Therapy Analytical Technologies, BioMarin

9:30 Presentation to be Announced

Ben Locwin, Vice President, Project Solutions, Black Diamond

9:50 Tips to Improve Impurity Analysis of your Gene Therapy Product

Santoshkumar L. Khatwani, PhD, Director, Analytical Development, Sangamo Therapeutics

9:50 Presentation to be Announced

Ben Locwin, Vice President, Project Solutions, Black Diamond

10:10 Networking Session

Full room networking provides all attendees opportunity to engage with speakers and fellow attendees. Ask questions, discuss ideas, walk away with ideas to improve process success.

OPTIMIZING PROCESS DEVELOPMENT

10:30 Advances in Process Development

Nick DiGioia, Manager, Process Development, LogicBio Therapeutics, Inc.

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.

11:00 Scale-Up of Suspension 293T/17-Based Cells for GMP Manufacture of AAV

Bryan A. Piras, PhD, Director of Manufacturing, Children's GMP LLC, St. Jude Children's Research Hospital

This presentation will discuss scale-up of a suspension cell-based process for manufacture of AAV from 5 to 200 liters. Results, including cell growth, titers, and impurities, were consistent across 5 L and 200 L scales and will be presented along with several challenges related to scale-up and manufacture.

11:30 Real-Time Monitoring of Viral Vector Quality Attributes During Downstream Processing

Waters | WYATT

John Champagne, Ph.D., Senior Regional Manager, Wyatt Technology

Real-time multi-angle light scattering (RT-MALS) provides in-line and on-line monitoring of viral vector quality attributes for downstream processing. Depending on the type of virus, it can determine empty/full ratio, titer and aggregation, and differentiate between viruses and free proteins or nucleic acids. This talk will introduce RT-MALS technology with case studies demonstrating its applications for AAV, lentovirus and adenovirus purification including chromatography and UF/DF.

11:45 Viral Vector Process Development and Manufacture

BIOVIAN

Eero Mustalahti, Head of Business Development, Biovian

Viral Vector Process Development and Manufacture

12:00 pm LUNCHEON PRESENTATION: AAVone: An All-in-One Plasmid System Resolving AAV Manufacturing Bottlenecks

AAVnerGene

Daozhan Yu, PhD, CEO and President, AAVnerGene

Adeno-associated viruses (AAVs) are promising vectors for gene therapy, but production bottlenecks preventing wider adoption include low efficiency, variation, scalability, and high cost. AAVone combines three plasmids into one, streamlining production. In our experiments with AAV9-CMV-eGFP, AAVone achieved 4x increase over the triple transfection method. With enhanced efficiency, simplified protocol, scalability, and lower cost, the adoption of AAVone could be the key to accessible AAV gene therapy.

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

OPTIMIZING DOWNSTREAM PROCESSING FOR AAVs

1:05 Chairperson's Remarks

Meisam Bakhshayeshi, PhD, Senior Director and Head of CMC, Intergalactic Therapeutics

1:10 Advanced AEX Platform for AAV Enrichment

Yonatan Abune, Senior Engineer, Process Development, Myeloid Therapeutics

In this investigation, we employed AEX chromatography to simultaneously enrich full AAV capsid and eliminate endotoxin from the final drug product. By optimizing the column conditions, we achieved a high level of AAV capsid purity, as well as a significant reduction in endotoxin levels. This strategy offers a promising solution for the production of safe and effective AAV-based therapeutics.

1:40 Lessons Learned: A Case Study in the Downstream Optimization of an AAV5 Production Process

Ashton Lavoie, PhD, Associate Director, Downstream Process Development, BridgeBio Gene Therapy

Manufacturing strategies and progress towards platform processes for adeno-associated virus (AAV) production have seen substantial advancement in support of the impressive clinical success for this modality. This presentation will provide a case study for the development of a robust, high yield downstream process for the production of AAV serotype 5. Key findings will be discussed in addition to pitfalls and challenges in this development work.

2:10 Improved Host Cell Protein Reduction through Affinity Chromatography in Adeno-Associated Virus Purification Process

Wenjun Di, PhD, Scientist I, Ultragenyx Pharmaceutical

In recombinant adeno-associated virus (rAAV) purification process, host cell proteins (HCPs) were reduced in multiple unit operations to ensure sufficient clearance in drug substance. Here, we demonstrated that additives could be introduced in affinity chromatography step to further reduce HCPs while maintaining rAAV genome recovery.

2:40 Networking Refreshment Break

2:55 Altering the Adsorption Dynamics of Empty Capsids on Anion Exchangers for the Enrichment of Full rAAV Particles

Ronald Jenkins, PhD, Senior Director, Passage Bio

3:25 Studying AAV Capsid Aggregation in Complex Matrix of Clarified Lysate

Yulia Ivanova, PhD, Principal Scientist, Bioprocess R&D, Pfizer Inc.

Recovery out of harvest is the least understood step in downstream purification of AAV vectors. Complexity of lysed cell culture coupled to relatively low protein concentration of AAV product makes it very difficult to analytically investigate this process space. Here we evaluate the ability of dynamic light scattering (DLS) to serve as analytical characterization tool that would allow AAV capsid aggregation investigation in a complex matrix of clarified harvest.

3:55 Plasmid Purification Process Development for Gene Therapy Applications

Jacob C. Cardinal, Associate Scientist III, Biogen

This talk may cover topics including selection of appropriate purification methods, process optimization, and quality control measures to ensure high-quality plasmid DNA for gene therapy applications.

4:25 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. Topics include product and process characterization, CMC strategies, autologous and allogeneic manufacturing strategies, automation, scale-up, and supply of CAR Ts and next-generation cell therapies such as NK cells, TILs, iPSCs, and TCR-based therapies.

Conference Programs

AUGUST 14-15

Cell Therapy CMC and Analytics

View Program »

AUGUST 16-17

Cell Therapy Manufacturing

View Program »

MONDAY, AUGUST 14

8:00 am Registration and Morning Coffee

CMC STRATEGIES FOR CELL THERAPIES

9:55 Chairperson's Remarks
Patrick J. Hanley, PhD, Associate Professor, Pediatrics; Chief & Director, Cellular Therapy Program, Children's National Hospital

10:00 KEYNOTE PRESENTATION: Innovative CMC Strategies for Cell and Gene Therapies
Zhimei Du, PhD, Vice President, Translational Research and Early Development, LandMark Bio
Most cell and gene therapy (CGT) programs have their origins in academic research and have been developed in academic settings. However, this has resulted in challenges with establishing robust manufacturing controls and ensuring consistency in quality. Therefore, it is crucial to develop a new roadmap and strategy for CGT to enhance the speed of clinical development and improve the success rate of commercialization.

10:30 Raw Material Qualification for Cell Therapies
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.

11:00 Standards Development and Control Strategies for Cell Characterization and Cell Viability
Sumona Sarkar, PhD, Biomedical Engineer, Biosystems and Biomaterials Division, Biomaterials Group, National Institute of Standards and Technology
The manufacturing and release of cellular therapy products (CTPs) requires high quality, robust, and validated analytical methods. Here I will describe recent efforts in standards development and public-private partnerships to support the development of critical analytical methods used in advanced therapies. A key aspect of analytical development for these new class of products is the need for a fit-for-purpose approach. Efforts to establish fit-for purpose viability assays will be described.

11:30 Update on FDA Draft Guidance: Manufacturing Changes and Comparability for Human Cellular and Gene Therapy Products
Scott R. Burger, Principal, Advanced Cell & Gene Therapy LLC

12:00 pm LUNCHEON PRESENTATION: Enhancing Safety and Quality Assurance in Cell-Based Therapies and ATMPs: The Power of Rapid NAT-Based Mycoplasma Detection
ThermoFisher SCIENTIFIC
Kathleen Dunphy, Staff Scientist, Molecular Biology, Bioproduction Group, Thermo Fisher Scientific
Ensuring the safety and quality of ATMP or cell-based products is crucial for their successful translation into clinical applications. Mycoplasma contamination poses a significant risk to the safety of cell therapies, necessitating robust and rapid detection methods. Join us as we discuss the current regulatory expectations, as well as the principles, considerations, and performance of rapid, NAT-based mycoplasma detection methods.

12:30 Session Break

BUILDING QUALITY INTO CELL THERAPY DEVELOPMENT

12:50 Chairperson's Remarks
Mo Heidaran, PhD, Head, Translational and Regulatory Strategy, GC Therapeutics, Former FDA Reviewer

12:55 Building Quality into the CMC – Real World Perspectives from an Academic GMP Facility
Patrick J. Hanley, PhD, Associate Professor, Pediatrics; Chief & Director, Cellular Therapy Program, Children's National Hospital
In this presentation we will discuss real world examples of how to build quality into the CMC and how real world deviations and incidents have led to a better quality program. Examples will include improvements to change control, training, operations, and testing.

1:25 Quality Control Testing of Cell Therapies
Jay Tanna, MS, RAC, Quality Assurance Manager, Cellular Therapy Laboratory, Children's National Hospital

1:55 Key Considerations and Successful Strategies for Cell Therapy CMC Analytics
Center for Breakthrough Medicines
Maryam Rahimian, Principal Scientist, Process Development, Center for Breakthrough Medicines
Early phase cell therapy faces challenges in raw materials qualification, product/process characterization, and the lack of standardized analytical methods, potentially resulting in incorrect products and regulatory/commercialization delays.
Advanced therapies CDMOs aid developers in surmounting these hurdles through early analytical development using orthogonal methods. Witness how employing these methods for raw material qualification/characterization can efficiently reduce risk, enhance quality, and enable regulatory strategies during the IND filing.

2:25 Networking Refreshment Break

2:40 CMC Considerations to Accelerate Cell Therapy Development into Phase 1 Clinical Studies
Bruce S. Thompson, PhD, CEO, Kincell Bio
The development of cell therapies, such as engineered Chimeric Antigen Receptor (CAR) T cells has great promise for patients. As the field continues to better understand the biological outcomes and clinical science, many new therapies are being pursued targeting different potential antigens or cancer indications. In this presentation, we will discuss early development considerations that can set up sponsors for success as their programs mature into late-stage.

3:10 PANEL DISCUSSION: CMC Challenges for Genetically-Modified Cell Therapies
Moderator: Mo Heidaran, PhD, Head, Translational and Regulatory Strategy, GC Therapeutics, Former FDA Reviewer
Panelists:
Ravi Bhatia, Scientific Director, Cell Technology, Johnson & Johnson Pharmaceutical R&D
Zhimei Du, PhD, Vice President, Translational Research and Early Development, LandMark Bio
Ben Clarke, PhD, Senior Scientist, USP

3:40 Session Break and Transition to Plenary Keynote Session

PLENARY KEYNOTE: SOLVING TODAY'S CHALLENGES

4:20 Chairperson's Remarks
Susan D'Costa, PhD, CTO, Genezen

4:30 Overcoming the Challenges of Bioprocesses: The Future of Biomanufacturing
Glen R. Bolton, PhD, Executive Director, Late Stage Bioprocess Development, Amgen, Inc.
Novel therapies and technologies are emerging to meet the needs of patients; however, the manufacturing of biopharmaceuticals remains a complex and challenging process. As demand for biopharmaceuticals grows, the industry faces new challenges in terms of scalability, cost, and process robustness. The implementation of innovative technologies to improve process efficiency and the importance of process control and data analytics in ensuring process robustness are key levers to meet these challenges.

5:00 Commercializing Gene Therapies—The Combined Power of Patient Advocacy and Cost-Effective Manufacturing
Rachel Salzman, DVM, Founder, The Stop ALD Foundation; Global Head, Corporate Strategy, Armatus Bio
This presentation will examine the development of an FDA-approved gene therapy where patient advocacy played a critical role resulting in the first ever clinical use of a lentiviral vector. Although manufacturing continues to represent a significant challenge throughout the entire R&D journey, there are opportunities for advocacy and manufacturing communities to seek alignment and combine their collective powers to achieve the common goal of increasing patient access to transformative medicines.

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

6:30 Close of Day

TUESDAY, AUGUST 15

7:30 am Registration and Morning Coffee

TECH TRANSFER, MOVING FROM ACADEMIC TO COMMERCIAL

7:55 Chairperson's Remarks
Christopher Bravery, PhD, Consulting Regulatory Scientist, Advanced Biologicals Ltd.

8:00 Technology Transfer for Cell Therapies
Scott R. Burger, Principal, Advanced Cell & Gene Therapy LLC
We explore how an objective technology transfer approach should be applied for cell therapy products and provide detail of the mechanisms and tools which should be used in order to make sure that the transition from development to cGMP is correctly achieved.

8:30 Accelerating T Cell Therapy Product Development through a Joint Industry-Academia Collaboration
Therese Choquette, PhD, Director, Analytics, Tigen
Tigen Pharma and CHUV in Lausanne, Switzerland, have formed a unique and close collaboration in development of tumor-infiltrating lymphocytes for treatment of solid tumors. The deep scientific knowledge of the Coukos and Harari teams at CHUV and the industrial expertise in Tigen accelerates the path of product to patient. This talk presents examples of this collaboration for progress in manufacturing and analytics for TIL in future clinical trials.

9:00 Evolution from Bench to Bedside: The Integral Role of Analytical Development in Cell Therapy Product and Process Characterization
Bo Yan, PhD, Director, Analytical Research & Development, Beam Therapeutics
Base editing is a next-generation approach to gene editing with single base precision. I will review academic pioneering work on base editing based on existing publications. I will focus on analytical development, adding value to essential steps from bench to bedside, in my presentation. Two case studies will be discussed: potency assay development and how analytical assays contribute to cell therapy drug development using a quality-by-design (QbD) approach.

9:30 Overcoming Plasmid DNA Challenges to Drive Cell and Gene Therapy Success
Sarah Li, PhD, Associate Director of PD/MSAT, GenScript ProBio USA
Plasmid DNA serves essential roles as critical materials for cell and gene therapy applications. However, producing high-quality plasmid DNA at scale presents significant technical and regulatory hurdles. This presentation will provide insights into key considerations for plasmid DNA manufacturing for cell therapies, including emerging methods for improved yield, characterization and quality control. Case studies will showcase innovations across process optimization to accelerate plasmid manufacturing and development timelines.

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

10:45 Breakout Discussion Groups
Breakout discussions provide an opportunity to discuss a focused topic with peers from around the world in an open, collegial setting. Select from the list of topics available and join the moderated discussion to share ideas, gain insights, establish collaborations or commiserate about persistent challenges. Please visit the breakout discussions page on the conference website for a complete listing of topics and descriptions.

TABLE: Trends in CMC for Cell Therapies
Scott R. Burger, Principal, Advanced Cell & Gene Therapy LLC

TABLE: TABLE: Potency Assays for Cell Therapies
Rian de Laat, PhD, Associate Director, CMC, Voisin Consulting Life Sciences
- Importance of the cell therapy potency assay
- Potency assay development challenges
- Bridging the gap between R&D and manufacturing
- The evolving regulatory landscape of potency assays??

LENTIVIRAL QUALITY AND ANALYSIS

11:30 New Programs, New Opportunities; Utilizing a Phase II Trial to Rethink Potency Workflow

Eric Bolf, PhD, Scientist, Analytical Development, 2SeventyBio

As we have gained experience with analytical methods, we have learned more about the pain-points associated with our assays. With renewed focus on efficiently bringing our pipeline into the clinic, we have taken this opportunity to redevelop how we measure lentiviral vector potency. This talk will discuss our new methodology, including approaches to improve assay throughput, improve consistency of the readouts, and reduce assay complexity.

12:00 pm Optimization of Nuclease Digestion in a Lentiviral Vector Process for Improved Reduction of DNA Impurities

James Xin, Scientist, Vector Process Development, ElevateBio

In the C&GT field, process-related DNA impurity levels are a safety focal point. Here, we provide the statistical analysis results based on several DoE studies or reduction of hcDNA and pDNA levels in our Lentiviral Vector (LVV) platform process. Focusing on several critical parameters including nuclease concentration, supplement concentration, incubation time, and incubation conditions (ph/temperature), this data shows the optimal factors and ranges in the nuclease digestion step.

12:30 Freezing Point Osmolality for mRNA-LNP Stability ADVANCED INSTRUMENTS

Emily Hyatt, Field Application Scientist, Advanced Instruments

- Formulation of LNPs using High Pressure Homogenizer and Probe Sonication Method
- Characterizations of formulations i.e. Osmolality, Size, Zeta Potential, PDI, DSC, Drug entrapment efficiency, Stability
- Statistical optimization of LNPs using osmolality centered approach

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

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

FLOW CYTOMETRY AND CHARACTERIZING IPSCs

2:10 Chairperson's Remarks

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

2:15 Sensitivity, Precision, and Misconceptions when Flow Cytometry Is Not Performed by You

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

Rare cell analysis on large numbers of cells is important in characterization of cell products as unwanted effects can be caused by just a few cells. Conventional flow cytometers are designed to accommodate assay development, allowing operators to fit instrument settings to their specific needs. The 'open' design of this instrument brings a significant level of complexity to flow cytometry and challenges reproducibility in manufacturing processes of therapeutic cells.

2:45 Moving toward Meaningful Characterization of iPSCs by Live Imaging to Accurately Monitor Cell Behavior

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

Live-cell imaging can provide quantitative dynamic and spatial characteristics of iPSCs in culture. Our research is geared toward developing systematic workflows quantifying the effects of culture and other handling conditions on iPS cell characteristics such as rates of mitosis, changes in morphology, and expression of relevant transcription factors to predict and evaluate the effects of manufacturing conditions and other perturbations on the state of the population.

3:15 Characterizing CARs from the Cell Surface Using Immunoprecipitation-Mass Spectrometry

Nicolle Serrano SantoDomingo, Research Scientist II, Novartis

CAR T are engineered T cells expressing a chimeric antigen receptor (CAR) on the surface. CARs allow T cells to engage antigens on tumor cells and activate downstream signaling, leading to tumor cell destruction. Surface expression of the CAR is critical for efficacy, but differences in efficacy are observed between constructs showing similar surface expression. We developed a workflow to characterize post-translational modifications by LC-MS to better understand efficacy differences.

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

ADVANCED ANALYTICS

4:30 Cell Therapy Analytical Toolkit

Victor Muthu, PhD, Principal Scientist, Analytical Development, National Resilience

My presentation will highlight the most recent analytical development methods in the cell therapy field and how our team at Resilience is pushing the boundaries of developing the cell therapy analytical toolbox by utilizing advanced platforms for assay development and qualification.

5:00 Sterility Control for Cell Therapies from a Regulatory Perspective

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

The nature of cell therapy products means they cannot be terminally sterilised or even sterile-filtered; this puts a greater onus on other aspects of sterility control. This talk will discuss how a holistic approach is needed to ensure safety of these products, including facilities, testing and methods of sterilisation.

5:30 Close of Cell Therapy CMC and Analytics Conference

WEDNESDAY, AUGUST 16

7:30 am Registration and Morning Coffee

MANUFACTURING CELL THERAPIES BEYOND CAR Ts

7:55 Chairperson's Opening Remarks
Dominic Clarke, CSO, Orange County Bio and ISCT Committee Chair

8:00 KEYNOTE PRESENTATION: Manufacturing Gamma Deltas
Kate M. Rochlin, PhD, COO, IN8bio, Inc.
IN8bio is a clinical-stage gamma-delta T cell therapy company with two clinical programs in Phase I and one in Phase II, in both solid and hematological tumors. Gamma-delta T cells are part of the innate immune system with the ability to recognize and kill malignant cells and our clinical manufacturing experience has shown that evaluation of the CQA and cellular effector memory profile may help predict expansion and potency.

8:30 FEATURED PRESENTATION: The (Re)emerging Field of Xenotransplantation
Knut Niss, PhD, CTO, eGenesis, Inc.
Through our transformative research, we are developing HuCo organs and cells to meet the increasing need. Our eGenesis Genome Engineering and Production (EGEN) platform leverages advances in gene editing technologies to address the historical challenges of xenotransplantation.

9:00 Optimization of Autologous TCR T Cell Manufacturing Process by Managing the Heterogeneity of the Starting Apheresis Material
Gagan Bajwa, PhD, Senior Scientist, Process Development, Immatics
This presentation will discuss: Heterogeneity of the starting leukapheresis poses challenges for successful manufacturing of autologous TCR T cells; cellular composition of the starting material impacts product characteristics; and optimization of starting material is critical to achieve adequate quantity and quality of the TCR T cell product.

9:30 A Mathematical Approach to Bridge Metabolic Parameters to CAR T Cell Growth in Limited Data Conditions
Keshav Patil, PhD, Scientist, Advanced Therapies, Janssen Pharmaceuticals Inc.

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

SPECIAL ISCT SESSION – PAT, PROCESS CONTROL, AND SCALE-UP

10:40 Cell Therapy Process Development and Manufacturing
Jerry Eriksson, MSc, Senior Research Scientist, AstraZeneca R&D

11:10 Process Analytical Technologies for Process Control Strategy Development of Cell & Gene Therapy Products
John Churchwell, PhD, Associate Lead Scientist, Cell & Gene Therapy Catapult
Advanced control strategies using PAT technologies have the potential to increase product consistency, reduce process variability and increase potential for process automation during the production of cell and gene therapy products. This presentation will discuss examples of real-time monitoring strategies and how an automated PAT lab set up can be utilized for improved process characterization and future manufacturing automation.

11:40 Decentralized Manufacturing
Scott R. Burger, Principal, Advanced Cell & Gene Therapy LLC
This presentation provides an overview of centralized and decentralized manufacturing models, pros/cons and suitable applications, approaches to overcoming challenges of decentralized manufacturing, and regulatory considerations.

12:10 pm Sponsored Presentation *(Opportunity Available)*

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

SPECIAL ISCT SESSION – PAT, PROCESS CONTROL, AND SCALE-UP (CONT.)

1:25 Chairperson's Remarks
Dominic Clarke, CSO, Orange County Bio and ISCT Committee Chair

1:30 Be More Closed-Minded
Ian D. Gaudet, PhD, ISCT Process & Product Committee Member, and Senior Director and Site Head, Process Sciences, Miltenyi Biotec, Inc.
Autologous cell therapy manufacturing costs still limit bringing these impactful medicines to more patients. Open process manipulations requiring expensive engineering controls are a significant driver of the overall manufacturing costs, including significant expenses in labor, equipment, materials, cleaning, validation, and facility footprint. Progress towards fully-closed system design suitable for commercial scale manufacturing in both centralized and point-of-care settings are discussed.

2:00 PANEL DISCUSSION: ISCT SESSION – Process Analytics, Automation, and Digitalization
Moderator: Dominic Clarke, CSO, Orange County Bio and ISCT Committee Chair
Panelists:
Jerry Eriksson, MSc, Senior Research Scientist, AstraZeneca R&D
Ian D. Gaudet, PhD, ISCT Process & Product Committee Member, and Senior Director and Site Head, Process Sciences, Miltenyi Biotec, Inc.
John Churchwell, PhD, Associate Lead Scientist, Cell & Gene Therapy Catapult

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

PLENARY KEYNOTE: LEADING TO TOMORROW'S ADVANCES

3:50 Chairperson's Remarks
Ran Zheng, CEO, Landmark Bio

4:00 Current and Future Trends in Biomanufacturing of New Modalities
Konstantin B. Konstantinov, PhD, CTO, Ring Therapeutics
Using exosomes as an example, this presentation examines the current and future trends in biomanufacturing, and the technologies needed to manufacture emerging modalities at scale.

Traditional biomanufacturing methods do not provide the industrialized, commercially scalable, highly efficient and reproducible manufacturing process essential for this new class of biotherapeutics—so we built it from the ground up.

4:30 The Digitalization of Biomanufacturing
Richard D. Braatz, PhD, Edwin R. Gilliland Professor, Chemical Engineering, Massachusetts Institute of Technology
A testbed is described for the end-to-end integrated and continuous manufacturing of monoclonal antibodies, which consists of parallel bioreactors, simulated moving bed chromatography systems, viral inactivation, and an autosampling system. Experimental results are compared with a digital twin. The increased consistency in the glycosylation profile of the monoclonal antibodies being produced is quantified when going from batch to semi-batch to perfusion mode, and when moving from start-up to quasi-steady conditions.

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

Bionova Scientific

6:00 Close of Day

THURSDAY, AUGUST 17

7:30 am Registration and Morning Coffee

LENTIVIRUS, AAV PROCESS DEVELOPMENT AND QUALITY

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

8:00 Overcoming the Challenges of Biomanufacturing Lentiviral Vector
Martin Loignon, PhD, Team Leader, Cell Engineering, National Research Council Canada
The demand for lentiviral vectors (LVs) for R&D and engineering cell therapies stems from their efficacy to deliver genes into targeted cells. Current LVs' production bioprocesses vary widely, significantly impacting quantities, quality, and costs. We have used a holistic approach to address challenges of upstream and downstream bioprocesses to increase titers and recovery.

8:30 Presentation to be Announced
Stanley Chung, PhD, Engineer II, Voyager Therapeutics

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

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9:30 Breakout Discussion Groups
Breakout discussions provide an opportunity to discuss a focused topic with peers from around the world in an open, collegial setting. Select from the list of topics available and join the moderated discussion to share ideas, gain insights, establish collaborations or commiserate about persistent challenges. Please visit the breakout discussions page on the conference website for a complete listing of topics and descriptions.

IN-PERSON ONLY BREAKOUT: Manufacturing Cell and Gene Therapies
Michael D. Jacobson, PhD, Managing Partner, Cambridge Biostrategy Associates LLC

MANUFACTURING IPSCs

10:30 Scalable Production of Pluripotent Stem Cell-Derived Hematopoietic Progenitor Cells and Functional T Cells in Stirred Tank Bioreactors
Liz Csaszar, PhD, Senior Director, Manufacturing Sciences, Tech Operations, Notch Therapeutics
Stirred suspension-based cell manufacturing can be used for scalable and controllable production of cell therapy products. We have developed custom reagents to modulate Notch signaling, which are compatible with suspension culture, and have implemented the production of pluripotent stem cell-derived CD8aß+ T cells in stirred tank bioreactors (STRs). STR-based culture enables process optimization and characterization using bioprocess solutions including automated feeding and in-process monitoring.

11:00 Engineering & Manufacturing iPSC-Derived Innate Cells to Provide Globally Scalable, Allogeneic Innate Therapies
Allen Qiang Feng, PhD, Founder and CSO, HebeCell Corp.
Human pluripotent stem cell (PSC)-derived natural killer (NK) cells combine the advantages of PSC and the safety profile of NK cells. At HebeCell we have developed our proprietary technology platform that is bioprocessing friendly and adaptable to GMP standards. Our feeder-free platform utilizes 3D spheroids to mimic the *in vivo* hematopoiesis to generate cytotoxic protoNK cells in bioreactors. Our platform offers a highly scalable approach for off-the-shelf cell therapies.

11:30 Scalable Production of Induced Pluripotent Stem Cell-Derived CD8+ CAR-T Cells in Stirred Tank Reactors Using DLL4/VCAM-1 Coated Beads
Vaisakh Rajan, Scientist II Cell & Molecular Biology, Manufacturing Sciences, Notch Therapeutics

11:30 Exploring the Production Potential and Mechanoresponsiveness of Induced Pluripotent Stem Cell-Derived Mesenchymal Stem/Stromal Cells for Extracellular Vesicle Biomanufacturing
Emily Powsner, Graduate Research Asst, Bioengineering, Univ of Maryland College Park

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

OPTIMIZING CELL THERAPY MANUFACTURING

1:05 Chairperson's Remarks
Scott R. Burger, Principal, Advanced Cell & Gene Therapy LLC

1:10 Streamlined Expansion of PBNK and CD147-CAR-NK Cells in the Grex-100M Closed System: Achieving Scalability and Efficiency
Xuening Wang, PhD, Senior Research Associate, Rutgers-New Jersey Medical School
NK and CAR-NK cells are potent immune cells with strong potential in targeted cancer therapy. Reliable and practical approaches are required for large-scale production. The G-Rex 100M bioreactor supports the expansion of both PBNK and CAR-NK cells, exhibiting high cytotoxicity against HCC cells. Cryopreservation minimally impacts the cytotoxicity of NK cells. Our method provides an effective platform for scalable NK and CAR-NK production, facilitating clinical use of "off-the-shelf" NK immunotherapy.

1:40 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*.

2:10 Encapsulated Cell Therapy: An Off-the-Shelf, Scalable Approach to Treating Solid Tumors

Lauren E. Jansen, PhD, Director, Process Development, Avenge Bio

Avenge Bio's LOCOcyte platform consists of polymer encapsulated, allogeneic cells genetically engineered to produce immunomodulatory molecules for the treatment of previously intractable cancers. This presentation will highlight our approach to a successful technology transfer of our lead IL-2 program, AVB-001, into Phase I GMP manufacturing. In addition, we will discuss key considerations for future development and scale-up of this innovative cell therapy.

2:40 Networking Refreshment Break

DIGITAL INTEGRATION, DECENTRALIZED MANUFACTURING

2:55 The Application of Digital Informatics Methods to Manage Development and Production Data for Cell and Gene Therapies

William E. Janssen, PhD, Principal, WEJ Cell & Gene Therapy Consulting Services LLC

Explosive growth in the fields of informatics and cell and gene therapy (CGT) has occurred over the last four decades. Application of Informatics tools in CGT manufacturing can facilitate prospective quality management, and can reduce errors in manufacturing, release, distribution, and administration. An informatics backbone will be essential for deployment of disseminated manufacturing. Implementation of informatics in CGT requires substantial planning, resources, and collaboration between CGT and informatics teams.

3:25 Could Your Cell Therapy Manufacturing Facility Be Working Harder for You?

Peter Walters, Fellow of Advanced Therapies, CRB

Optimizing cell therapy facilities is challenging due to small-scale batch sizes. With batch sizes as small as one patient, manufacturers must scale out rather than up, duplicating every inefficiency in the process. This presentation explores four manufacturing design approaches for small-scale cell therapies. Each approach has challenges, and manufacturers must balance risk tolerance, capital spending, and growth expectations to achieve manufacturing goals. Case studies comparing efficient designs will be compared.

3:55 Considerations in Development of Lentiviral-Based *in vivo* Gene Therapy

Mukesh Mayani, PhD, Head of Process Development, Gene Therapy, National Resilience, Inc.

Lentiviral vectors (LVV) have demonstrated noteworthy clinical success during *ex vivo* CAR T and HSC GT applications. The third-gen SIN LVVs have shown improved safety profiles for a conceivable durable treatment of rare disease and cancer indications during clinics. However, their *in vivo* use is severely limited due to manufacturing, safety, and quality challenges. This presentation will highlight development and manufacturing considerations for generation of LV suitable for *in vivo* applications.

4:25 Close of Summit

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STREAM #5
ANALYTICAL & QUALITY

The Summit's 2023 Analytical & Quality stream offers in-depth updates on critical steps in biopharmaceutical development that impact product quality, safety, and regulatory compliance. Separate two-day meetings in this stream will focus on the detection, analysis and removal of host cell proteins and the acceleration of analytical development steps and timelines. Over thirty in-depth presentations will give attendees insight into the best practices being applied across large and small industry R&D groups.

Conference Programs

AUGUST 14-15

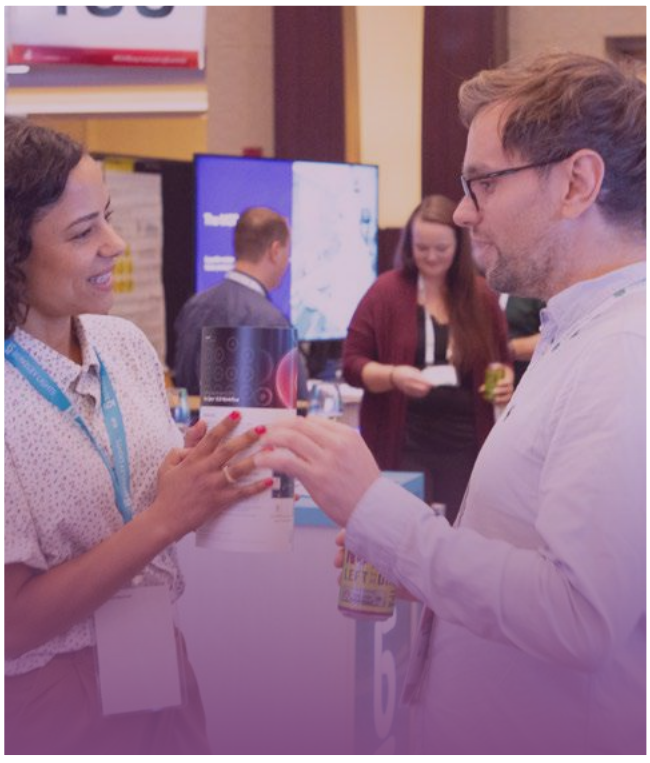
Host Cell Proteins

View Program »

AUGUST 16-17

Accelerating Analytical Development

View Program »



MONDAY, AUGUST 14

8:00 am Registration and Morning Coffee

9:55 Chairperson's Opening Remarks
Nisha Palackal, PhD, Director, Protein Biochemistry, Regeneron Pharmaceuticals, Inc.

10:00 FEATURED PRESENTATION: A Journey through the Evolution of HCP Detection Methods: From Early Commercial ELISA Kits to Specific Assays
Nisha Palackal, PhD, Director, Protein Biochemistry, Regeneron Pharmaceuticals, Inc.
This presentation will cover the evolution of HCP assays from generic to process-specific to platform, highlighting the importance of 2D gels and 2D westerns in establishing coverage. The advent of MS technologies has significantly changed HCP detection, and problematic host cell proteins have led to the implementation of individual HCP assays for process control. Additionally, new technologies are being utilized for detecting and quantifying HCPs, leading to more efficiency.

NEW LC-MS APPLICATIONS AND TECHNOLOGIES

10:30 Host Cell Protein Analysis of AAV-Based Gene Therapy Products by LC-MS
Yue (Emma) Zhang, PhD, Scientist, Analytical Development, Biogen
LC-MS has become an increasingly valuable analytical approach in the analysis of host cell protein (HCP) for biotherapeutic products. There are unique challenges in the application of LC-MS for HCP analysis of new modalities, including adeno-associated virus (AAV) products. The presentation discusses how to leverage our existing knowledge and expertise from biologics using LC-MS to overcome these challenges and generate comprehensive HCP profiles for AAV-based gene therapy products.

11:00 HCP Assays and GMP Release Testing with LC-MS for Vaccines and Advanced Cell & Gene Therapies
Thomas Kofoed, PhD, Co-Founder & CEO, Alphalyse, Denmark
Regulatory authorities are increasingly requesting orthogonal LC-MS data for residual protein characterization and documentation. Often, available ELISAs do not have sufficient HCP coverage for the new manufacturing process and complex product. The presentation will present and discuss experiences from using quantitative LC-MS analysis on more than 300 projects, with case examples from vaccines, viral vector therapies, method validation, and GMP release testing.

11:30 Enjoy Lunch on Your Own

12:30 pm Session Break

PRODUCT QUALITY AND IN-PROCESS CONTROLS

12:50 Chairperson's Remarks
Eric Bishop, Vice President, R&D, Cygnus Technologies

12:55 Formation of Transient Highly-Charged mAb Clusters Resulted in Poor Clearance of Host Cell Proteins during Downstream Processing
Haibin Luo, PhD, Associate Director, AstraZeneca
Protein A chromatography with a high salt wash usually leads to robust clearance of host cell proteins (HCPs) in most recombinant monoclonal antibodies (mAbs), but a small subset of recalcitrant mAbs still show significant HCP copurification. Based on our investigation of 30-ish mAb molecules, we proposed a novel mechanism for HCP copurification; i.e., mAb-clustering strengthens interactions between mAb with HCPs. Breaking mAb-clusters effectively prevents HCP copurification.

1:25 Managing HCP Impurities during Development and Lifecycle
Erika M. Friedl, PhD, Quality Expert, Haematology & Transfusion Medicine, Paul Ehrlich Institute, Germany
For production of high-quality medicines, efficient removal and control of impurities during product development and lifecycle is mandatory. As critical quality attributes, HCP impurities are covered by specific guidelines. It is often challenging meeting the regulatory expectations regarding HCP removal, characterization, and control throughout the product lifecycle. Therefore, HCP case studies will be presented to highlight and mitigate the hurdles affecting the quality, efficacy, and safety of medicinal products.

1:55 HEK293 Total Host Cell Protein ELISA Development to Support AAV Gene Therapy Programs
Jianming Kang, PhD, Senior Scientist, Regeneron
Host cell proteins (HCPs) are process-related impurities monitored during expression of biotherapeutics. HCPs must be well-characterized, monitored, and reported. The most widely used method for monitoring HCP is an enzyme-linked immunosorbent assay (ELISA), due to its high-throughput, sensitivity, and selectivity. We report the development of a robust HEK293 total HCP ELISA which demonstrates superior sensitivity and precision for process clearance and DS samples over all existing assays.

2:25 Networking Refreshment Break

2:40 Characterization of Polysorbate Degrading Enzymes in Biopharmaceuticals by a Novel, High-Throughput Assay
Sanjay Gupta, PhD, Scientist, Analytical Development, Roche, Germany
Problematic HCPs in extremely low quantities in biological drug products poses a major challenge towards their identification and characterization. We developed a highly sensitive, fast and high-throughput method to monitor the presence of hydrolytic activities in samples generated during bioprocessing. By utilizing a custom-designed surrogate substrate combined with a well-established and robust detection platform, the method provides a rapid electrochemiluminescence-based readout of the hydrolytic impurity status in a given sample.

3:10 Presentation to be Announced

3:40 Session Break and Transition to Plenary Keynote Session

PLENARY KEYNOTE: SOLVING TODAY'S CHALLENGES

4:20 Chairperson's Remarks
Susan D'Costa, PhD, CTO, Genezen

4:30 Overcoming the Challenges of Bioprocesses: The Future of Biomanufacturing
Glen R. Bolton, PhD, Executive Director, Late Stage Bioprocess Development, Amgen, Inc.
Novel therapies and technologies are emerging to meet the needs of patients; however, the manufacturing of biopharmaceuticals remains a complex and challenging process. As demand for biopharmaceuticals grows, the industry faces new challenges in terms of scalability, cost, and process robustness. The implementation of innovative technologies to improve process efficiency and the importance of process control and data analytics in ensuring process robustness are key levers to meet these challenges.

5:00 Commercializing Gene Therapies—The Combined Power of Patient Advocacy and Cost-Effective Manufacturing

Rachel Salzman, DVM, Founder, The Stop ALD Foundation; Global Head, Corporate Strategy, Armatus Bio

This presentation will examine the development of an FDA-approved gene therapy where patient advocacy played a critical role resulting in the first ever clinical use of a lentiviral vector. Although manufacturing continues to represent a significant challenge throughout the entire R&D journey, there are opportunities for advocacy and manufacturing communities to seek alignment and combine their collective powers to achieve the common goal of increasing patient access to transformative medicines.

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

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6:30 Close of Day

TUESDAY, AUGUST 15

7:30 am Registration and Morning Coffee

EMERGING METHODS AND TECHNOLOGIES

7:55 Chairperson's Remarks

Georgeen Gaza-Bulseco, Principal Research Scientist, AbbVie

8:00 KEYNOTE PRESENTATION: Standard-Free Absolute Quantitation of Antibody Deamidation Degradation and Host Cell Proteins by Coulometric Mass Spectrometry

Hao Chen, PhD, Professor, Chemistry and Environmental Science, New Jersey Institute of Technology

Recently we developed a coulometric mass spectrometry (CMS) approach for absolute quantitation of proteins without the use of standards, based on the electrochemical peptide oxidation followed by MS measurement of the oxidation yield. CMS can be used for absolute quantitation of a low-level target protein in a mixture; for instance, 500 ppm of PLBL2, a problematic host cell protein (HCP), in the presence of mAb was successfully quantified by CMS.

8:30 Population Balance Modelling Captures Host Cell Protein Dynamics in CHO Cell Cultures

Sakhr Alhuthali, PhD, Honorary Research Fellow, Chemical Engineering, Imperial College London, United Kingdom

Increases in mAb titre has been achieved mainly by cell culture feed improvement and cell line engineering to increase cell density and specific mAb productivity. This has caused a higher host cell proteins (HCPs) in the supernatant. Herein, a population balance model (PBM) has been built to capture Chinese hamster ovary (CHO) cell behaviour in bioreactors to predict HCP dynamics. The mathematical model increases cost-efficiency while minimising impurity levels.

9:00 USP Standards to Support HCP Analysis by Mass Spectrometry

Anthony Blaszczyk, PhD, Senior Scientist, Global Biologics, US Pharmacopeia

Residual HCPs in biopharmaceuticals can impact product quality and safety. As mass spectrometry has become a common approach for identification and quantitation of specific HCPs, stakeholders identified a need for standards and tools to support consistency of HCP measurements. This presentation will summarize the proposed general chapter <1132.1> *Residual Host Cell Protein Measurement in Biopharmaceuticals by Mass Spectrometry* and provide an update on reference materials to support identification and quantitation.

9:30 CASE STUDIES USING LC-MS TO MITIGATE PROBLEMATIC HCPS

CYGNUS TECHNOLOGIES part of Maravai LifeSciences

Jared Isaac, Associate Director Chromatography and Mass Spectrometry, R&D, Cygnus Technologies

Mass Spectrometry (MS) is an orthogonal technique to ELISA. ELISA is a well-established method for lot release testing, and MS supports ELISA during early-stage drug substance (DS) purification, DS batch testing, and when transferring biomanufacturing process between CDMOs. During downstream process development (DSP) and manufacturing scale up problematic HCPs may be enriched to above specification levels. MS can be used to perform antibody coverage analysis to ensure the QC ELISA is not missing HCPs and identify potentially problematic HCPs. We will present several case studies of problematic HCPs that were not cleared during the DSP. AAE-MS was applied to show that the ELISA was appropriately monitoring the DSP and used to identify problematic HCPs.

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

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10:45 Breakout Discussion Groups

Breakout discussions provide an opportunity to discuss a focused topic with peers from around the world in an open, collegial setting. Select from the list of topics available and join the moderated discussion to share ideas, gain insights, establish collaborations or commiserate about persistent challenges. Please visit the breakout discussions page on the conference website for a complete listing of topics and descriptions.

IN-PERSON ONLY BREAKOUT: HCP Detection, Analysis and Control

Rosalind Ang, PhD, Associate Principal Scientist, Merck

IN-PERSON ONLY BREAKOUT: Use of LC-MS as a Release Method for HCP

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

11:30 Analysis of Biologically-Relevant Concentrations of Therapeutic Host Cell Proteins through an Ultrasensitive Quantification Method Coupling Limited Digestion to ProteoMiner Technology

Hui Xiao, PhD, Senior Principal Scientist, Regeneron Pharmaceuticals, Inc.

A new method is developed to quantify HCPs at sub-ppm levels with ProteoMiner enrichment, coupled with limited digestion, followed by targeted analysis with nano liquid chromatography-parallel reaction monitoring. The method can achieve LLOQ values as low as 0.06 ppm, with an accuracy of 85%–111% of the theoretical value, and inter-run and intra-run precision within 12% and 25%, respectively.

12:00 pm Development of a Custom Automated Method for HCP Detection in Gene Therapy Products

Matthew J. Lotti, Senior Research Associate II, Ultragenyx Pharmaceutical, Inc.

During drug manufacture, large volumes of samples are submitted for host cell protein (HCP) quantitation to assess purification efficiency and ensure patient safety. Therefore, it's beneficial to have methods with enhanced throughput. Here, we describe development of a custom HCP method that

incorporates automated sample preparation with automated immunoassay and data analysis, resulting in a higher throughput assay that produces high-quality data needed to support gene therapy product development.

12:30 **Enjoy Lunch on Your Own**

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

PROBLEMS & SOLUTIONS

2:10 **Chairperson's Remarks**
Rachel Chen, PhD, Associate Director, Analytical Development, Biogen

2:15 **Case Studies of HCP Analytical Method Bridging and Phase-Appropriate Validation**
Rosalind Ang, PhD, Associate Principal Scientist, Merck
Host cell proteins must be removed to acceptable low levels due to their potential impact on product quality, safety, and efficacy. The suitability of the analytical method (i.e., stringency) used for its detection and quantitation is related to the development cycle of the biologics. The bridging and validation strategy used to support HCP analytical method change in late-phase development, to meet an accelerated regulatory filing timeline, will be discussed.

2:45 **Choosing the Right Method: LC-MS/MS Based HCP Workflows and Case Studies for Process-Specific Method Development**
Jia Guo, PhD, Principal Scientist, Analytical Development & Quality Control, Genentech, Inc.
The presentation shows our strategy for LC-MS/MS based HCP characterization workflows to enable fast support of process-specific development. Three case studies will be discussed, including 1) supporting downstream process development using a robust high-load LC-MS/MS method, 2) identification of low-level polysorbate degradation enzymes in the final purification pools, and 3) using quantitative MS methods to support process-specific method development for PRDX1 clearance, when the specific ELISA is not available.

3:15 **Increase the Depth and Breadth of Host Cell Protein Analysis through Analytical Method Innovation and Digital Tools**
Rachel Chen, PhD, Associate Director, Analytical Development, Biogen
Host cell proteins (HCPs) are one of the process-related impurities that may cause issues with the safety and stability of biotherapeutics. This presentation will cover recent analytical method innovations in sample preparation and mass spectrometry detection, to achieve in-depth characterization for low abundant HCPs. In addition, comprehensive HCP profiling by LC-MS coupled with proteomics analysis could help understand the HCP abundance change and its impact on various biomanufacturing processes.

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

4:30 **Methods Used to Identify, Quantitate, and Monitor High-Risk Host Cell Proteins**
Georgeen Gaza-Bulseco, Principal Research Scientist, AbbVie
Host cell proteins (HCPs) are process-related impurities that have the potential to impact patient safety and product efficacy. Even with state-of-the-art downstream processing to remove HCPs, some make their way through the process and copurify with the biopharmaceutical product. High-risk HCPs that make it through the process should be evaluated. Methods used to detect, quantitate, and monitor high risk HCPs will be presented along with several case studies.

5:00 **Small Company Perspectives on CRO/CMO Support for HCP Control**
Seth Levy, PhD, Director, Bioprocess Development, Modalis Therapeutics
Modalis delivers its CRISPR Guide Nucleotide Directed Modulation (CRISPR-GNDM) therapy via a single AAV vector. Cells used in the production of recombinant AAV contain host cell proteins (HCPs) that can contaminate drug products. Regulatory bodies expect these impurities be reduced to the lowest levels possible. Modalis utilizes an automated workflow evaluating HCP reduction across clarification, concentration, and purification steps, and ensures HCP levels at CDMOs align with internally developed processes.

5:30 **Close of Host Cell Proteins Conference**

WEDNESDAY, AUGUST 16

7:30 am Registration and Morning Coffee

7:55 Chairperson's Opening Remarks
Xue (Shelly) Li, Associate Director, Biologics Development, Bristol Myers Squibb Co.

8:00 KEYNOTE PRESENTATION: How to Align the Definition of "Compendial Method" with Speed of Innovation?
Elena A. Smith, PhD, Analytical CMC Leader – Vaccine, Sanofi
Novel manufacturing platforms may have regulatory challenges for the analytical strategy as often regulations and guidance are developed and optimized for traditional platforms. The speed for the development of next-generation technologies is significantly faster than the updating compendial methodology in guidance and regulations. Current talk will challenge the definition of "compendial method" in modern-day environment in order to pave the way to propel the innovation implementation.

PLATFORMS & WORKFLOWS FOR NOVEL MODALITIES

8:30 Dual-Detection Approach for Charge Variant Analysis of Monoclonal Antibody Combination Products Using Imaged Capillary Isoelectric Focusing
Xue (Shelly) Li, Associate Director, Biologics Development, Bristol Myers Squibb Co.
We report a novel methodology to accurately quantify charge variants of monoclonal antibody mixtures that span 40-fold in ratio. With the wide concentration ranges of combination products, one component may fall within the linear range while the other does not. Imaged capillary isoelectric focusing conjugated with the multiple detection techniques allowed us to overcome this challenge.

9:00 In-Depth Characterization of High Molecular Weight (HMW) Formation in Alternative Format (AF) Drug Molecules
Timothy-Neil Tiambeng, PhD, Scientist, Regeneron Pharmaceuticals
Alternative-format biopharmaceuticals which feature domains linked by (Gly4Ser)n linkers provide new therapeutic opportunities through potentially enhanced binding properties. However, characterization of the intermolecular domain interactions that give rise to oligomeric species remains challenging, due to the variety of possible interaction interfaces at the subdomain level. In this study, we present an online, middle-up LC-MS-based method to characterize covalent and non-covalent subdomain interactions in alternative format drug substances.

9:30 Integration of Benchtop NMR as a PAT Tool for Optimizing Bioprocess Monitoring and Control
Gabriella Gerzon, PhD Candidate, York University, Toronto, Canada
Matteo Pennestri, Manager, Pharmaceutical PAT & Automation, Bruker BioSpin
Magnetic Resonance is a powerful quantitative analysis technique that has many applications within the pharmaceutical industry. Traditionally used for structure elucidation and verification in the drug discovery process, magnetic resonance has been migrating to the drug development and manufacturing areas due to its strength in bioprocess characterization and quantification. To improve access to these analyses, Bruker has launched a portable benchtop NMR solution to simplify the use of this advanced technique to Bioprocess advanced control. In collaboration with Sanofi, this talk will focus on how NMR can be implemented for bioprocess monitoring and control. The application for the metabolites measurement in the upstream cultures will be discussed.

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

OVERCOMING THE ANALYTICAL BOTTLENECKS FOR AAVs

10:40 Overcoming Analytical Challenges to Expedite Gene Therapy Development
Victor Chen, Principal Scientist, Regenxbio
Gene therapy products have demonstrated great potential for treating devastating diseases and are being extensively evaluated in clinical trials for many disease indications. The structural and biological properties of these products are complex and yet to be fully understood. Analytics are key to developing safe and efficacious products. Strategies to overcome analytical challenges to ensure the highest product quality and consistency in gene therapy development will be presented.

11:10 Analysis of Critical Quality Attributes for AAV Products Using Mass Spectrometry
Zhirui (Jerry) Lian, PhD, Senior Director, Eli Lilly and Company
Adeno-associated virus (AAV) has emerged as one of the most used vectors for gene therapy. Many structural features of AAV particles such as empty/full ratio, stoichiometry of capsid viral proteins, post-translational modifications of the viral proteins, as well as the residual host cell proteins and viral proteins are considered potential critical quality attributes (CQA). Characterization and quantitation of these CQAs using mass spectrometry techniques will be discussed in this presentation.

11:40 Improving the Throughput of a Very Low Throughput Method (CE-SDS) for Purity Analysis of mAb and AAV Samples
Andy Blum, Scientist, Biogen
A limitation of CE-SDS as a method for purity determination of protein and gene therapy products is its low throughput. Method optimization and application of CE-SDS to support process development are inefficient. A new instrument capable of running eight capillaries in parallel, enabling an 8-fold increase in throughput, has become available. This talk focuses on a thorough evaluation of this instrument. Details and results of experiments performed will be described.

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

DIGITAL INITIATIVES TO ACCELERATE ANALYTICAL DEVELOPMENT

1:25 Chairperson's Remarks
Zhirui (Jerry) Lian, PhD, Senior Director, Eli Lilly and Company

1:30 Standardization of Data Collection and Analysis in Analytical Development
Nicholas Alden, PhD, Scientist, Analytical Development, Ultragenyx
This presentation will focus on the necessary steps to standardize data collection and automation practices in an established analytical development laboratory with 30 analysts running 40 different assays. We will detail our

challenges and progress to standardize data storage practices, automate routine assay setup, and make our data searchable to our broader organization. We will discuss the advantages of this approach and present our roadmap for future development.

2:00 Expanding Assay Robustness with Computer Vision
George Van Den Driessche, PhD, Lab Data Scientist, Biogen
Cell cultures expressing biotherapeutic molecules are routinely monitored using cell counters to differentiate healthy and dead cells to measure overall viability. Process development scientists can infer additional cell morphology insights by inspecting cell counter images, but this is a time-intensive process that does not scale. We applied transfer learning and fine-tuning principles to the YOLOv5 algorithm to develop a computer vision model for monitoring cell morphology.

2:30 Know Your Process: Leverage At-Line and On-Line Analytics for Faster Bioprocess Feeding Strategy Development and Optimization
Milla Neffling, Marketing Segment Manager, Bioprocessing, 908 Devices
Bioprocess intensification by, for example, higher cell densities and prolonged bioproduction processes aiming at increased yields, while maintaining the end-product quality, are key initiatives in many biopharma process development labs. Here, we show two case studies of improved control of cell culture processes: the first one focuses on the optimization of feeding strategy by daily spent media at-line amino acid analysis, and the second focuses on automated glucose control by near real-time online measurements of glucose and lactate.
Key takeaways:
• Bring your bioprocess analytics to the point-of-need to enable better understanding and decision-making in your development projects
• Expedite process development with fast, easy-to-use analytics on key nutrients and metabolites
• Remove barriers through characterization of your bioprocess and the nutrient levels required for high yields of safe and efficient product

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

PLENARY KEYNOTE: LEADING TO TOMORROW'S ADVANCES

3:50 Chairperson's Remarks
Ran Zheng, CEO, Landmark Bio

4:00 Current and Future Trends in Biomanufacturing of New Modalities
Konstantin B. Konstantinov, PhD, CTO, Ring Therapeutics
Using exosomes as an example, this presentation examines the current and future trends in biomanufacturing, and the technologies needed to manufacture emerging modalities at scale. Traditional biomanufacturing methods do not provide the industrialized, commercially scalable, highly efficient and reproducible manufacturing process essential for this new class of biotherapeutics—so we built it from the ground up.

4:30 The Digitalization of Biomanufacturing
Richard D. Braatz, PhD, Edwin R. Gilliland Professor, Chemical Engineering, Massachusetts Institute of Technology
A testbed is described for the end-to-end integrated and continuous manufacturing of monoclonal antibodies, which consists of parallel bioreactors, simulated moving bed chromatography systems, viral inactivation, and an autosampling system. Experimental results are compared with a digital twin. The increased consistency in the glycosylation profile of the monoclonal antibodies being produced is quantified when going from batch to semi-batch to perfusion mode, and when moving from start-up to quasi-steady conditions.

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

6:00 Close of Day

THURSDAY, AUGUST 17

7:30 am Registration and Morning Coffee

EMERGING METHODS AND TECHNOLOGIES

7:55 Chairperson's Remarks
Michael Butler, PhD, Principal Investigator, Cell Technology, National Institute for Bioprocessing Research & Training (NIBRT), Ireland

8:00 Leveraging Automation for Increased Throughput of Vg Titer Assays
Miriam Menezes, PhD, Senior Scientist, High-throughput Technologies and Operations, Spark Therapeutics
Manual execution of Vg titer assays (qPCR and dPCR) leads to sample testing bottlenecks, resulting in a long turnaround time for Vg titer results. This talk will present case studies highlighting how Spark Therapeutics is using automation to increase sample testing capacity (4X over manual). It will describe strategies to expand throughput using integrated work cells, which will culminate in faster delivery of life-saving gene therapies to patients.

8:30 Applications of Native Mass Spectrometry for Biotherapeutic Characterization
Delia Li, Technical Development Scientist, Protein Analytical Chemistry, Genentech
Native MS is a technique enabling the study of intact and non-covalent protein species that has become increasingly popular as a biotherapeutic characterization tool during analytical development. Coupling nanospray MS with native chromatographic separation methods allows us to directly characterize variants without time-consuming offline fractionation. We have used native MS to investigate format-specific variants for multiple biotherapeutics, including co-formulations and novel bispecifics, which will be discussed in this presentation.

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

9:30 Breakout Discussion Groups
Breakout discussions provide an opportunity to discuss a focused topic with peers from around the world in an open, collegial setting. Select from the list of topics available and join the moderated discussion to share ideas, gain insights, establish collaborations or commiserate about persistent challenges. Please visit the breakout discussions page on the conference website for a complete listing of topics and descriptions.

IN-PERSON ONLY BREAKOUT: Accelerating Analytical Development of Novel Modalities: Challenges & Potential Solutions
Xue (Shelly) Li, Associate Director, Biologics Development, Bristol Myers Squibb Co.

IN-PERSON ONLY BREAKOUT: Technological Innovations to Accelerate Analytical Development
Zhirui (Jerry) Lian, PhD, Senior Director, Eli Lilly and Company

10:30 Affinity Chromatography Mass Spectrometry – A Function-Structure-Based Approach for Streamlined Critical Quality Attribute Assessment of Biotherapeutics

Steffen Lippold, PhD, Postdoctoral Researcher, Protein Analytical Chemistry, Genentech

Affinity chromatography with online mass spectrometry hyphenation (AC-MS) on relevant binding partners of therapeutic proteins allows streamlining of the assessment of critical quality attributes. We are establishing an affinity column toolbox, including important receptors and targets, which are of high interest for the biopharmaceutical field. AC-MS as novel technology provides unprecedented resolution and sensitivity of functionally distinct proteoforms and, hence, is expected to overcome current analytical challenges of biopharmaceutical analysis.

SPECIAL PRESENTATION

11:00 USP Standards and Tools to Support Implementation of MAM

Li Jing, PhD, Principal Scientist, USP

MAM for analytical testing of biotherapeutics is gaining traction due to its potential to improve efficiency and provide more detailed information on PQAs as compared to conventional methods. This presentation will focus on the development of USP standards and tools to support MAM for product characterization and quality control. Considerations that impact the quality and consistency of MAM, approaches to enhance the robustness, and case studies will be discussed.

11:30 Enjoy Lunch on Your Own

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

PRODUCT QUALITY AND IN-PROCESS ANALYTICS

1:05 Chairperson's Remarks

Shawn Li, PhD, Principal Scientist, Analytical Research and Development (AR&D) Mass Spectrometry, Merck & Co., Inc.

1:10 Increasing the Throughput of Process Development Analytical Studies

Hirsh Nanda, PhD, Director, Analytical Sciences, Janssen

We developed a robotic automation and cloud-based database analytical pipeline for mass spectrometry characterization of biologics. The end-to-end system eliminates the need for manual sample preparation and instrument operation, improving consistency, bandwidth, and accelerating early molecule development. This lab-of-the-future, high-throughput approach enables optimal sample processing, data analysis, and interpretation, addressing a bottleneck in biologic discovery and development.

1:40 Bio-Capacitance as an Analytical Tool to Monitor Cell Growth and Metabolic Status

Michael Butler, PhD, Principal Investigator, Cell Technology, National Institute for Bioprocessing Research & Training (NIBRT), Ireland

Bio-capacitance has become a standard method to estimate biomass in cell-based bio-manufacturing processes. There is now a good understanding behind the apparent discrepancies between bio-capacitance and conventional trypan blue-based cell counts in the later stage of culture. Furthermore, capacitance measurements can determine the metabolic status of cells from changes in cytoplasmic conductivity that are indicative of the first stages of nutrient deprivation.

2:10 Transition of Multi-Attribute Method (MAM) to GMP for Biotherapeutics

Shawn Li, PhD, Principal Scientist, Analytical Research and Development (AR&D) Mass Spectrometry, Merck & Co., Inc.

The use of MAM for identity and purity testing of biopharmaceuticals offers the ability to complement and replace multiple conventional analytical technologies with a single mass spectrometry (MS) method. We developed and qualified a MAM workflow for therapeutic mAbs and their coformulations with Lys-C digestion. The developed platform MAM workflow and assay performance evaluation paved the way for its GMP qualification and enabled clinical GMP release of mAb products.

2:40 Networking Refreshment Break

2:55 LC-MS-Based Product Quality of Antibody-Based Therapeutics Direct from Cell Culture Supernatants

Juan José Bonfiglio, PhD, Science and People Lead, Mass Spectrometry, Roche, Germany

Development and production of innovative biotherapeutics demands bioprocesses that consistently yield a high-quality product. However, current methods to determine product quality do not necessarily capture the actual mix of product and related impurities in cell culture supernatant, but rather what can be captured after purification. We developed a highly-sensitive method that can be applied to the detailed characterization of cell culture supernatants from bioreactors without a falsifying pre-purification step.

3:25 Development of a Novel and Rapid HPLC-Based Method for a Comparative Assessment of Monoclonal Antibody Structural Heterogeneity

Jackie Cullinan, MS, Research Analyst, U.S. FDA

Glycosylation, a post-translational modification, is a well-defined critical quality attribute of monoclonal antibodies (mAb). Established methods to assess glycan structures and Fc effector activity are costly and time-consuming. Recently, the FcR-IIIA-NPR HPLC affinity column emerged which could provide rapid mAb glycosylation assessment. The feasibility of the FcR-IIIA-NPR HPLC affinity column as a rapid assay to identify differences in glycosylation of therapeutic mAbs is being ascertained.

3:55 Identification Testing on Product Release by 1H NMR Spectroscopy

Jacob Trotta, PhD, Senior Scientist, Analytical Development, Alkermes, Inc.

Identification tests in the pharmaceutical industry should demonstrate that the intended components in a product are present at lot release. These tests should "be able to discriminate between compounds of closely related structure" (ICH Q6A), and so limited specificity in methods such as FTIR can make identification challenging. A case study on formulations with common pharmaceutical excipients and surfactants demonstrates how NMR can serve as a powerful identification method.

4:25 Close of Summit

STREAM #6
STABILITY & FORMULATION

The Stability & Formulation stream brings together experts in formulation, analytical sciences, drug delivery, and process scientists to share practical insights and case studies via virtual and in-person presentations. These two conferences will feature methods, cutting-edge approaches for understanding and managing formulation and stability issues in biologics and novel formats, product development, screening tools and strategies to manage contaminants and impurities, aggregation issues, device integration, formulation, and process strategies for high-concentration protein formulation and protein device combinations.

Conference Programs

AUGUST 14-15

Rapid Methods to Assess Stability and Impurities in Biologics

View Program »

AUGUST 16-17

Formulation and Delivery of High-Concentration Proteins and New Modalities

View Program »

MONDAY, AUGUST 14

8:00 am Registration and Morning Coffee

AI/MACHINE LEARNING, PREDICTIVE TECHNIQUES FOR DEVELOPABILITY AND STABILITY

9:55 Chairperson's Opening Remarks
Alejandro D'Aquino, PhD, Principal Investigator, GSK

10:00 Use of Biophysical Techniques and Other Predictive Parameters during Late Phase Formulation Development of Drug Products
Alejandro D'Aquino, PhD, Principal Investigator, GSK
Differences in shape of nDSF samples as well as calculated and predictive methods, such as kD, have been used to predict the long-term stability of 90 mg/mL to 150 mg/mL formulations of drug product. The results obtained allow the use of this approach to determine relative stability as early as 1 month during long-term stability studies in the development of drug products.

10:30 Machine Learning Predictions of Chemical Stability in Early Stages of Antibody Discovery
Kyle A. Barlow, PhD, Scientist, Computational Biology, Adimab LLC
Chemical modifications such as deamidation, isomerization, and oxidation can affect the function of antibody therapeutics and complicate development. Given that experimental assessment is resource-intensive, we present machine learning models, trained on data from over 700 antibody samples, that predict sites where liabilities are likely to occur from sequence input alone. These models can be run throughout the discovery and lead optimization process, allowing for proactive removal of sequence liabilities.

11:00 Tailoring Antibody Developability Assays for Machine Learning to Speed up Lead Optimization
Dennis Åsberg, PhD, Senior Scientist, Biophysics and Injectable Formulation, Novo Nordisk A/S, Denmark
In silico assessment of antibody developability have the potential to speed up antibody development. However, advances in computational tools such as machine learning models are often limited by the lack of suitable training data sets of high quality. Here, I present improvements of common antibody developability assays; e.g., AC-SINS, with the aim of enabling optimal data for modelling. Important parameters like dynamic range, calibration and data processing are discussed.

11:30 Subvisible Particle Characterization in Cell and Gene Therapies: A High-Throughput, Low-Volume Approach
Karessa White, PhD, Field Application Scientist, Halo Labs
Learn about analyzing subvisible particles for product purity and stability using high-throughput, low-volume analysis with Aura+. This technology images, counts, and sizes subvisible particles in a variety of biologics, including protein, cell, and gene therapies. Additionally, it can identify viral vector and cellular aggregates, and external impurities in high throughput to control stability.

12:00 pm Enjoy Lunch on Your Own

12:30 Session Break

TOOLS AND METHODS FOR SCREENING DEVELOPABILITY AND STABILITY

12:50 Chairperson's Remarks
John P. Marino, PhD, Group Leader, Biomolecular Structure & Function Group, NIST

12:55 Predictive Nature of High-Throughput Assays in ADC Formulation Screening
Siyuan Ren, PhD, Senior Scientist I, Global Pharmaceutical R&D, AbbVie, Inc.
High-throughput screening techniques for biophysical properties analysis in early screening studies is warranted due to limited material and large number of molecules. Predictability of the data to long-term stability is critical. In this study, biophysical properties and 8-week stability of two ADCs in 16 formulations were evaluated, and the predictive capabilities was assessed. Tagg and B22 is more predictive than Tm for stability. High-throughput assays also identified poor-performing formulations.

1:25 Investigations of a Bispecific Antibody Dimerization via Hydroxyl Radical Footprinting
Harsha Gunawardena, PhD, Principal Scientist, Mass Spectrometry, Janssen Pharmaceutical Companies of Johnson & Johnson
Aggregation of recombinant proteins is a major consideration in their developability, safety, and immunogenicity. While detection of aggregates and analysis of their basic properties is routine, understanding the molecular mechanism involved is much more challenging. Structural mass spectrometry techniques such as hydroxyl radical protein footprinting (HRPF) was used to decipher mechanism of aggregate in biotherapeutics development to reduce development timelines

1:55 Tackle High-Concentration Biologics with the Right Toolkit
Andre Mueller, PhD, Marketing Manager, Biologics Solutions, Unchained Labs
High-concentration biologics for subcutaneous administration require complex formulation studies. Unchained Labs offers solutions to buffer exchange and quantify biologics, then screen quality, stability and viscosity. Join my talk for a case study using a high-throughput, low volume workflow to screen the effects of common excipients on four monoclonal antibodies at low and high concentrations. Impacts of formulation vary for each antibody and concentration, making access to effective screening tools crucial.

2:25 Networking Refreshment Break

2:40 KEYNOTE PRESENTATION: A NIST-FDA Initiative to Benchmark Methods for Profiling and Predicting the Stability of mAbs
John P. Marino, PhD, Group Leader, Biomolecular Structure & Function Group, NIST
The benefits of predicting the quality and stability of formulated monoclonal antibodies (mAbs) under storage and transport conditions are widely recognized in the biopharma industry and by regulators and efforts to predict these properties of biologics are long-standing. To this end, a robust framework will be described for accelerating understanding and confidence in the performance of experimental approaches and models proposed for profiling and predicting the thermal stability of mAbs.

3:10 Characterizing Stability and *in vitro/in vivo* Translatability of Novel Large Molecule Modalities Using Complementary Bioanalytical Tools
Jeff Lin, PhD, Senior Scientist, Genentech
Novel therapeutic modalities are emerging to deliver sophisticated mechanism of actions to modulate the "undruggable" targets. However, little is known about the *in vivo* stability liabilities with these new modalities. Here we report a multi-pronged approach using LC-MS and capillary electrophoresis-based methods to characterize and quantify biotransformation liabilities and the *in vitro/ex vivo* vs. *in vivo* translatability, including but not limited to linker deconjugation, clipping, and amino acid level modifications.

3:40 Session Break and Transition to Plenary Keynote Session

PLENARY KEYNOTE: SOLVING TODAY'S CHALLENGES

4:20 Chairperson's Remarks
Susan D'Costa, PhD, CTO, Genezen

4:30 Overcoming the Challenges of Bioprocesses: The Future of Biomanufacturing
Glen R. Bolton, PhD, Executive Director, Late Stage Bioprocess Development, Amgen, Inc.
Novel therapies and technologies are emerging to meet the needs of patients; however, the manufacturing of biopharmaceuticals remains a complex and challenging process. As demand for biopharmaceuticals grows, the industry faces new challenges in terms of scalability, cost, and process robustness. The implementation of innovative technologies to improve process efficiency and the importance of process control and data analytics in ensuring process robustness are key levers to meet these challenges.

5:00 Commercializing Gene Therapies—The Combined Power of Patient Advocacy and Cost-Effective Manufacturing
Rachel Salzman, DVM, Founder, The Stop ALD Foundation; Global Head, Corporate Strategy, Armatus Bio
This presentation will examine the development of an FDA-approved gene therapy where patient advocacy played a critical role resulting in the first ever clinical use of a lentiviral vector. Although manufacturing continues to represent a significant challenge throughout the entire R&D journey, there are opportunities for advocacy and manufacturing communities to seek alignment and combine their collective powers to achieve the common goal of increasing patient access to transformative medicines.

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

6:30 Close of Day

TUESDAY, AUGUST 15

7:30 am Registration and Morning Coffee

PROTEIN AGGREGATION, PROCESS IMPURITIES, AND IMPURITY CONTROL

7:55 Chairperson's Remarks
Philip M. Kim, PhD, Professor, The Donnelly Centre for Cellular and Biomolecular Research, Department of Molecular Genetics Department of Computer Science, University of Toronto

8:00 Protein Aggregation under Flow: Mechanisms and Applications
Leon Willis, PhD, Postdoctoral Research Fellow, School of Molecular and Cellular Biology, University of Leeds
Therapeutic proteins are susceptible to aggregation throughout their lifetime, with hydrodynamic forces and interfacial stresses being major culprits. We have developed a low-volume extensional flow device (EFD) to understand the kinetic mechanism underpinning flow-induced aggregation. Armed with this knowledge, we can then apply the device as a screening tool for formulations which promote long-term stability under storage conditions.

8:30 Machine Learning Methods for the Design and Engineering of Protein Therapeutics
Philip M. Kim, PhD, Professor, The Donnelly Centre for Cellular and Biomolecular Research, Department of Molecular Genetics Department of Computer Science, University of Toronto
I will present methodologies developed in my lab for the design and engineering of antibodies. We use machine learning for *de novo* design and make use of modern technologies to ensure all our designs are developable. We show high yields and other developability criteria of our hit antibodies.

9:00 Analytical Procedure Development and Validation—Apply New ICH Guidelines to Biotech QC Lifecycle Management
Kevin Zen, PhD, Senior Director, IGM Biosciences
Analytical Quality by Design offers a systematic and robust approach to the development of analytical procedures involving all stages of the product's lifecycle. The presentation will overview the new draft ICH guidelines on analytical procedure development, validation, and lifecycle management. Special emphasis will be placed on the analytical procedures commonly used in biotherapeutics for GMP release and stability.

9:30 Selected Poster Presentation: Progress on a Sub-Micrometer Particle Number Concentration Reference Material
Kurt D. Benkstein, PhD, Research Chemist, Biomolecular Measurement Division, NIST
Measurements of the size and number concentration of sub-micrometer particles can have large errors, especially for polydisperse samples often encountered in biopharmaceutical applications. We are developing multi-modal silica particle mixtures as potential reference materials to optimize instrument settings and measurement repeatability. We use particle tracking analysis, a common method in this size regime, to examine the effects of acquisition and analysis parameters on size and count errors.

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

10:45 Breakout Discussion Groups
Breakout discussions provide an opportunity to discuss a focused topic with peers from around the world in an open, collegial setting. Select from the list of topics available and join the moderated discussion to share ideas, gain insights, establish collaborations or commiserate about persistent challenges. Please visit the breakout discussions page on the conference website for a complete listing of topics and descriptions.

TABLE: Host cell proteins (HCPs): Challenges and Opportunities
Sunny Zhou, PhD, Professor, Chemistry & Chemical Biology, Northeastern University
- High-risk host cell proteins (HCPs): what and why
- Characterization: challenges and opportunities
- Removal (purification): challenges and opportunities

PROTEIN AGGREGATION, PROCESS IMPURITIES, AND IMPURITY CONTROL (CONT.)

11:30 Co-Presentation: Development of a Platform Approach for the Affinity Capture and Characterization of Problematic Host Cell Proteins (HCPs)
Michael Dolan, Staff Engineer, Process Development US, Takeda Pharmaceuticals
Sunny Zhou, PhD, Professor, Chemistry & Chemical Biology, Northeastern University
Despite of the advances in protein purification, host cell proteins (HCPs) remain a potential concern in protein therapeutics, as they may affect both product quality and immunogenicity in patients. Deeper understanding of the chemical nature of HCPs will guide rational design for their control and removal. In this talk, we will discuss our new approaches to enrich HCPs for their subsequent characterization.

12:00 pm Characterization of Therapeutic Antibody Charge Variants in Drug Development by Microfluidic Native Capillary Electrophoresis-Mass Spectrometry
Zhijie Abe Wu, PhD, Principal Scientist, Analytical Chemistry, Regeneron Pharmaceuticals, Inc.
Therapeutic antibodies are a major class of biopharmaceuticals that can treat a variety of diseases. As an important type of product-related impurities, charge variants arising from post-translational modifications and truncations can affect the stability, efficacy, and safety of drug products. In this study, we present the development and application of microfluidic native capillary electrophoresis coupled to mass spectrometry to monitor the charge variants in therapeutic antibody drug candidates.

12:30 Enjoy Lunch on Your Own

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

ANALYTICAL TOOLS AND CHARACTERIZATION STRATEGIES FOR VACCINES

2:10 Chairperson's Remarks
Marina Kirkitadze, PhD, Head Bioprocess Support & PAT Platform, Analytical Sciences, Sanofi Pasteur

2:15 FEATURED PRESENTATION: Advances in Quality Control Standards for Polysaccharide Conjugate Vaccines
John Cipollo, PhD, Senior Principal Scientist and Team Lead, USP
Polysaccharide Conjugate vaccines are the most successful preventatives against bacterial disease and are composed of defined polysaccharides individually conjugated to carrier protein. This presentation will provide an update on standards and tools to support vaccine quality from raw materials through release testing. The recent revisions to General Chapter <198>, focused on NMR for bacterial polysaccharide for identity, and reference materials to support testing of quality attributes will be discussed.

2:45 Characterization of Vaccine Drug Product by Contact-Free Sensors
Marina Kirkitadze, PhD, Head Bioprocess Support & PAT Platform, Analytical Sciences, Sanofi Pasteur
The focus of the presentation is evaluation of wNMR as an emerging noninvasive analytical technology to characterize aluminum-adjuvanted vaccine formulations. In this work, wNMR and optical techniques such as laser diffraction and laser scattering were used to characterize vaccine formulations containing different antigen loads adsorbed onto AlPO4 adjuvant microparticles, including the fully dispersed state and the sedimentation process. The results of the study will be discussed.

3:15 Single-Particle Imaging to Quantitate Biophysical Properties of mRNA Lipid Nanoparticles and Engineer Improved Vaccines
Sabrina Leslie, PhD, Associate Professor, Physics and Astronomy Department, The University of British Columbia
We present quantitative single-particle imaging platform that enables simultaneous measurements of the size, mRNA-payload, and dynamic properties of vaccines in cell-like conditions. We investigate dependence of mRNA-lipid-nanoparticle structure and fusion dynamics on formulation, using commercially available formulations as a starting point. These measurements are made on confined, freely diffusing particles, and during reagent-exchange such as in response to solution pH, in order to emulate intracellular dynamics in a controlled setting.

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

4:30 Size Characterization of Vaccine Antigens: Ensemble vs. Single Particle Analysis Approach
Rahul Misra, PhD, Scientist, Biophysics and Process Analytical Technology, Sanofi
Single particle analysis approach involves TRPS technology which measures size of individual particles passing through a nanopore and claims to provide more accurate particle size distribution data within a sample. Particle concentration analysis is also based on single-particle measurements thereby ensuring highly accurate calculations for each size band compared to the ensemble-based approach. The study performs comparative analysis of size distribution of vaccine antigens to evaluate performance of these methods.

5:00 Microfluidic Electrophoresis-Based Detection and Characterization of dsRNA Contaminants in mRNA Vaccines
Anubhav Tripathi, PhD, Professor, Engineering & Medical Sciences, Brown University
We propose a dual dynamic staining high-throughput microfluidic electrophoresis analytical method for the detection and characterization of dsRNA contaminants in mRNA vaccines. With an mRNA maximum loading capacity of 13 ng/µL, we can detect dsRNA contaminants as low as 0.1-0.6% of the total concentration.

5:30 Close of Rapid Methods to Assess Stability Conference

WEDNESDAY, AUGUST 16

7:30 am Registration and Morning Coffee

HIGH-CONCENTRATION PROTEIN FORMULATIONS

7:55 Chairperson's Opening Remarks
Namita Sawant, PhD, Senior Scientist, Amgen

8:00 Sequence Engineering to Improve the "Syringeability" of High-Concentration Monoclonal Antibody Formulations
Georgina Armstrong, PhD Student, Senior Scientist, BioPharm Process Research, Drug Substance Development, GSK
High viscosity of high-concentration monoclonal antibody formulations poses risks to manufacturability, product quality, immunogenicity, and injectibility in volume-limited autoinjector device. We present viscosity data from mutant candidates of an IgG1, proposed to alter the viscosity against the wild-type from *in silico* predictions, as well as investigating the biophysical impact from single-point mutations. Promising mutants will progress onto device experiments to infer their relevant syringeability and incite future mutant design.

8:30 *In silico*-Based Design of High-Concentration Protein Formulation
Maral Adeli Koudehi, PhD, Scientist, Drug Product Development, Johnson & Johnson
Advanced AI/ML models can be applied to protein science, specifically in high-concentration antibody formulation. *In silico* methods improve the selection process and design steps in protein development. This presentation summarizes strategies being developed and implemented for structural assessment of biotherapeutic proteins in combination with designing high-throughput formulation using modeling and simulation.

9:00 Strategies in the Development and Manufacturing of Low-Viscosity, Ultra-High-Concentration Formulation for IgG1 Antibody
Vaibhav Deokar, Principal Scientist, Formulation Development, Biotechnology Division, Lupin Ltd.
The present research provides comparative evaluation of scalable manufacturing strategies to develop low-viscosity (150mg/mL) formulation for lyophilized biosimilar IgG1; suitable for single, subcutaneous injection ~600mg/3.0mL, per dose.IgG1 was concentrated to ~200mg/mL and provides a comparative evaluation of manufacturing strategies and their impact on the chemical and structural stability of IgG1. Techniques used for concentration of IgG1 are tangential flow filtration (TFF), spray drying (SPD), and spray freeze drying (SFD).

9:30 Trehalose, Sucrose, and Amino Acids: Essential Components of Platform Biopharma Formulations Pfanstiehl
Sudhakar Voruganti, PhD, Director, Business Development, Pfanstiehl, Inc.
Bullet points:
• Commercial Biotherapeutics Stabilized with Trehalose / Sucrose
• Understanding physicochemical properties of Trehalose and Sucrose
• Advantages of Trehalose over Sucrose
• From Liposome to m-RNA vaccines – importance of highly purified characterized Excipients
• Typical components in mRNA-LNP vaccine Excipients
• Examples for utilizations of Sucrose and Trehalose in Covid 19 related formulations
• Amino Acid Buffers in commercial formulations – Importance of highly characterized AAs as Excipients
• Methionine as Biopharmaceutical Stabilizer and Antioxidant

9:45 Novel Recombinant Hyaluronidase for Subcutaneous Delivery of Biologics BMI KOREA
Hojun Choi, Executive Vice President, Chief Strategy Officer, BMI KOREA
BMI introduces BMI-2004, a novel recombinant ovine sequenced hyaluronidase for improved subcutaneous biologic delivery. The developmental goal of BMI 2004 is minimize the change of original Mab formulation. The main advantages of the enzyme is 1) the same pH range to Mab at pH 5, 2) highly concentrated form, 3) proprietary liquid formulation technology of hyaluronidase enzyme. Phase1 trial (n=250) and pre-clinical study results will be shared.

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

HIGH-CONCENTRATION PROTEIN FORMULATIONS (CONT.)

10:40 Phase-Appropriate and State-of-the-Art Approaches for Developing High-Dose Subcutaneous Drug Products
Namita Sawant, PhD, Senior Scientist, Amgen
In this talk, I will discuss phase-appropriate and state-of-the-art approaches for developing high-dose subcutaneous drug products.

11:10 Upper Concentration Limits of mAb Therapeutics—Strategies for High-Concentration (>200mg/ml) Drug Products
Ian Roy, Scientist, Formulation, Janssen R&D
Viscosity increases exponentially and stability decreases precipitously as mAbs are concentrated above 200 mg/ml but the challenges don't end there! Process limitations, DP characteristics, and available administration options all present upper bounds on the concentration of a mAb. This presentation will summarize a recent attempt to develop a mAb that is "as concentrated as physically possible."

11:40 Evaluation of a Silicone-Free Syringe and Stopper Presentation for Use in Biopharmaceutical Drug Product Development
Cait Sofa (Quaile), Principal Scientist, Biopharmaceutical Drug Product Development, GlaxoSmithKline
Subcutaneous biopharmaceutical drug products are often supplied in a siliconized syringe with stopper. Levels of siliconization vary lot-to-lot leading to different levels of drug product-silicone interactions during storage. This presentation includes an assessment of product quality for three drug products using two syringe systems, an unsiliconized glass syringe with polytetrafluoroethylene coated stopper, and a siliconized syringe and stopper. The study demonstrates that an unsiliconized presentation is a suitable alternative.

12:10 pm Enjoy Lunch on Your Own

12:40 Refreshment Break in the Exhibit Hall with Poster Viewing ORFLO Personal Cell Analysis

IMPORTANT FORMULATION DEVELOPMENT CONSIDERATIONS FOR BIOLOGICS

1:25 Chairperson's Remarks
Jennifer Litowski, PhD, Senior Principal Scientist, Drug Product Technologies, Amgen, Inc.

1:30 In the Patient's Hands: In-Use Compatibility Testing and Strategies
Jennifer Litowski, PhD, Senior Principal Scientist, Drug Product Technologies, Amgen, Inc.
Drug products undergo handling by patients and healthcare professionals, including reconstitution of lyophilized products, transfer to syringes, and dilution into IV bags for administration. In-use compatibility studies are performed to show that stability and concentration are maintained, ensuring patient safety and accurate dosing. However, regulatory expectations are not well-defined, leading to uncertainty in study parameters. Recent cross-product data and a multi-company collaboration offer guidance for streamlined experimental design.

2:00 Presentation to be Announced

2:30 Sponsored Presentation *(Opportunity Available)*

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

PLENARY KEYNOTE: LEADING TO TOMORROW'S ADVANCES

3:50 Chairperson's Remarks
Ran Zheng, CEO, Landmark Bio

4:00 Current and Future Trends in Biomanufacturing of New Modalities
Konstantin B. Konstantinov, PhD, CTO, Ring Therapeutics
Using exosomes as an example, this presentation examines the current and future trends in biomanufacturing, and the technologies needed to manufacture emerging modalities at scale. Traditional biomanufacturing methods do not provide the industrialized, commercially scalable, highly efficient and reproducible manufacturing process essential for this new class of biotherapeutics—so we built it from the ground up.

4:30 The Digitalization of Biomanufacturing
Richard D. Braatz, PhD, Edwin R. Gilliland Professor, Chemical Engineering, Massachusetts Institute of Technology
A testbed is described for the end-to-end integrated and continuous manufacturing of monoclonal antibodies, which consists of parallel bioreactors, simulated moving bed chromatography systems, viral inactivation, and an autosampling system. Experimental results are compared with a digital twin. The increased consistency in the glycosylation profile of the monoclonal antibodies being produced is quantified when going from batch to semi-batch to perfusion mode, and when moving from start-up to quasi-steady conditions.

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

6:00 Close of Day

THURSDAY, AUGUST 17

7:30 am Registration and Morning Coffee

DRUG-DEVICE COMBINATIONS

7:55 Chairperson's Remarks
Christoph Brandenbusch, PhD, Assistant Professor, Bioprocess Separations & Biologics Formulation Development, TU Dortmund University, Germany

8:00 Characterizing Silicone Oil Migration and Its Impact on Biologic Drug Product in Prefilled Syringes
Xi Zhao, PhD, Senior Scientist, Sterile and Specialty Products, Merck
Despite the benefits that prefilled syringes (PFS) can provide to patients, the silicone oil pre-coated on the glass barrel may migrate into the drug product and impact drug product quality. I will present risk assessments of silicone oil migration by variables to enable a thorough and optimal selection of primary container closure and de-risk the impact of silicone oil on drug product stability.

8:30 Addressing Challenges in Combination Product Development for SC Delivery
Ajit M. D'Souza, PhD, Director Combination Product Development & Manufacturing, Combination Product Development & Manufacturing, Kiniksa Pharmaceuticals Corp.
As healthcare systems increasingly rely on medical care administered at home, the focus on ease of administration, safety, compliance and sustainability is driving the development of combination products for home use. This presentation will discuss approaches to addressing three key challenges: i) drug-device compatibility, ii) reliability and robustness of performance and iii) usability issues in the context of high concentration mAb formulations.

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

9:30 Breakout Discussion Groups
Breakout discussions provide an opportunity to discuss a focused topic with peers from around the world in an open, collegial setting. Select from the list of topics available and join the moderated discussion to share ideas, gain insights, establish collaborations or commiserate about persistent challenges. Please visit the breakout discussions page on the conference website for a complete listing of topics and descriptions.

IN-PERSON ONLY BREAKOUT: Talk Title to be Announced
Amit Kumar, PhD, Global Engineering- Site Lead Biomanufacturing Facility Design and Capital Project, Moderna

PLATFORM TECHNOLOGIES, FORMULATION, AND DELIVERY TECHNOLOGIES

10:30 Formulation and Delivery Vehicles for Vaccines
Soumen Saha, PhD, Senior Research Scientist, Duke University
Lipid nanoparticles (LNPs) used in mRNA vaccines are stabilized by PEG. Up to 70% of individuals have anti-PEG antibodies that can significantly reduce the efficacy and induce allergic reactions to PEG-containing vaccines. To address these limitations of PEG, we are developing LNPs with a next-generation PEG-like polymer that does not bind pre-existing PEG antibodies and that has minimal immunogenicity but has similar efficacy as PEG-containing LNPs.

11:00 Formulation Development Considerations for AAVs
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, in-use administration, and shipping/handling. This entails screening for buffer pH, ionic strength, and the impact of added surfactants on stability/degradation trends of drug product.

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 SYSTEMS: FORMULATION, ANALYSIS, PROCESS DEVELOPMENT, AND DELIVERY

1:05 Chairperson's Remarks
Amey Bandekar, PhD, Associate Director, Drug Product Development, Sanofi

1:10 FEATURED PRESENTATION: Advances in Chemistry Made RNAi Therapeutics Possible
Mano Manoharan, PhD, Distinguished Scientist & Senior Vice President, Innovation Chemistry, Alnylam Pharmaceuticals
For siRNAs, chemical modifications are necessary to regulate metabolic stability, potency, and safety. We have evaluated numerous chemical modifications These include backbone chiral phosphorothioates, glycol nucleic acids, altriol nucleic acids, gem 2'-deoxy-2'-α-F-2'-β-C-methyl, 5'-morpholino, and amino-oxy click chemistry (AOCC)-mediated conjugates. Furthermore, novel spatial architectures like circular siRNAs have also been evaluated. This presentation will summarize how chemistry has made possible the currently exciting world of RNAi therapeutics.

1:40 Equilibrium and Stability Considerations in the Development and Manufacturing of Liposome and LNP Formulations
Christoph Brandenbusch, PhD, Assistant Professor, Bioprocess Separations & Biologics Formulation Development, TU Dortmund University, Germany
Liposome and LNP formulations have evolved as highly potent drug delivery systems. Recent literature gives valuable insights in developing and manufacturing these formulations, with focus mainly set on delivering different liposome and LNP compositions/sizes. This presentation will give some insight into thermodynamic equilibrium considerations, such as long-term (size) stability of liposomes and LNPs at various temperature, as well as equilibrium radius considerations in manufacturing liposomes and LNPs.

2:10 Considerations and Challenges in Early LNP Development for Non-Viral Gene Therapy
Yuefei Shen, PhD, Principal Scientist, CMC Drug Product Development, Sanofi
LNP technology shows the ability to deliver nucleic acid therapeutics for non-viral gene therapy (NVGT). Compared to intramuscular vaccine delivery, intravenous (IV) delivery of an LNP formulation for gene therapy shows unique challenges. An LNP formulation for gene therapy via IV may require different lipid and formulation design. Here, we will discuss the considerations and challenges in early LNP development and impact on tissue targeting for NVGT.

2:40 Networking Refreshment Break

LNPs & NOVEL DELIVERY SYSTEMS (CONT.)

2:55 Process Development Considerations for LNP Manufacturing
Amey Bandekar, PhD, Associate Director, Drug Product Development, Sanofi
In the development of LNP-based drug product, the choice of manufacturing technology is one of the key factors for success. The choice of technology can have a significant impact on the biophysical properties, structural characteristics, colloidal stability, and efficacy of the LNP. This study describes the impact of different manufacturing parameters and the scale-up consideration to enable successful LNP drug product manufacturing.

3:25 De-Risk mRNA Adduct Formation in Lipid Nanoparticle Formulation Intended for Glycogen Storage Disease Type-1a Using Analytical Tools
Siddharth Bhoraskar, PhD, Scientist II, Beam Therapeutics
In this talk, we discuss de-risking mRNA adduct formation in lipid nanoparticle formulation intended for glycogen storage disease type-1a using analytical tools.

3:55 Close of Summit

STREAM #7
VACCINE & mRNA THERAPIES

Vaccines and mRNA-based therapies have gained a lot of attention recently due to COVID-19. The Vaccine & mRNA Therapies stream will explore the technical challenges facing the formulation, development, characterization, manufacturing, and supply of next-generation vaccines, mRNA vaccines, mRNA gene therapies, RNAi, and gene therapies. Experts from pharma, biotech, academia, and government labs will convene in Boston in person and virtually to deep dive into the challenges associated with successfully developing and delivering these next-generation therapeutics.

Conference Programs

AUGUST 14-15

Vaccine Development and Manufacturing

View Program »

AUGUST 16-17

mRNA-Based Therapies

View Program »

MONDAY, AUGUST 14

8:00 am Registration and Morning Coffee

CHALLENGES & OPPORTUNITIES IN VACCINES

9:55 Chairperson's Opening Remarks
Lisa A. Kueltzo, PhD, Director, Formulation & Stability, Vaccine Production Program Lab, NIH NIAID

10:00 PANEL DISCUSSION: The Power of Public-Private Partnerships to Support Multi-Use Technologies in the Next Generation of Vaccine Development
Moderator: Rachel Rath, Director, BARDA Alliance at Johnson & Johnson Innovation, JLABS
Early-stage companies and innovation leaders will discuss the advancement of multi-use technologies to support the next generation of vaccine development and how public-private partnerships can accelerate these innovative efforts.
Panelists:
Larry Forman, Founder & CEO, CHO Plus
Timothy T. Belski, Biologist, BARDA, US Department of Health & Human Services
Jacob Glanville, Founder, CEO & President, Centivax
Sogra Ali, Senior Manufacturing Engineer, Jurata Thin Film
Peter Berglund, PhD, CSO, HDT Bio

11:00 KEYNOTE PRESENTATION Opportunities to Enhance Vaccine Development and Approval Process: On a Path to More Personalized Vaccinology
John Mattison, Operating Partner/Chief Medical Information Officer, Arsenal Capital Partners
The remarkable speed of delivery of effective vaccines to address SARS-CoV-2 was not matched with commensurate predictive value of durability of those vaccines against emerging variants, nor about predicting who might suffer adverse events. This talk will lay a foundation for how early R&D and later clinical trials can systematically and cost-effectively maintain a high speed to market while providing better information to enable more predictive value and durability.

11:30 Schistosomiasis Vaccine Development Collaboration
Albert Reger, Early Molecule Business Development Manager, MilliporeSigma
Texas Children's Hospital Center for Vaccine Development in collaboration with MilliporeSigma successfully developed a purification process for a vaccine against Schistosomiasis, a tropical disease caused by parasitic worms. Schistosomiasis is second only to malaria in terms of global economic impact. The overall goal of this work was to design a safe, and low-cost manufacturing process for the purification of the vaccine antigen. This was accomplished by redesigning the original process.
MilliporeSigma assisted Texas Children's with studies to eliminate the dilution prior to lysate clarification and to also streamline the process to enable Texas Children's to simultaneously clarify and concentrate the yeast lysate.
This presentation will detail the optimized clarification and concentration process and highlight the economic and process simplification benefits of using the cascade TFF system, which can also be successfully applied to other lysate processes, particularly in gene therapy, which will also be highlighted and detailed in the presentation.

12:00 pm Enjoy Lunch on Your Own

12:30 Session Break

CELL LINE DEVELOPMENT & OPTIMIZATION FOR VACCINES

12:50 Chairperson's Remarks
Mark A. Emalfarb, Founder & President & CEO, Dyadic International, Inc.

12:55 Filamentous Fungal Cell Cultures Bloom with Benefits for Manufacturers and Patients Globally
Mark A. Emalfarb, Founder & President & CEO, Dyadic International, Inc.
Filamentous fungal cell cultures outperform conventional cell cultures in the production of biologics. Cell lines represent a particularly tight bottleneck in biomanufacturing. Conventional cell lines—such as CHO, bacterial, and baculovirus (insect cell) expression systems reveal their limitations when supplies of vaccines and biologics fall short of demand. An obvious example: shortages of COVID-19 vaccines. Less remarked upon, but even more worrying, are global shortages of monoclonal antibodies and other biologics.

1:25 Overcoming Manufacturing and Analytical Challenges to Develop a Successful Insect-Cell Expressed Protein-Based COVID-19 Vaccine
Nikolai Petrovsky, PhD, Research Director, Vaxine Pty Ltd.
Recombinant proteins remain the most reliable, low cost and safe approach to pandemic vaccine delivery but face challenges in design, manufacture, structural characterisation and scale up. The successful launch of SpikoGen vaccine as the first spike-protein vaccine to receive regulatory approval shows protein-based approaches still have much to offer and have not been supplanted by newer genetic vaccine technologies.

1:55 OPUS Pre-Packed Columns for Risk Reduction: Statistical Process Control Analysis Demonstrates Performance Consistency
Shelly Parra, Senior Director, OPUS & ELISA Product Management, Repligen
The OPUS manufacturing team packed, tested, and gathered process data for > 6,000 OPUS Columns spanning 150+ resin types. Consistency is observed for efficiency and asymmetry values across each sizing segment within the OPUS Column portfolio based on analysis of frequently produced configurations. Due to the control of OPUS® Pre-packed Chromatography Column input parameters, Repligen offers confidence in predictive results for output performance metrics.

2:10 Sponsored Presentation *(Opportunity Available)*

2:25 Networking Refreshment Break

FORMULATION DEVELOPMENT & STABILITY OF VACCINES

2:40 Development of Virus-Like Particle (VLP) Vaccine Candidates
Trudy G. Morrison, Professor, Microbiology & Physiology System, University of Massachusetts
My laboratory has developed a novel vaccine platform, virus-like particles (VLPs), formed with the structural proteins of Newcastle disease virus (NDV), an avian paramyxovirus. Using our platform, we have constructed VLPs containing the respiratory syncytial virus (RSV) F and G glycoproteins. Our preclinical studies have shown that these VLPs are excellent vaccine candidates for protection from RSV infection of neonates by maternal immunization and the protection of the elderly.

3:10 Pre-Formulation Developability Assessments: Design and Critical Considerations
Lisa A. Kueltzo, PhD, Director, Formulation & Stability, Vaccine Production Program Lab, NIH NIAID
Developability assessments are key in making decisions on how, and indeed if, a therapeutic or vaccines candidate can move from bench to clinical trial. The assessment design and implementation must also consider program capabilities and goals, limitations, and risk tolerance. In this presentation, we will discuss the elements of development assessments, their alignment with pre-formation programs, and successful incorporation into early-stage development programs.

3:40 Session Break and Transition to Plenary Keynote Session

PLENARY KEYNOTE: SOLVING TODAY'S CHALLENGES

4:20 Chairperson's Remarks
Susan D'Costa, PhD, CTO, Genezen

4:30 Overcoming the Challenges of Bioprocesses: The Future of Biomanufacturing
Glen R. Bolton, PhD, Executive Director, Late Stage Bioprocess Development, Amgen, Inc.
Novel therapies and technologies are emerging to meet the needs of patients; however, the manufacturing of biopharmaceuticals remains a complex and challenging process. As demand for biopharmaceuticals grows, the industry faces new challenges in terms of scalability, cost, and process robustness. The implementation of innovative technologies to improve process efficiency and the importance of process control and data analytics in ensuring process robustness are key levers to meet these challenges.

5:00 Commercializing Gene Therapies—The Combined Power of Patient Advocacy and Cost-Effective Manufacturing
Rachel Salzman, DVM, Founder, The Stop ALD Foundation; Global Head, Corporate Strategy, Armatus Bio
This presentation will examine the development of an FDA-approved gene therapy where patient advocacy played a critical role resulting in the first ever clinical use of a lentiviral vector. Although manufacturing continues to represent a significant challenge throughout the entire R&D journey, there are opportunities for advocacy and manufacturing communities to seek alignment and combine their collective powers to achieve the common goal of increasing patient access to transformative medicines.

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

6:30 Close of Day

TUESDAY, AUGUST 15

7:30 am Registration and Morning Coffee

DEVELOPMENT, BIOPROCESSING, AND MANUFACTURE OF VACCINES

7:55 Chairperson's Remarks
Alois Jungbauer, PhD, Professor & Head, Biotechnology, Institute of Bioprocess Science and Engineering, University of Natural Resources and Life Sciences (BOKU)

8:00 Pre-CPE Cellular Events as Surrogate for Virus Potency Analytics—A Key for Accelerated Vaccine Development
Johanna Bacher, PhD, Junior Researcher, Biotechnology, ACIB GmbH
TCID50 and plaque assay are still gold-standard virus potency assays and rely on the induction of a cytopathogenic effect (CPE) as an endpoint for evaluation. These morphological changes are usually only visible after 1-2 weeks of infection and can be replaced by cellular events induced early after virus infection like a pro-inflammatory innate immune response in the form of secreted cytokines.

8:30 Laser Force Cytology as a Process Analytical Technology (PAT) to Improve the Efficiency and Consistency of Biologics Research, Development, and Production
Colin G. Hebert, PhD, Senior Vice President, Scientific and Business Operations, LumaCyte
Developing vaccines can be challenging and time-consuming due to the complex nature of the raw materials, process, and final product. Many analytical methods face challenges in terms of speed, reproducibility, and resource requirements. Laser force cytology is a real-time PAT to characterize viral infectivity and other critical quality attributes to inform and optimize production and a tool for offline release and potency assays used to ensure product quality and consistency.

9:00 Development and Optimization of a New Process to Maximize the Yield of Recombinant Hemagglutinin Production, a Component of Flublok Influenza Vaccine
Jamal Meghrous, PhD, Senior Scientist, Cell Culture & Production, Sanofi
Sanofi's influenza vaccine is the only recombinant influenza vaccine approved by the FDA and by European Medicines Agency. Successful commercialization of influenza vaccines is a key growth driver for Sanofi Pasteur to increase the yield, decrease the cost per dose, and increase Manufacturing (MFG) capacity. We developed a new Fed-Batch process for production of recombinant HAs that results in 80% increase in rHA yield compared to the current production process.

9:30 Fast Track Analysis for Pandemic Readiness: Approaches for Accelerated Adventitious Virus Testing in Vector Vaccines
Oliver Klepsch, Research Associate Analytical Development, Analytical Development, IDT Biologika
NGS allows rapid detection of unwanted adventitious viruses in pharmaceutical products. However, detection of viral contaminants by NGS is not capable of indicating viability or replication competence of the found virus. As conventional viability assays are based on long lasting cell culture, different approaches for rapid differentiation of replicating viral infections from inert viral sequences were developed to assess accelerated viral safety evaluation.

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

10:45 Breakout Discussion Groups

Breakout discussions provide an opportunity to discuss a focused topic with peers from around the world in an open, collegial setting. Select from the list of topics available and join the moderated discussion to share ideas, gain insights, establish collaborations or commiserate about persistent challenges. Please visit the breakout discussions page on the conference website for a complete listing of topics and descriptions.

IN-PERSON ONLY BREAKOUT: Formulation Development, Bioprocessing, and Manufacturing of Vaccines

Christopher P. Locher, PhD, Co-Founder & CEO, Biology, Versatope Therapeutics

- Continuous manufacturing
- Reducing costs for mRNA synthesis
- Formulations for thermostability

DEVELOPMENT, BIOPROCESSING, AND MANUFACTURE OF VACCINES (CONT.)

11:30 A Recombinant Influenza Vaccine Made from Membrane Vesicles

Christopher P. Locher, PhD, Co-Founder & CEO, Biology, Versatope Therapeutics

Since bEVs co-express a lipopolysaccharide (LPS), we have improved the safety and tolerance of the nano-vesicles by creating a genetically-modified cell line that does not induce high levels of pyrogenicity. We found that a bEV-expressing M2e protects against fatal H5N1 infection in ferrets as well as mice at nano-gram doses with two immunizations

12:00 pm Revisiting Membrane Chromatography and Absorbers Viral Vaccine Purification

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

- A lot of choices of membrane absorbers and fiber materials for virus purification compared to the past
- What is the selection criterion for a membrane adsorber and fiber material
- The ideal separation process for a viral vaccine

12:30 Enjoy Lunch on Your Own

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

ANALYTICAL TOOLS AND CHARACTERIZATION STRATEGIES FOR VACCINES

2:10 Chairperson's Remarks

Marina Kirkitadze, PhD, Head Bioprocess Support & PAT Platform, Analytical Sciences, Sanofi Pasteur

2:15 FEATURED PRESENTATION: Advances in Quality Control Standards for Polysaccharide Conjugate Vaccines

John Cipollo, PhD, Senior Principal Scientist and Team Lead, USP

Polysaccharide Conjugate vaccines are the most successful preventatives against bacterial disease and are composed of defined polysaccharides individually conjugated to carrier protein. This presentation will provide an update on standards and tools to support vaccine quality from raw materials through release testing. The recent revisions to General Chapter <198>, focused on NMR for bacterial polysaccharide for identity, and reference materials to support testing of quality attributes will be discussed.

2:45 Characterization of Vaccine Drug Product by Contact-Free Sensors

Marina Kirkitadze, PhD, Head Bioprocess Support & PAT Platform, Analytical Sciences, Sanofi Pasteur

The focus of the presentation is evaluation of wNMR as an emerging noninvasive analytical technology to characterize aluminum-adjuvanted vaccine formulations. In this work, wNMR and optical techniques such as laser diffraction and laser scattering were used to characterize vaccine formulations containing different antigen loads adsorbed onto AlPO4 adjuvant microparticles, including the fully dispersed state and the sedimentation process. The results of the study will be discussed.

3:15 Single-Particle Imaging to Quantitate Biophysical Properties of mRNA Lipid Nanoparticles and Engineer Improved Vaccines

Sabrina Leslie, PhD, Associate Professor, Physics and Astronomy Department, The University of British Columbia

We present quantitative single-particle imaging platform that enables simultaneous measurements of the size, mRNA-payload, and dynamic properties of vaccines in cell-like conditions. We investigate dependence of mRNA-lipid-nanoparticle structure and fusion dynamics on formulation, using commercially available formulations as a starting point. These measurements are made on confined, freely diffusing particles, and during reagent-exchange such as in response to solution pH, in order to emulate intracellular dynamics in a controlled setting.

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

4:30 Size Characterization of Vaccine Antigens: Ensemble vs. Single Particle Analysis Approach

Rahul Misra, PhD, Scientist, Biophysics and Process Analytical Technology, Sanofi

Single particle analysis approach involves TRPS technology which measures size of individual particles passing through a nanopore and claims to provide more accurate particle size distribution data within a sample. Particle concentration analysis is also based on single-particle measurements thereby ensuring highly accurate calculations for each size band compared to the ensemble-based approach. The study performs comparative analysis of size distribution of vaccine antigens to evaluate performance of these methods.

5:00 Microfluidic Electrophoresis-Based Detection and Characterization of dsRNA Contaminants in mRNA Vaccines

Anubhav Tripathi, PhD, Professor, Engineering & Medical Sciences, Brown University

We propose a dual dynamic staining high-throughput microfluidic electrophoresis analytical method for the detection and characterization of dsRNA contaminants in mRNA vaccines. With an mRNA maximum loading capacity of 13 ng/µL, we can detect dsRNA contaminants as low as 0.1-0.6% of the total concentration.

5:30 Close of Vaccine Development and Manufacturing Conference

WEDNESDAY, AUGUST 16

7:30 am Registration and Morning Coffee

CHALLENGES AND OPPORTUNITIES

7:55 Chairperson's Opening Remarks
Mohamed ElSayed, PhD, Executive Vice President & CTO, RVAC Medicines, Inc.

8:00 Challenges and Opportunities in mRNA-Based Therapies
Mohamed ElSayed, PhD, Executive Vice President & CTO, RVAC Medicines, Inc.

8:30 Public Perception of mRNA Therapies: The Patient Matters
Ben Locwin, Vice President, Project Solutions, Black Diamond
While over the past couple of decades, mRNA technology has advanced dramatically and provided one way out of the COVID-19 pandemic, at the same time public perception and favor has polarized, and strong and vocal holdouts have unequivocally affected general interest and uptake.

mRNA-BASED THERAPIES FOR ONCOLOGY AND OTHER INDICATIONS

9:00 Novel RNAs for Treating Cancer
Mark Kay, MD, PhD, Dennis Farrey Family Professor of Pediatrics and Genetics, Department of Pediatrics and Genetics, Stanford University
I will discuss a miRNA that has long been known to have anti-oncogenic properties and is derived from a long primary transcript, which we recently discovered has a separate tumor suppressor function. The role and anti-oncogenic mechanism by which this long non-coding RNA functions will be discussed. In addition, the therapeutic implications for therapeutic intervention will also be examined.

9:30 Selected Poster Presentation: Bench Scale to Clinical Trial Manufacturing: Overcoming Challenges in Scaling Up an mRNA Lipid Nanoparticle Vaccine
Nick Murphy, PhD, Scientist I, Biopharmaceutical Product Development, CSL Seqirus
Successful sa-mRNA encapsulation into lipid nanoparticles involves small-scale process development, followed by scale-up. We successfully scaled up from bench scale to clinical trial manufacturing scale. Challenges faced during scale-up were addressed, including increased processing time, effects of container materials, and foaming during tangential flow filtration. Investigation of peristaltic pumping on drug product also ensured consistent quality across scales. Our scale-up achieved reproducibility and met quality attributes of bulk drug product.

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

mRNA-BASED THERAPIES FOR ONCOLOGY (CONT.)

10:40 Next-Generation Self-Replicating RNA Vectors for Vaccines and Immunotherapies
Shigeki Miyake-Stoner, PhD, Director R&D & Head, Technology, Replicate Bioscience, Inc.
Self-replicating RNA (srRNA) technology has been enabled for vaccines, and requires lower doses than conventional mRNA, due to its ability to amplify *in situ*. Most srRNA approaches are derived from the same alphavirus backbone. Since viruses can differentially impact host cell mechanisms, we have explored alternate alphaviral vectors to assess their biological utility. We show that new synthetic srRNA vectors can enable us to build better next-generation therapies.

11:10 KEYNOTE PRESENTATION: miRNA-Based Logic Circuits Encoded on Self-Amplifying RNA for Highly Specific Cancer Cell Classification
Ron Weiss, PhD, Professor, Biological Engineering, Massachusetts Institute of Technology
We developed self-amplifying RNA and modified RNA platforms into vectors capable of carrying synthetic circuitry payloads that can provide a variety of desirable dynamics. We also encoded miRNA target sites on our RNA vectors to provide for highly specific cell type classification. We are using this technology to create next-generation cancer immunotherapy RNA vectors capable of activating therapeutic payloads discriminately in cancer cells.

11:40 Applying an mRNA Vaccine Platform and New Developments to Next-Generation Products
Nicole Schiavone, PhD, Principal Scientist, Pfizer Inc.
The SARS-CoV-2 virus continues to evolve which presents the need to adapt and broaden protection provided by mRNA vaccines. This talk will highlight the importance of robust analytical methods to enable rapid mRNA vaccine development. It will also feature the application of Pfizer's established mRNA analytical platform in combination with new developments to support next-generation products aimed at addressing the continuously changing viral landscape.

12:10 pm Enjoy Lunch on Your Own

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

CONSIDERATIONS FOR DEVELOPMENT OF mRNA VACCINES AND THERAPIES

1:25 Chairperson's Remarks
Jay Sarkar, PhD, Visiting Scholar, Stanford University

1:30 Developing Dendritic Cell-Targeted mRNA Vaccines
Daryl Drummond, CSO, Akagera Medicines, Inc.
Akagera is developing ligand-targeted lipid nanoparticles encapsulating mRNA as vaccines against some of the world's most intractable infectious diseases. Here we describe our efforts to develop novel ionizable lipids to improve endosomal escape and pair them with small molecule lipid ligands to increase their uptake and subsequent expression in dendritic cells. This increased efficiency has significant potential impact on manufacturing capacity, cost of goods, tolerability, and flexibility to incorporate multiple mRNAs.

2:00 Combinational RNA Therapies and Novel Strategies for Multi-Cargo Distribution
Jay Sarkar, PhD, Visiting Scholar, Stanford University
The value of the RNA modality comes from its nature as a precise, versatile, and real-time instruction coding. Its initial use cases utilize a single action coding – for instance, for the production of a viral antigen component. However, more advanced applications are rapidly developing around coding entire instruction sets for combinational logic and benefit. The promise of such approaches will be discussed along with novel strategies for implementing them.

2:30 Reduction of Double-Stranded mRNA

Lin Jin, Co-Founder, CATUG Biotechnology
Double-stranded RNA (dsRNA) is an undesired byproduct formed during *in vitro* transcription (IVT) and it is a major trigger of the immune pathway. For mRNA therapeutics that require large doses or repeated doses, control and removal of dsRNA is critical during CMC. Here, we are summarizing recent progress from CATUG Biotechnology for dsRNA control and removal in the upstream and downstream process development, as well as related analytical development.

2:45 Presentation to be Announced

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

PLENARY KEYNOTE: LEADING TO TOMORROW'S ADVANCES

3:50 Chairperson's Remarks
Ran Zheng, CEO, Landmark Bio

4:00 Current and Future Trends in Biomanufacturing of New Modalities
Konstantin B. Konstantinov, PhD, CTO, Ring Therapeutics
Using exosomes as an example, this presentation examines the current and future trends in biomanufacturing, and the technologies needed to manufacture emerging modalities at scale. Traditional biomanufacturing methods do not provide the industrialized, commercially scalable, highly efficient and reproducible manufacturing process essential for this new class of biotherapeutics—so we built it from the ground up.

4:30 The Digitalization of Biomanufacturing
Richard D. Braatz, PhD, Edwin R. Gilliland Professor, Chemical Engineering, Massachusetts Institute of Technology
A testbed is described for the end-to-end integrated and continuous manufacturing of monoclonal antibodies, which consists of parallel bioreactors, simulated moving bed chromatography systems, viral inactivation, and an autosampling system. Experimental results are compared with a digital twin. The increased consistency in the glycosylation profile of the monoclonal antibodies being produced is quantified when going from batch to semi-batch to perfusion mode, and when moving from start-up to quasi-steady conditions.

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

6:00 Close of Day

THURSDAY, AUGUST 17

7:30 am Registration and Morning Coffee

ANALYTICAL GUIDANCE AND TOOLS FOR mRNA VACCINES & THERAPIES

7:55 Chairperson's Remarks
Gaofei He, PhD, Principal Scientist, Pfizer Inc.

8:00 Biophysical Properties of mRNA
Alois Jungbauer, PhD, Professor & Head, Biotechnology, Institute of Bioprocess Science and Engineering, University of Natural Resources and Life Sciences (BOKU)
- The flexibility of mRNA is a challenge for chromatography separation
- Structural interconversion upon adsorption is more pronounced than observed with proteins
- How pure is pure for mRNA therapeutics and vaccines

8:30 mRNA Quality Assessment and USOP Guideline
John Cipollo, PhD, Senior Principal Scientist and Team Lead, USP
Following the rapid and successful deployment of mRNA vaccines during the COVID-19 pandemic, over 150 mRNA vaccines are in development, with further therapeutic applications in oncology, cardiovascular and genetic diseases. This presentation will provide an overview of USP updated guidelines based on stakeholder feedback and further assessment and qualification of analytical methods presented therein to address common quality attributes for mRNA products.

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

9:30 Breakout Discussion Groups
Breakout discussions provide an opportunity to discuss a focused topic with peers from around the world in an open, collegial setting. Select from the list of topics available and join the moderated discussion to share ideas, gain insights, establish collaborations or commiserate about persistent challenges. Please visit the breakout discussions page on the conference website for a complete listing of topics and descriptions.

IN-PERSON ONLY BREAKOUT: dsRNA in LNP-mRNA Vaccine Products
Christina Schier, PhD, Associate Principle Scientist, Merck & Co., Inc.
- How do we measure dsRNA? – Current analytical methods.
- What are the biological implications for dsRNA in vaccines?
- What might the non-emergency dsRNA regulatory landscape look like?

ANALYTICAL GUIDANCE AND TOOLS FOR mRNA VACCINES & THERAPIES (CONT.)

10:30 High-Throughput mRNA Integrity Analysis and mRNA Fragment Characterization
Gaofei He, PhD, Principal Scientist, Pfizer Inc.
Ribonucleic acids (RNAs) have recently shown success in the vaccine space and promise as candidates for new therapeutics. RNA integrity is a key critical quality attribute (CQA) for ensuring safety and efficacy, and has been assessed as a key quality attribute for optimization of the drug substance and drug product processes. This presentation discusses a capillary electrophoresis-based methodology to separate and resolve RNA of varied size and length.

11:00 mRNA Vaccines and Therapeutics: Development, Delivery, Safety, and Manufacturing
Trevor P. Castor, PhD, President & CEO, Aphios Corp.
Significant advances have been made over the last three decades in the development, delivery, safety, and manufacturing of mRNA constructs. The goal of this presentation is to review and address fundamentals and unit operations for mRNA functionality, stability, delivery, safety, and manufacturing. Within the context of this review, we will provide insights and novel technologies that may be helpful for furthering the development of mRNA vaccines and therapeutics.

11:30 High-Throughput Definition and Characterization of Cell-Based Assays for mRNA-LNP Vaccine Potency
Christina Schier, PhD, Associate Principle Scientist, Merck & Co., Inc.
mRNA-lipid nanoparticle vaccines provide many advantages inclusive of antigen specificity and rapid vaccine development. Characterization of this platform is relatively novel, yet requisite for vaccine production and licensing. Therefore, a cell-based assay was developed to quantify transgene protein expression efficiency and product potency in this platform. Appropriate assay factors were characterized, inclusive of monolayer morphology and mRNA-cassette protein expression kinetics, and effects of lipid nanoparticle properties were evaluated.

12:00 pm RNA Activation and Delivery
Nagy Habib, ChM, FRCS, Professor of Surgery, Imperial College London
Small activating RNAs (saRNA) are double-stranded 21 nucleotide RNA that can target promoters or enhancers leading to mRNA upregulation. MTL-CEBPA is an investigative drug that resulted from the conjugation of saRNA

CEBPA with NOV 340 liposomes that targets tumour-associated macrophages in order to alter favourably the tumour microenvironment. MTL-CEBPA has been administered safely in over 140 patients with advanced cancer with encouraging efficacy.

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

LNPs & NOVEL DELIVERY SYSTEMS: FORMULATION, ANALYSIS, PROCESS DEVELOPMENT, AND DELIVERY

1:05 **Chairperson's Remarks**
Amey Bandekar, PhD, Associate Director, Drug Product Development, Sanofi

1:10 **FEATURED PRESENTATION: Advances in Chemistry Made RNAi Therapeutics Possible**
Mano Manoharan, PhD, Distinguished Scientist & Senior Vice President, Innovation Chemistry, Alnylam Pharmaceuticals
For siRNAs, chemical modifications are necessary to regulate metabolic stability, potency, and safety. We have evaluated numerous chemical modifications These include backbone chiral phosphorothioates, glycol nucleic acids, altriol nucleic acids, gem 2'-deoxy-2'-α-F-2'-β-C-methyl, 5'-morpholino, and amino-oxy click chemistry (AOCC)-mediated conjugates. Furthermore, novel spatial architectures like circular siRNAs have also been evaluated. This presentation will summarize how chemistry has made possible the currently exciting world of RNAi therapeutics.

1:40 **Equilibrium and Stability Considerations in the Development and Manufacturing of Liposome and LNP Formulations**
Christoph Brandenbusch, PhD, Assistant Professor, Bioprocess Separations & Biologics Formulation Development, TU Dortmund University, Germany
Liposome and LNP formulations have evolved as highly potent drug delivery systems. Recent literature gives valuable insights in developing and manufacturing these formulations, with focus mainly set on delivering different liposome and LNP compositions/sizes. This presentation will give some insight into thermodynamic equilibrium considerations, such as long-term (size) stability of liposomes and LNPs at various temperature, as well as equilibrium radius considerations in manufacturing liposomes and LNPs.

2:10 **Considerations and Challenges in Early LNP Development for Non-Viral Gene Therapy**
Yuefei Shen, PhD, Principal Scientist, CMC Drug Product Development, Sanofi
LNP technology shows the ability to deliver nucleic acid therapeutics for non-viral gene therapy (NVGT). Compared to intramuscular vaccine delivery, intravenous (IV) delivery of an LNP formulation for gene therapy shows unique challenges. An LNP formulation for gene therapy via IV may require different lipid and formulation design. Here, we will discuss the considerations and challenges in early LNP development and impact on tissue targeting for NVGT.

2:40 **Networking Refreshment Break**

LNPs & NOVEL DELIVERY SYSTEMS (CONT.)

2:55 **Process Development Considerations for LNP Manufacturing**
Amey Bandekar, PhD, Associate Director, Drug Product Development, Sanofi
In the development of LNP-based drug product, the choice of manufacturing technology is one of the key factors for success. The choice of technology can have a significant impact on the biophysical properties, structural characteristics, colloidal stability, and efficacy of the LNP. This study describes the impact of different manufacturing parameters and the scale-up consideration to enable successful LNP drug product manufacturing.

3:25 **De-Risk mRNA Adduct Formation in Lipid Nanoparticle Formulation Intended for Glycogen Storage Disease Type-1a Using Analytical Tools**
Siddharth Bhoraskar, PhD, Scientist II, Beam Therapeutics
In this talk, we discuss de-risking mRNA adduct formation in lipid nanoparticle formulation intended for glycogen storage disease type-1a using analytical tools.

3:55 **Close of Summit**

STREAM #8
BIOMANUFACTURING & DIGITALIZATION

We have come to an age where smart biomanufacturing is transforming the bioprocessing landscape. From continuous/intensified processing to digitalization and big data, the industry is abuzz with new technologies and novel platforms that aim to revolutionize the biomanufacturing industry. CHI's Biomanufacturing & Digitalization stream presents strategies and opportunities for companies to enable next-generation biopharm production using intensified processes, real-time monitoring and control, digital twins and predictive modeling, as well as data-driven knowledge management tools driven by AI/ML, to deliver higher throughput, reduce cost, and produce consistently safe and high-quality products.

Conference Programs

AUGUST 14-15

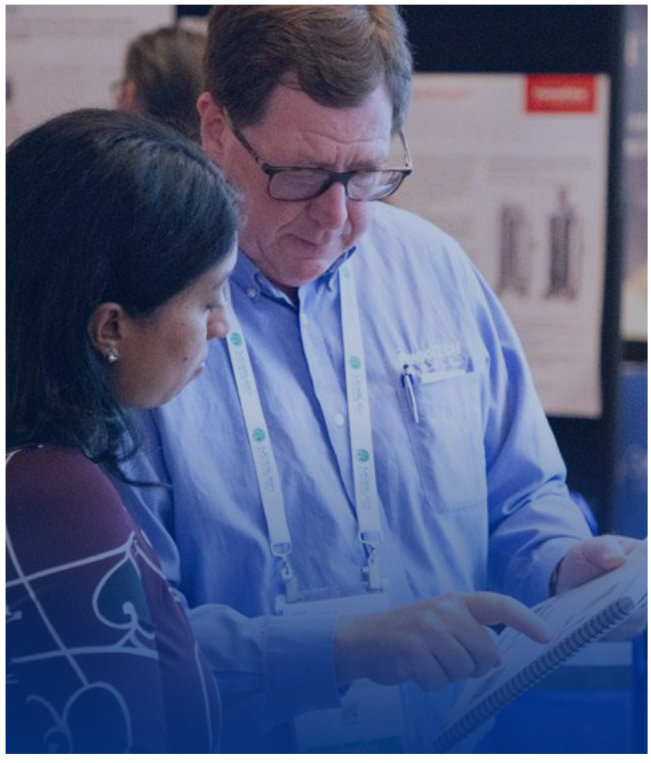
Intensified & Continuous Processing

View Program »

AUGUST 16-17

Smart Biomanufacturing & Digitalization

View Program »



MONDAY, AUGUST 14

8:00 am Registration and Morning Coffee

PERFUSION AND PROCESS INTENSIFICATION APPROACHES

9:55 Chairperson's Opening Remarks
Andrew Sinclair, MSc, CEng, FIChemE, FREng, President & Founder, BioPharm Services Ltd.

10:00 KEYNOTE PRESENTATION: Which Program Is Not Accelerated? Increasing Efficiencies in Process Development for Speed, Quality, and Safety
Gisela M. Ferreira, PhD, Director, AstraZeneca
The talk will exemplify some examples of business and scientific strategies that support program acceleration. Specifically, the exploration of column reuse for clinical production will be described as chromatography resins are largely underutilized during process development. While the study describes the use of three antibodies, the data supporting the proof of concept for resin reuse is demonstrated.

10:30 Implementation of N-1 Perfusion in Production of Biologics
Rok Brisar, PhD, Head of Tactical Manufacturing, Novartis
The production of recombinant proteins using mammalian cell culture has become an increasingly important process in the biopharmaceutical industry. The N-1 perfusion method has emerged as a promising approach for improving protein yields and reducing production costs. The aim of this discussion is to explore the advantages and disadvantages of this method compared to traditional batch and fed-batch processes.

11:00 Scaled-Down Models of N-1 Perfusion Enable Screening and Development of Intensified Upstream Fed-Batch Processes
Justin T. Huckaby, PhD, Process Development Scientist, Upstream Process Development, Shattuck Labs, Inc.
A high-seed density upstream fed-batch process was developed through the implementation of N-1 perfusion scale-down models in shake flasks and bench-scale bioreactors. A greater than 50% increase in harvest titer yields with comparable product quality was achieved as proof-of-concept for a bifunctional fusion protein using this intensified seed train process. By adding a few days to the seed train duration, we achieved significant gain in harvest titer while reducing COGS.

11:30 Talk Title to be Announced
Speaker to be Announced

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

12:30 Session Break

MAXIMIZING YIELD AND IMPROVING PERFUSION PERFORMANCE

12:50 Chairperson's Remarks
Gisela M. Ferreira, PhD, Director, AstraZeneca

12:55 Optimisation of Commercial-Scale Intensified Cell Culture
Andrew Sinclair, MSc, CEng, FIChemE, FREng, President & Founder, BioPharm Services Ltd.
Scaling up a bioprocess for manufacturing is complex and the impact of cell culture parameters influence manufacturing modalities. BioSolve Process incorporating Multi-objective Bayesian Optimization is used to analyse the complex design space to help identify optimal solutions. This case study identifies optimal configurations in terms of Fed Batch, Perfusion, or Intensified Fed Batch. The outcomes of the optimisation studies identify those factors that maximise economic, and sustainable benefits.

1:25 Maximizing Yield of Perfusion Cell Culture Processes: Evaluation and Scale-Up of Continuous Bleed Recycling
Christoph Herwig, PhD, Head of Research Area Bioprocess Technology, TU Vienna, Austria
Stable operation in mammalian perfusion cell cultures can be achieved using a bleed to remove excess biomass from the reactor. An acoustic settler was used to recycle the clarified portion to the bioreactor, which led to an increased overall productivity. There were no fouling/clogging risks as it is the case for many other cell retention devices. Operating conditions were compared to evaluate the yield improvement, leading to 2.25-fold yield improvement.

1:55 Talk Title to be Announced
Speaker to be Announced

2:25 Networking Refreshment Break

2:40 Evaluating Filter Chemistry as a Lever for Improving Perfusion Performance
Alexandria Triozzi, Engineer I, Biogen
Here, we evaluated three commercially available, commonly utilized membrane chemistries from multiple manufacturers and compared their sieving efficiency performance.

3:10 Evaluation of a Single-Use Small-Scale Continuous Centrifuge as a Scale-Down Model for Future Manufacturing Continuous Disc Centrifuge
Hirenkumar Panchal, Research Investigator, Incyte Corp.
Lack of a proper scale-down model makes the implementation of continuous centrifugation usually a try-and-error operation directly at large-scale. In this study, with the intention to develop a proper scale- down model, we side-by-side compared a single-use pilot-scale centrifuge to a bench-top centrifuge. Turbidity, lactate dehydrogenase (LDH), and host cell protein were all evaluated for comparison.

3:40 Session Break and Transition to Plenary Keynote Session

PLENARY KEYNOTE: SOLVING TODAY'S CHALLENGES

4:20 Chairperson's Remarks
Susan D'Costa, PhD, CTO, Alcyone Therapeutics, Inc.

4:30 Overcoming the Challenges of Bioprocesses: The Future of Biomanufacturing
Jerry A. Murry, PhD, Senior Vice President, Process Development, Amgen
Novel therapies and technologies are emerging to meet the needs of patients; however, the manufacturing of biopharmaceuticals remains a complex and challenging process. As demand for biopharmaceuticals

grows, the industry faces new challenges in terms of scalability, cost, and process robustness. The implementation of innovative technologies to improve process efficiency and the importance of process control and data analytics in ensuring process robustness are key levers to meet these challenges.

5:00 Commercializing Gene Therapies – The Combined Power of Patient Advocacy and Cost-Effective Manufacturing

Rachel Salzman, DVM, Founder, The Stop ALD Foundation & Executive Vice President, Portfolio, External Affairs & Development, Alcyone Therapeutics

This presentation will examine the development of an FDA-approved gene therapy where patient advocacy played a critical role resulting in the first-ever clinical use of a lentiviral vector. Although manufacturing continues to represent a significant challenge throughout the entire R&D journey, there are opportunities for advocacy and manufacturing communities to seek alignment and combine their collective powers to achieve the common goal of increasing patient access to transformative medicines.

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

6:30 Close of Day

TUESDAY, AUGUST 15

7:30 am Registration and Morning Coffee

DIGITALIZATION AND MECHANISTIC MODELING FOR CONTINUOUS PROCESSING

7:55 Chairperson's Remarks

Stefan R. Schmidt, MBA, PhD, COO & Head, Operations, BioAtrium AG

8:00 End-to-End Mechanistic Models of Integrated and Continuous Biomanufacturing Processes

Nehal Patel, Downstream Bioprocessing Practice Director, Digital Industries Process Automation Software, Siemens

Robert Taylor, PhD, Associate Scientist, Bioseparation Sciences, Merck Manufacturing Division

We will describe examples of how Siemens customers are building and applying dynamic end-to-end mechanistic models of integrated and continuous biomanufacturing processes (ICB) to determine the impact of expected disturbances, deviations, and uncertainties on product quality. We will show practical examples where these models can generate value by performing tasks that are not possible experimentally due to the prohibitive material requirements and complexity of building end-to-end processes in the lab.

8:30 Moving towards Advanced Automation of Continuous Processing

Sean Ruane, PhD, Senior Data Scientist, CPI

In the Integrated Continuous Biomanufacturing project, CPI and its partners have produced an end-to-end continuous mAb production and purification system that demonstrates the possibilities of Advanced Process Control, where CQAs are measured in real-time and controlled in a flexible process. The system also utilises a flexible digital architecture to enable model-based control while maintaining robustness, and a novel flow-balancing architecture to greatly simplify continuous processing.

9:00 A Spiking-Augmentation Method to Improve the Prediction Performance of FTIR-Titer Model on New Molecules

Yuxiang Zhao, PhD, Scientist, Bristol Myers Squibb Co.

Intensified and continuous processes require fast and robust methods for in-line titer monitoring. FTIR and chemometric-based multivariate modeling are promising tools for real time titer monitoring. This presentation demonstrates an adaptive modeling strategy: the model was initially built using a calibration set of available CB samples and then updated by augmenting spiking samples of the new molecules to the calibration set to improve the model robustness.

9:30 Talk Title to be Announced

Speaker to be Announced

GORE

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

10:45 Breakout Discussion Groups

Breakout discussions provide an opportunity to discuss a focused topic with peers from around the world in an open, collegial setting. Select from the list of topics available and join the moderated discussion to share ideas, gain insights, establish collaborations or commiserate about persistent challenges. Please visit the breakout discussions page on the conference website for a complete listing of topics and descriptions.

TOWARD COMMERCIAL-SCALE AND SUSTAINABLE BIOMANUFACTURING

11:30 Intensification Strategies: Moving from Lab-Scale to Clinical- and Commercial-Scale

Stefan R. Schmidt, MBA, PhD, COO & Head, Operations, BioAtrium AG

Processes can be intensified at all scales and at all dimensions. However, that requires implementing approaches to achieve "more, with less efforts, faster" already at the beginning. This presentation gives a comprehensive overview on strategies how to integrate process intensification through the whole product life cycle and when you switch scales and facilities. The opportunities from early development to continuous process improvements will be summarized in this talk.

12:00 pm Sustainable Biologics Manufacturing – Current State and Future Outlook

Sri Madabhushi, PhD, Associate Principal Scientist & Associate Director, Merck

Sustainability of biologics manufacturing processes is critical in ensuring the efficient production of these life-saving therapies in a resource constrained world. This presentation will provide an overview of the current state of biologics sustainability for different modalities and discuss the findings from process mass intensity (PMI) and life cycle assessments (LCA). The work highlights the need for a comprehensive metric(s) that will drive innovations in sustainability of biologics manufacturing.

12:30 Talk Title to be Announced

Speaker to be Announced

Wheeler Bio

1:00 Luncheon Presentation to be Announced

Speaker to be Announced

METTLER TOLEDO

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

DOWNSTREAM PROCESS INTENSIFICATION

2:10 Chairperson's Remarks
Philip Probert, PhD, Technology Lead, CPI, United Kingdom

2:15 Ultrafiltration of Adeno-Associated Virus Clarified Cell Lysate for Downstream Process Intensification
Christopher Yehl, PhD, Scientist, Downstream Process Development, Spark Therapeutics, Inc.
Affinity Chromatography operational time is directly related to affinity load volume. Implementing an ultrafiltration step to concentrate AAV Clarified Cell Lysate (CCL) prior to Affinity loading can reduce overall operational time, maintain product quality, reduce cost of goods, and simplify the manufacturing procedure. Three commercially available membranes were evaluated over a range of conditions to show proof of concept, reproducibility, scalability, maintained or improved product quality and high product recovery.

2:45 Process Characterization of Multi-Column ProteinA
Lauren D. Powers, Senior Scientist, Merck

3:15 Optimizing Continuous Chromatography through MPC and EKF: A Novel Approach to Address Resin Aging
Touraj Eslami, PhD, Automation Engineer, Downstream Processing, Institute of Bioprocess Science and Engineering, University of Natural Resources & Life Sciences
The aging of chromatography columns impacts process economics intensively, including productivity, resin utilization, and buffer consumption. Our online optimization approach employs a residence time gradient during the loading step to balance these demands. This approach can forecast optimal conditions to maximize productivity and resin utilization. Results showed a savings of up to 43% in buffer consumption and increased productivity and resin utilization beyond the feasible range with classic chromatography.

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

INTENSIFIED PROCESSES FOR NOVEL & EMERGING BIOLOGICS

4:30 De-Risking Transfer of a HEK293 Exosome Production Process across Multiple Single-Use Production Bioreactors and Manufacturing Sites
Sunaina Prabhu, Scientist II, Integrated Drug Substance Development, Codiak Biosciences
A well-executed technology transfer is critical for manufacturing success. Codiak BioSciences has produced engineered exosomes using fed-batch and perfusion GMP processes at several contract manufacturing organizations for multiple clinical programs. Transferring a scaled-up process to a new manufacturing facility with different single-use bioreactors, configurations and manufacturing equipment requires a thorough engineering characterization and process modifications. This presentation will highlight the challenges and effective mitigation strategies employed to ensure consistent performance.

5:00 PANEL DISCUSSION: Intensified Processing for Novel Modalities – mRNAs, AAVs, EVs, and More: Hype vs. Reality
Moderator: Philip Probert, PhD, Technology Lead, CPI, United Kingdom
Novel modalities have the unrealised potential to revolutionise the treatment of disease. Access to these therapies, however, is limited by the high cost of goods of these products related to scale-up and yield challenges. Process intensification provides a solution to these issues – in this panel the current state-of-the-art, limitations, and future perspectives will be discussed.
Panelists:
Sunaina Prabhu, Scientist II, Integrated Drug Substance Development, Codiak Biosciences
Christopher Yehl, PhD, Scientist, Downstream Process Development, Spark Therapeutics, Inc.

5:30 Close of Intensified & Continuous Processing Conference

WEDNESDAY, AUGUST 16

7:30 am Registration and Morning Coffee

KEYNOTE SESSION: DIGITAL INNOVATIONS DRIVING BIOTHERAPEUTICS DEVELOPMENT

7:55 Chairperson's Remarks
Jun Huang, PhD, Senior Director, Data Enablement and Analytics, Regeneron Pharmaceuticals
Cenk Undey, PhD, Vice President & Global Head, PTD Data & Digital, Roche/Genentech

8:00 Digital Innovation in Transforming Molecules of Today into the Medicines of Tomorrow
Cenk Undey, PhD, Vice President & Global Head, PTD Data & Digital, Roche/Genentech
We generate significant amount of data during development and manufacturing of biopharmaceutical therapeutic proteins. Managing data and applying predictive/prescriptive analytics including artificial intelligence and *in silico* modeling tools right from the start throughout the product development lifecycle into manufacturing is critical for seamless data flow, robust design, and accelerating the development activities. Digital innovation paired with digital mindset is a significant enabler bringing life-changing medicines to patients.

8:30 The Golden Thread: Digital Powered Seamless Therapeutic Translation and Industrialization
Shanti Chari, AVP, Digital Technologies and Innovation, Landmark Bio
From early discovery to preclinical, through clinical manufacturing, quality control to commercial manufacturing, the golden(digital) thread will connect all disparate data sources and provide the basis for a digital, paperless shop floor at Landmark Bio. The automation of internal and external tech transfer will be foundational for seamless therapeutic translation and expose more information across business systems, including those used by external partners.

9:00 Preparing for the Smart Factories of the Future: CDER's Journey
Thomas F. O'Connor, PhD, Deputy Director, Office of Pharmaceutical Quality, CDER, FDA
The advanced technologies and manufacturing approaches needed to enable smart factories may have the potential to deliver higher output, increased manufacturing safety, improved quality, better value, increased agility, additional flexibility, and reduced waste. The presentation will share CDER's experience engaging with manufacturers on digital technologies through the Emerging Technology Team. CDER's initiatives on evaluating the existing regulatory framework in the context of digital technologies and AI will also be discussed.

9:30 Talk Title to be Announced
Speaker to be Announced

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

AI/ML & DIGITALIZATION – IMPACT AND OUTLOOK FOR BIOLOGICS MANUFACTURING

10:40 Digitalization – Another Technology?
Oliver Hesse, Head, Biotech Data Science & Digitalization, Bayer U.S. LLC
Digitalization is one of the buzzwords that is on every organization's goals. The talk will discuss what digitalization is and what it is not.

11:10 Hybrid Model and Machine Learning Enable Efficient Knowledge Generation for Bioprocess Development of mAbs and New Modalities
Michael Sokolov, PhD, Lecturer, ETH Zurich
In this presentation, we show how advanced machine learning and hybrid modeling approaches can be exploited to significantly improve process understanding, performance, and automated operation as digital twins. All presentations will be centered on industrial implementation examples for mAb, cell & gene therapy, and mRNA processes with numerous big pharma and CDMO partners allowing to quantify efficiency gains in process development.

11:40 PANEL DISCUSSION: The Short-Term and Long-Term Outlook of AI-Centered Technologies in Biomanufacturing
Moderator: Michael Sokolov, PhD, Lecturer, ETH Zurich
- Which technologies are used today and how?
- What value is expected from these technologies?
- What are the main challenges to consistently drive value from data?
- How can business cases be defined leading to success stories?

Panelists:
Oliver Hesse, Head, Biotech Data Science & Digitalization, Bayer U.S. LLC
Jun Huang, PhD, Senior Director, Data Enablement and Analytics, Regeneron Pharmaceuticals
Thomas F. O'Connor, PhD, Deputy Director, Office of Pharmaceutical Quality, CDER, FDA
Cenk Undey, PhD, Vice President & Global Head, PTD Data & Digital, Roche/Genentech

12:10 pm Sponsored Presentation *(Opportunity Available)*

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

DATA INFRASTRUCTURE & MANAGEMENT

1:25 Chairperson's Remarks
Jun Huang, PhD, Senior Director, Data Enablement and Analytics, Regeneron Pharmaceuticals
Cenk Undey, PhD, Vice President & Global Head, PTD Data & Digital, Roche/Genentech

1:30 Advancing Digital and Data Infrastructure for Bioprocess Development
Jun Huang, PhD, Senior Director, Data Enablement and Analytics, Regeneron Pharmaceuticals

2:00 A Digital Transformation Journey in Process Development – Building Automated Data Flows, from Equipment to eLN to Advanced Analytics
Christian Airiau, PhD, Global Head, Data Sciences Biologics Development, Sanofi
Sanofi CMC/Process Development is implementing a comprehensive Digital Transformation program to improve our productivity and reduce our development timelines. We are deploying a standardized digital workflow

across our development sites globally (target: 2,000 users) and building automated data access to ensure scientists can document, visualize, and analyze their experimental work. We will discuss the progress, successes, and pain-points encountered by the development teams as we progress towards our digital ambition.

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

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

PLENARY KEYNOTE: LEADING TO TOMORROW'S ADVANCES

3:50 **Chairperson's Remarks**
Ran Zheng, PhD, CEO, Landmark Bio

4:00 **Current and Future Trends in Biomanufacturing of New Modalities**
Konstantin B. Konstantinov, PhD, CTO, Codiak Biosciences
Using exosomes as an example, this presentation examines the current and future trends in biomanufacturing, and the technologies needed to manufacture emerging modalities at scale. Traditional biomanufacturing methods do not provide the industrialized, commercially scalable, highly efficient and reproducible manufacturing process essential for this new class of biotherapeutics – so we built it from the ground up.

4:30 **The Digitalization of Biomanufacturing**
Richard D. Braatz, PhD, Edwin R. Gilliland Professor, Chemical Engineering, Massachusetts Institute of Technology
A testbed is described for the end-to-end integrated and continuous manufacturing of monoclonal antibodies, which consists of parallel bioreactors, simulated moving bed chromatography systems, viral inactivation, and an autosampling system. Experimental results are compared with a digital twin. The increased consistency in the glycosylation profile of the monoclonal antibodies being produced is quantified when going from batch to semi-batch to perfusion mode, and when moving from start-up to quasi-steady conditions.

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

6:00 **Close of Day**

THURSDAY, AUGUST 17

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

AUTOMATION, DIGITALIZATION, AND MODELING APPROACHES

7:55 **Chairperson's Remarks**
Christoph Herwig, PhD, Head of Research Area Bioprocess Technology, TU Vienna, Austria
Oliver Hesse, Head, Biotech Data Science & Digitalization, Bayer U.S. LLC

8:00 **Monitoring, Modeling, and Controlling the Basis for Automated and Autonomous Biomanufacturing**
Alois Jungbauer, PhD, Professor & Head, Biotechnology, Institute of Bioprocess Science and Engineering, University of Natural Resources and Life Sciences (BOKU)
The long-term vision of biomanufacturing is autonomous bioprocessing. To achieve this state, it is necessary to automate bioprocesses, which require sensors to control a manufacturing system. Currently, for a lot of quality/process parameters sensors are not available and soft sensors and self-learning algorithms must be applied. The state-of-the-art of monitoring and control of bioprocesses will be provided and to which extent integrated continuous biomanufacturing necessitates autonomous bioprocessing.

8:30 **Application of Physics-Informed Neural Networks in Real-Time Cell Culture Bioreactor Modeling**
Huiyi Cao, PhD, Senior Scientist, Pfizer Inc.
Shu Yang, PhD, Senior Scientist, Pfizer Inc.
Monitoring and control of viable cell density, metabolite concentration, and titer is critical for optimizing the development and manufacturing of cell cultures. A real-time bioreactor model has been developed using the novel modeling approach, physics-informed neural networks. This framework combines the power of AI with the robustness of mechanistic laws to reliably predict key product attributes. A proof-of-concept of this model has been implemented and tested in a bench-scale bioreactor.

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

9:30 **Breakout Discussion Groups**
Breakout discussions provide an opportunity to discuss a focused topic with peers from around the world in an open, collegial setting. Select from the list of topics available and join the moderated discussion to share ideas, gain insights, establish collaborations or commiserate about persistent challenges. Please visit the breakout discussions page on the conference website for a complete listing of topics and descriptions.

10:30 **Holistic Experimental Design and Deployment Strategies of Digital Twins for Accelerating Bioprocess Life-Cycling**
Christoph Herwig, PhD, Head of Research Area Bioprocess Technology, TU Vienna, Austria
Acceleration of commercialization of biologics, including the filing of a robust control strategy, is of utmost importance for biosimilars up to new modalities. Digital twins capture CMC knowledge and allow multiple deployments. We will show how end-to-end digital twins can help save 50% of experimental effort by incorporating drug substance specification when designing unit operations and how real-time application allows for prediction and control on process performance.

11:00 **Process Modeling for Ultrafiltration and Formulation**
Poonam Phalak, PhD, Associate Director & Process Modeling Lead, GSK
Model-based approaches in the biopharma industry have the potential to accelerate decision-making, optimize the product to market time, and reduce costs. *In silico* representation of manufacturing processes is becoming easier thanks to analytical tools, process modeling software, and machine learning algorithms. In this contribution, we demonstrate the use of model-based approaches for decision-making for ultrafiltration and formulation processes.

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

PAT (PROCESS ANALTYICAL TECHNOLOGY) AND APC (ADVANCED PROCESS CONTROLS)

1:05 Chairperson's Remarks

Antonio G. Cardillo, Senior Scientist, GSK Vaccines

Reza Kamyar, PhD, Director of AI and Advanced Control Solutions, Global Technology & Engineering, Pfizer Inc.

1:10 PAT Deployment in GSK Vaccine from R&D to Manufacturing

Antonio G. Cardillo, Senior Scientist, GSK Vaccines

Biopharmaceutical industry traditionally relies on pharmaceutical manufacturing practices to monitor processes and release products. The use of Process Analytical Technologies (PAT) can improve the process monitoring and control and at the same time increasing the process understanding and modelling capabilities. This talk examines the possible PAT application to vaccine processes using a phase- and technology-appropriate approach to reach a fully implemented in-line monitoring (ILM).

1:40 PAT and Automation for Robust Upstream Stem Cell Processing

Jens Traenkle, PhD, Head, PAT & Automation, Product Supply, Pharmaceuticals, Bayer AG

In this talk, we will present our recent developments in PAT and automation for transferring manual adherent iPSC cultivation processes to highly automated and fully closed cultivation systems. New PAT methods allow for rapid in-process testing of parameters specific to these new modalities and in combination with our robotized cell cultivation platform, the transition from a laboratory process to a closed and data-driven industrialized process is enabled.

2:10 Enabling Global Operations with Realtime Sensing: A Case Study in Digital Product Management

Cylia Chen, Director, Business Performance, Amgen

Brian McBreen, Director, Digital Product Management – Sensing, Amgen

In this session, you will learn about an important digital transformation effort at Amgen focused on delivering insights to senior leadership. The presentation will focus on a key use case for the global operations function (process development, manufacturing, quality, supply chain, etc.) along with the practice of digital product management. Learn how this approach improves agility as well as the nature of challenges that arise.

2:40 Networking Refreshment Break

2:55 Smart Process Analytics for the Prediction of Critical Quality Attributes in End-to-End Batch Manufacturing of Monoclonal Antibodies

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

For many modern biopharmaceutical processes, manufacturers develop data-driven models using data analytics/machine learning methods. The challenge is how to select the best methods for a specific dataset to construct the most accurate and reliable model. This presentation describes the application of smart process data analytics software to industrial end-to-end biomanufacturing datasets for monoclonal antibody production to automate the determination of the best DA/ML tools for model construction and process understanding.

3:25 Accelerated Raman Development for Implementation at Large-Scale

Kurtis Denny, Engineer I, Cell Culture Development, Biogen

Raman spectroscopy has been utilized for many different applications in cell culture bioprocesses, however, the adoption of PAT into commercial environments has been slow. A toolbox methodology will be shown with the aim of reducing time to implement Raman spectroscopy applications in cell culture processes.

3:55 PAT and Process Control for Cell and Gene Therapy Products

Sam Thompson, PhD, Analytical Development Scientist, Data Science, Cell & Gene Therapy Catapult

Developing a robust digital infrastructure that supports the dynamic aspect of cell and gene therapy development from research through to manufacture is an industry challenge. It is crucial to integrate equipment and software into a contained digital workflow for controlling and characterising a process, delivering finer parameter control and significant time-savings.

4:25 Close of Summit

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