August 15-18, 2022 • Boston, MA
Sheraton Boston & Virtual

The 14th Annual BIOPROCESSING SUMMIT

Solving Today's Challenges, Leading to Tomorrow's Advances

KEYNOTE AND FEATURED SPEAKERS

Nicholas Warne
PhD, Vice President, Pharmaceutical Research and Development, BioTherapeutics Pharmaceutical Sciences, Pfizer Inc.

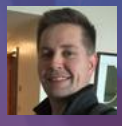
Hari Pujar
PhD, Operating Partner, Flagship Pioneering; COO, Tessera Therapeutics

David B. Volkin
PhD, Distinguished Professor, Pharmaceutical Chemistry, University of Kansas, Lawrence

Christine Carapito
PhD, Director, Research, University of Strasbourg

Elizabeth H. Scheideman
PhD, Staff Scientist, Cell Line Development, NIH NIAID

Jonathan L. Coffman
PhD, Senior Director, Bioprocess Technology & Engineering, AstraZeneca



Herbert A. Runnels
PhD, Global CMC AAV Analytical Sponsor, Gene Therapy, Pfizer Inc.

Sunitha Lakshminarayanan
Head & Executive Director, Cell Therapy Global Process Engineering, Bristol Myers Squibb Co.

Sandeep Yadav
PhD, Senior Director, Drug Product Formulation & Fill & Finish, Drug Product Formulation & Process Development, Sangamo Therapeutics

Nathalie Clément
PhD, CEO, Unicorn Consultations, LLC

Barbara Kraus
PhD, Head, Gene Therapy Process Development, Takeda

Jeff Blue
Executive Director, Vaccine Drug Product Development, Merck & Co., Inc.

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BioprocessingSummit.com

For more than 10 years,

The Bioprocessing Summit has been recognized as the premier forum for industry leaders to share the latest research in bioprocess R&D, scale-up, quality and analytics. As we navigate the new realities of conferences and gatherings, you can count on CHI's commitment to deliver quality content and facilitated networking opportunities to help achieve your research goals.

#CHIBioprocessingSummit

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Your Safety is Our Top Priority

To ensure maximum safety, CHI has instituted mandatory health and safety protocols for all attendees, exhibitors, speakers, and staff who attend in person. Attendees that cannot participate because of this policy, or due to travel restrictions, are encouraged to participate using our highly praised virtual event platform. Our virtual events are designed to provide you with an in-person experience at your convenience, anywhere, anytime. We are actively following news and recommendations around COVID-19 and the Omicron variant. These protocols are subject to change as we continue to learn more.

All in-person attendees must: Have a negative COVID-19 test result from an FDA-authorized over-the-counter antigen test within 24 hours prior to arriving at the event. You will be asked about your results at registration. CHI recommends all attendees: Have an updated COVID-19 vaccination and wear a mask in public spaces at the event.

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Plenary Keynote Sessions

Monday AUGUST 15

SOLVING TODAY'S CHALLENGES

4:20pm Plenary Introduction

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

4:30pm Lessons Learned from the Pandemic

Nicholas Warne, PhD, Vice President, Pharmaceutical Research and Development, BioTherapeutics, Pharmaceutical Sciences, Pfizer Inc.

5:00pm Advances in Vaccine Formulation and Stability

David B. Volkin, PhD, Distinguished Professor, Pharmaceutical Chemistry, University of Kansas, Lawrence

This presentation will provide an overview of analytical characterization and formulation development considerations for new vaccine candidates targeted for use in low- and middle-income countries (LMICs). Illustrative case studies with vaccine candidates (e.g., live-virus, adjuvanted recombinant protein) will highlight implementing state-of-the-art stability-indicating assays to enable development of stable formulations. Challenges with developing lower-cost formulations (e.g., multi-dose, combination, non-parenteral) to expand vaccine coverage in LMICs will also be discussed.

Wednesday AUGUST 17

LEADING TO TOMORROW'S ADVANCES

3:50pm Plenary Introduction

Nathalie Clément, PhD, CEO, Unicorn Consultations, LLC

4:00pm New Therapeutic Modalities and Moore's Law in Biomanufacturing

Hari Pujar, PhD, Operating Partner, Flagship Pioneering; COO, Tessera Therapeutics

These last two decades have seen the emergence of new therapeutic modalities beyond the traditional ones of small molecules and recombinant proteins. These new modalities, including recombinant proteins, have been essential in the rescuing of what seemed like an unsustainable investment path of our industry. Manufacturing technology advances have enabled the widespread distribution of small molecule medicines at very low cost, and biologics are following suit. As we have embarked on newer, more complex modalities, biomanufacturing has appeared to stumble. Viral vectors and cell therapy have been at the tip of the spear of this challenge. Low productivity, limited capacity, and complex operations came in the way of fully realizing the full biological potential of these modalities. Separately, we have seen the immense success of mRNA vaccines, enabled by unprecedented biomanufacturing feats, resulting in the distribution of billions of doses from a zero start. The talk will chronicle the advancements in biomanufacturing of different therapeutic modalities, drawing parallels to semiconductor chip manufacturing, and establishing the rightful and bright future of biomanufacturing.

4:30pm Cell and Gene Therapy (R)evolution

Mercedes Segura Gally, PhD, Vice President, Process Development, ElevateBio

The concept of gene therapy arose nearly half a century ago. Turning that concept into a therapeutic reality required years of scientific discovery, technological advances, and pioneering efforts, culminating in several regulatory approvals over the last decade. These success stories paved the road for a second wave of advanced therapies that leverage new technologies more recently made available in the cell and gene therapy toolbox. Compared with traditional biologics, cell and gene therapy products pose unique product characterization and manufacturing challenges. This presentation aims to summarize the progress made on cell and gene therapy drug development in recent years.

Event At-A-Glance

CONFERENCES	AUGUST 15-16	AUGUST 17-18
STREAM #1 **UPSTREAM PROCESSING**	Cell Culture & Cell Line	Bioproduction: Smart Biomanufacturing
STREAM #2 **DOWNSTREAM PROCESSING**	Continuous Processing	Purification & Recovery
STREAM #3 **GENE THERAPY**	Gene Therapy CMC & Analytics	Gene Therapy Manufacturing
STREAM #4 **CELL THERAPY**	Cell Therapy CMC & Analytics	Cell Therapy Manufacturing
STREAM #5 **ANALYTICAL & QUALITY**	Host Cell Proteins	Analytical Development
STREAM #6 **STABILITY & FORMULATION**	Stability & Impurities	Formulation and Delivery
STREAM #7 **VACCINES AND RNAs**	Vaccine Manufacturing	RNA and Genomic-Based Therapeutics

Training SEMINARS

By Cambridge Healthtech Institute

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.

TS1: Introduction to Bioprocessing

Instructors: *Sheila Magil, PhD, Vice President, CMC & Quality, Elevation Oncology*

Frank Riske, PhD, Managing Director, Industry Specialized Services, BDO

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 follows. 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 industry, including scientific, technical, business, marketing or support functions, who would benefit from a detailed overview of this field.

MONDAY, AUGUST 15 10:00 AM-3:30 PM,
TUESDAY, AUGUST 16 8:00 AM-1:00 PM

TS2: Introduction to Gene Therapy and Viral Vectors

Instructors: *Scott Cross, Senior Principal, Dark Horse Consulting Group*

Jacob Staudhammer, Senior Consultant, Dark Horse Consulting Group

Christina Fuentes, PhD, Consultant, Dark Horse Consulting Group

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.

TS3: Data Science and Digital Twins for the Product Life Cycle

Instructors: *Christoph Herwig, PhD, Head of Research Area Bioprocess Technology, TU Vienna, Austria*

Lukas Marschall, Principal Consultant, Korber

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 processes validation (Stage 1-3) remains unresolved in industrial practice. This is due to the necessity of using scale-down models, the cost-intensive setup of experiments, and the complexity due to the interactivity of a multitude of unit operations. The commonly accepted hypothesis is that sound data science and digital twin approaches will be a success factor in this endeavor.

WEDNESDAY, AUGUST 17 8:00 AM-3:00 PM,
THURSDAY, AUGUST 18 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 Chemistry Manufacturing and Controls (CMC) of Biotechnology Products

Instructor: *Kevin Zen, PhD, Executive Director, Chemistry Manufacturing and Controls, AnaptysBio, Inc.*

The chemistry manufacturing and controls (CMC) of biologics is 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 modality including recombinant proteins, monoclonal antibodies (Mab), 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 high 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 better cell culture media, better cell line engineering, single use bioreactors, continuous perfusion, etc. The next decade will see smart biomanufacturing coming to the forefront, incorporating omics technology, automation, PAT, miniaturization, process intensification, machine learning, digital twins, sensors integration and data analytics. The Upstream Processing stream will dive into the many exciting trends and innovations driving the next wave of upstream development.

Conference Programs

AUGUST 15-16

Optimizing Cell Line and Cell Culture Processes

View Program »

AUGUST 17-18

Bioproduction: Smart Biomanufacturing and Digitalization

View Program »

MONDAY, AUGUST 15

9:00 am Main Conference Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Constitution A

CELL LINE PROCESS IMPROVEMENTS

9:55 Chairperson's Opening Remarks
William C.W. Chen, MD, PhD, Research Scientist, Massachusetts Institute of Technology

10:00 KEYNOTE PRESENTATION: CLD Process Improvements to Support Rapid Response Initiatives
Elizabeth H. Scheideman, PhD, Staff Scientist, Cell Line Development, NIH NIAID
This talk will cover process improvements aimed at decreasing cell line development timelines and increasing throughput to allow rapid response to future pandemics.

10:30 Generation of CHO Cell Line Producing Afucosylated Antibodies Using a Novel Approach
Simon Joubert, PhD, Research Officer & Team Lead, Cell Line Development, National Research Council Canada
Antibodies missing core fucose show enhanced ADCC effector function and anti-tumor activity. Expression of an anti-alpha-(1,6)-fucosyltransferase (FUT8) intrabody engineered to reside in the cell can efficiently reduce FUT8 activity and therefore the core-fucosylation of an antibody. Cell engineering to inhibit directly and specifically FUT8 activity allows for the production of g/L levels of IgGs with strongly enhanced ADCC effector function, for which the level of fucosylation can be selected.

11:00 Cell Line Development and Plasmid Optimization to Improve AAV Transient Titers
Ping Liu, PhD, Senior Scientist & Head, Cell Line Development, REGENXBIO, Inc.
In this presentation, we will introduce the efforts at REGENXBIO to adapt our HEK293 host cell lines from adherent to suspension and further improve AAV productivity of cell lines by multiple cloning efforts. Furthermore, to improve AAV titers, we made a sequential modification of our helper plasmid. By combining the new cell lines and new helper plasmids, we increased our overall transient yield >20-fold.

11:30 New Solutions for Reducing Complexity and Increasing Automation in Stable Cell Line Development CYTENA A BICO COMPANY
Julian Riba, PhD, CEO, CYTENA
There is an ever-growing need to make the CLD process more efficient in order to keep up with the demand for better therapies. I will present the benefits of the UP.SIGHT, CYTENA's new single-cell cloning and plate imaging instrument that achieves a probability of clonality >99.99% using 3D Full Well Imagining. I will also introduce a new, automated work station for screening hundreds of clones without user interaction.

11:45 Sponsored Presentation *(Opportunity Available)*

12:00 pm Enjoy Lunch on Your Own

NOVEL APPROACHES FOR GLYCOSYLATION ANALYSIS AND OPTIMIZATION

1:20 Chairperson's Remarks
Susan Sharfstein, PhD, Professor, Nanobioscience, Nanoscale Science and Engineering, SUNY Polytechnic Institute

1:25 Flux Analysis of N-Linked Glycosylation in Chinese Hamster Ovary Cell Cultures
Rudiyanto Gunawan, PhD, Associate Professor, Chemical & Biological Engineering, SUNY Buffalo
N-linked glycosylation is a critical quality attribute of therapeutic monoclonal antibodies (mAbs). There exists naturally-occurring heterogeneity in mAb glycosylation produced by Chinese hamster ovary cell cultures. In this talk, I will describe Compartmental Glycosylation Flux Analysis, a model-driven data analysis for estimating intracellular glycosylation fluxes, and its application to CHO cell culture production of immunoglobulin-G. Insights into the controlling factors of N-glycosylation from the analysis will be discussed.

1:25 Sponsored Presentation *(Opportunity Available)*

1:55 Enabling Faster CQA Monitoring through Rapid Multi-Component at-Line Mass Spec Analytics 908devices
Nick Randall, Application Scientist, 908 Devices
Biologics production has a need for faster development cycles and intensified processes. Running multiple microbioreactors in parallel enables expedited optimization, and assays are required to achieve desired critical quality attributes and high productivity. While automated microbioreactors have enabled better scale-down models for bioprocess, low microbioreactor volumes limit the media available for daily extractions for metabolite and nutrient analysis. In addition, CQAs such as glycosylation and protein charge variants analysis can be time-consuming and require specialized skill set. We discuss a data-driven and accelerated process optimization approach leveraging tandem automated at-line microfluidic capillary electrophoresis mass spectrometry (CE-MS) analyzer

2:25 Networking Refreshment Break (Grand Ballroom Foyer)

2:40 Clone Selection and Bioprocess Manipulation for Optimization of Glycosylation of a Monoclonal Antibody Biosimilar
Susan Sharfstein, PhD, Professor, Nanobioscience, Nanoscale Science and Engineering, SUNY Polytechnic Institute
We developed a novel cell line selection technology, PTSelect, that we used to rapidly generate clones producing the monoclonal antibody, adalimumab. In addition to evaluating productivity and stability, we characterized the glycan profiles of the antibodies produced. Lower productivity clones had glycan profiles that more closely resembled the innovator drug. Medium and bioprocess variations were applied to improve the glycosylation profiles in higher productivity clones.

3:10 Towards Monitoring and Investigating Cell Culture Process Parameters and Glycosylation Using Digital Twin Modeling
Woo Ahn, PhD, Principal Scientist, Upstream, Global MSAT, DS, Sanofi
Root cause analysis for out-of-trend processes can be a challenge when there are confounding factors, multiparameter interactions, and missing data. *In silico* cell culture modeling by first principles is a promising methodology to identify root causes and reduce the evaluation time. Here, we present an example using digital twin modeling to investigate variation in metabolism and glycosylation across manufacturing sites, elucidate potential causes, and propose corrective actions.

3:40 Session Break and Transition to Plenary Keynote

ROOM LOCATION: Constitution A&B

PLENARY KEYNOTE: SOLVING TODAY'S CHALLENGES

4:20 Plenary Introduction
James Warren, PhD, Vice President, Pharmaceutical Development, Ultragenyx Pharmaceutical

4:30 Lessons Learned from the Pandemic: mRNA-LNP Vaccine Development
Nicholas Warne, PhD, Vice President, Pharmaceutical Research and Development, BioTherapeutics Pharmaceutical Sciences, Pfizer Inc.
The speed and scale of industry response to the COVID pandemic was unprecedented, ultimately leading to the availability of several vaccines in under a year. This presentation will discuss the approach taken by Pfizer, with their partner BioNTech, in the development, manufacture, and distribution of the vaccine drug product while reflecting on lessons that may, or may not, be applicable to future product development.

5:00 Advances in Vaccine Formulation and Stability
David B. Volkin, PhD, Distinguished Professor, Pharmaceutical Chemistry, University of Kansas, Lawrence
This presentation will provide an overview of analytical characterization and formulation development considerations for new vaccine candidates targeted for use in low- and middle-income countries (LMICs). Illustrative case studies with vaccine candidates (e.g., live-virus, adjuvanted recombinant protein) will highlight implementing state-of-the-art stability-indicating assays to enable development of stable formulations. Challenges with developing lower-cost formulations (e.g., multi-dose, combination, non-parenteral) to expand vaccine coverage in LMICs will also be discussed.

5:30 Welcome Reception in the Exhibit Hall with Poster Viewing (Grand Ballroom)

6:30 Close of Day

TUESDAY, AUGUST 16

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Constitution A

STABLE EXPRESSION OF COMPLEX BIOLOGICS

7:55 Chairperson's Remarks
Jean-Francois P. Hamel, PhD, Lecturer, Chemical Engineering, Massachusetts Institute of Technology

8:00 Accelerating HTP Stable Expression of Complex Biologics
Whitney Liu, PhD, Principal Scientist, Bristol Myers Squibb Co.
Here, we describe a novel rapid HTP transposon-based CHOZN CLD platform that produces high-titer stable pools and clones with desired product quality attributes. This new platform enables us to: (1) design and screen as many vectors and vector combinations as necessary for selecting the lead cell lines with high productivity and quality; (2) shorten expression timelines to 7 to 10 weeks (1 month shorter than conventional electroporation process).

8:30 Production of SARS-CoV-2 Soluble Trimeric Spike Proteins from Stable CHO Pools in Stirred Tank Bioreactors
Phuong Lan Pham, PhD, Research Officer, Human Health Therapeutics, National Research Council Canada
Using stable CHO pools, high expression levels up to 1 g/L of SARS-CoV-2 trimeric Spike Proteins can be obtained in fed-batch culture within 6 weeks post-transfection. The scalability and stability of stable pools expressing variants of concern (VOCs) have been assessed in 1L bioreactors. Our data also demonstrate process reproducibility, robustness, and versatility.

9:00 Development of Recombinase-Based Targeted Integration Systems for Production of Exogenous Proteins Using Transposon-Mediated Landing Pads
Nina B. Reese, Scientist, Cell Line Development, Just Evotec Biologics
Current methods for stably expressing biotherapeutics in CHO cells often rely on random or semi-random integration events which result in widely heterogeneous cell populations. In this study, we developed a targeted integration system that expresses recombinant proteins using transposon-mediated landing pads. By targeting predefined genomic locations that support high expression of exogenous proteins, several cell lines expressing different biotherapeutics can be established with a high degree of specificity and reproducibility.

9:30 BEYOND TITER: Identify Top Producers with Favorable Critical Quality Attributes within 5 Days of Cloning
BERKELEY LIGHTS
Aurora Fabry-Wood, Product Manager, Cell Line Development, Berkeley Lights, Inc
CHO cell line selection is a painful bottleneck in biotherapeutic development, particularly for complex molecules like bispecifics. The Opto™ CLD workflow on the Beacon® system accelerates early CLD by integrating high throughput cell sorting, cloning, culture, productivity, growth, and product quality assays into a single, 5-day automated process. Hear about capabilities of on-chip detection that pinpoints best clones early on.

10:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

10:45 Breakout Discussions
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: Considerations in Successful Scale-Up of Cell Culture Processes from Bench to GMP Production
Susan D. Jones, PhD, Chief of Technical Operations, Tourmaline Bio
- How representative are different small-scale process development models?
- Factors to incorporate in planning scale-up of cell culture processes
- Discuss selected case studies of successful and unsuccessful scale-up
- Do different CHO hosts have different responses and considerations during scale-up? What are some typical areas of concern for CHO cells?

11:30 Identification and Mitigation of Amino Acid Misincorporations during Cell Culture Development
Shanta Boddapati, PhD, Senior Scientist, Bioprocess Development, Seagen, Inc.

12:00 pm Synthetic Transcription Systems for Therapeutic Protein Production and Cell Engineering

William C.W. Chen, MD, PhD, Research Scientist, Massachusetts Institute of Technology

Mammalian protein expression often relies on a limited collection of natural promoters, which offer discrete expression levels and could be challenging to predict their yields in distinct cell types. Additionally, conventional strategies using transient expression or random genome integration with gene(s) of interest are laborious and time-consuming. To address those issues, we have developed versatile and scalable synthetic biology toolkits to transform mammalian protein expression and cell engineering.

12:30 CARON's Next-Generation CO2 Incubators with Antimicrobial Technology and Controlled Humidity for Cell Culture Applications

Jiten Pant, ME, PhD, Global Director, Research and Innovation, Caron Products

Microbial contamination is one of the most universal challenges in the R&D and manufacturing of cell culture-based biologics such as cell and gene therapy, and monoclonal antibodies. This costs a substantial amount of time, efforts and exorbitant amount of money. Researchers have estimated that mycoplasma had the potential to affect hundreds of millions of dollars of NIH-funded research (Tanabe et. al., 2020). The common methods to overcome microbial contamination such as radiation, elevated heating, the addition of antibiotics in cell media, and manual cleaning with antimicrobial agents all come with their challenges and are not a guaranteed way of preventing contamination.

This is where Caron's owned antimicrobial technology based on the hydrogen peroxide (H_2O_2) module can be a game changer. This patented technology covers several unique features, including rapid cycle management, a highly repeatable sensor-driven process, and a compact & effective sterilant catalyst system. Our research has shown a 12-log reduction in 2 hours at 37 degrees C resulting in water and oxygen as the end products avoiding any need for cleanup post the sterilization cycle. As a result, Caron's patented H_2O_2 technology has been an integral part of cell culture labs in the world's most reputed biotech and biopharma companies.

12:45 Protein Engineered Cas-CLOVER Yields 99% Gene Editing Efficiency and Ultra-Low Toxicity

Corey Brizzee, PhD, Strain Engineering Scientist, Yeast and Protein Engineering, Demeetra

Cas-CLOVER is a proprietary dimeric nuclease system comprising restriction endonuclease, Clo051. Using this enzyme for genome cutting makes it fully dimeric, giving it precise site-specificity. However, there is an unmet need to improve the efficiency of the Cas-CLOVER system, and reduce its cellular toxicity, which are discussed in this talk. Case studies in suspension CHO cells targeting key genes for yield selection and enhanced ADCC will be covered.

1:00 Luncheon Presentation: Using In-Silico Science to Accelerate Upstream Process Development

Tiffany McLeod, Life Science Market Manager, Product Strategy Data Analytics, Sartorius Stedim Biotech

Achieving a high-yielding process requires a combination of robust technologies and a masterful process design. In this presentation we are proud to present Cell Insights by Umetrics® Studio, the first self-service analytics application that combines data-driven information and mechanistic knowledge to capture the complexity of cell growth, metabolism, and productivity in-silico.

1:30 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

UPSTREAM PROCESSING AND MANUFACTURING PLATFORMS FOR EMERGING BIOLOGICS

2:10 Chairperson's Remarks

Philip Probert, PhD, Head of Technical, Biologics, CPI

2:15 Strategies and Considerations in the Development of a Manufacturing Platform for mRNA-Based Product

Philip Probert, PhD, Head of Technical, Biologics, CPI

The potential for mRNA-based products to treat previously untreatable diseases has created significant interest in how these products can be manufactured consistently and scalably, and the feasibility for a platform-based approach for manufacture of different mRNA-LNP products. This talk will discuss the different strategies being taken for development of manufacturing platforms for mRNA-based products and associated challenges, including data and reflections based on our own work at CPI.

2:45 Optimizing CHO Expression for Enhance TriTAC Product Quality

Bryan Lemon, PhD, Vice President, Protein Science, Harpoon Therapeutics

T cell engagers are protein therapeutics that tether T cells to surface antigens on tumor cells, leading to activation of those T cells and destruction of the tumor. The TriTAC (Tri-specific T cell Activating Construct) technology was designed to optimize therapeutic window by addressing half-life and stability limitations of pioneering bispecific T cell engagers. This presentation will highlight process development strategies influencing TriTAC product quality.

3:15 Development of Stable Cell Lines for AAV Production – From Transient Expression to PCL Generation

Karin Plante, Research Assoc, AAV Producer Cell Line Dev, Sanofi

The Genomic Medicine Unit (GMU) CMC group at Sanofi is dedicated to the establishment of best-in-class manufacturing platforms to support development of life-changing advanced cell and gene therapy products. This presentation will touch upon the technologies available to produce recombinant adeno-associated virus (AAV)-based viral vectors towards treatment of various disease and will feature Sanofi's PCL-based platform for production of gene therapy vectors.

3:45 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

4:30 Medium Feed Strategy Development for AAV Production through Understanding of Metabolism and Pathways

Sha Sha, PhD, Scientist II, Upstream Process Development, Ultragenyx

Adenovirus-associated virus (AAV) production could pose specific requirements for nutrients or function compounds to meet the need of viral production in host cells. In this study, we use RNA-Seq-based study and spent medium analysis to drive understanding of pathways involved in AAV production. We conducted experiments to optimize culture medium with the supplements chosen to potentially affect AAV production metabolisms.

5:00 X-RAP Suspension Cell Line and Its Platform: Vedere's Solution for rAAV Manufacturing

Xiaozhi Ren, PhD, Director, Cell Line & Viral Vector Process Development, Vedere Bio II

By using a *de novo* serum-free adaptation and single-cell cloning method, we successfully developed a HEK293 suspension cell line X-RAP for rAAV production. Compared with other commonly used HEK293 suspension cells, it demonstrates no cell aggregation and robust cell growth. By using half of the nutrients of the Thermo VPC's need in the bioreactor, X-RAP showed 1.8-fold higher genome titer and 4.5-fold higher packaging efficiency in purified rAAV.

5:30 Close of Optimizing Cell Line & Cell Culture Processes Conference

WEDNESDAY, AUGUST 17

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Constitution A

DIGITAL TRANSFORMATION AND INDUSTRY 5.0

7:55 Chairperson's Opening Remarks
Robert F. Dream, PhD, Managing Director, HDR Co. LLC

8:00 KEYNOTE PRESENTATION: Digital Transformation in BioPharma Manufacturing – Current Business, Organizational and Technological Challenges, and Opportunities
Michael Sokolov, PhD, Co-Founder and COO, DataHow AG, Lecturer, ETH Zurich
Pharma manufacturing is still mostly focusing on the exploration phase of digital technology. The major goal of this presentation is to identify major drivers and challenges for digitalization and digital transformation in pharma manufacturing. Three main perspectives, namely technology, organization and culture as well as management and business strategy will be highlighted. The results are based on extensive collaboration with several tensof pharma, biotech and CDMO companies.

8:30 The Factory of the Future
Robert F. Dream, PhD, Managing Director, HDR Co. LLC
New technologies and innovative thinking are profoundly changing biopharmaceutical drug production industry holistically, giving rise to "the factory of the future." What will the factory of the future with respect to its structure, technologies, processes, and drug product delivery to the patient be? What enablers will manufacturers need in terms of strategy, leadership, employee skills, IT infrastructure, regulatory oversight, and suppliers to make this a reality?

9:00 How Digital Twins Facilitate the Factories of Tomorrow: Current Obstacles and Solutions for the Biopharma Industry
Maximilian Krippl, PhD, Head of DSP Modeling, Novasign GmbH
Currently, "Industry 4.0" and "Digital Twins" are inflationary used words in the bioprocess industry, but not a single fully automated bioprocess for the production of biopharmaceuticals was filed so far. The talk will highlight the current limitations of digital twins but also demonstrate with several up- and downstream showcases the potential of bioprocess digital twins to accelerate process development and fully automated manufacturing.

9:30 Expression Cassette Optimization – A Contextually Aware Platform for Expression Vector Optimization and Manufacture CORNING
Harrison Brown, PhD, Director, Computational Biology, Expression Therapeutics
Transgene optimization often takes a "one size fits all" approach that uses the same optimization process irrespective of a transgene's desired expression profile. Rather, factors unique to each vector's use case should be considered during optimization. We have developed the Expression Cassette Optimization (ECO) platform as an AI-driven optimization tool that considers the gene transfer vector, target tissue, and real-world manufacturing constraints to output optimized sequences tailored to each transgene's desired expression profile. The ECO pipeline interfaces with our discovery-to-GMP pipeline to enable in-house production of high-titer vector by leveraging fixed bed bioreactor technology from the Corning Ascent platform.

10:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

ECONOMICS AND TECHNOLOGY CONSIDERATIONS IN DIGITALIZING BIOMANUFACTURING

10:40 Environmental and Economic Challenges of Bioprocessing
Alois Jungbauer, PhD, Professor & Head, Biotechnology, Institute of Bioprocess Science and Engineering, University of Natural Resources and Life Sciences (BOKU)
Two extreme production scenarios are applied in biopharmaceutical industry: a fully-disposable factory with the characteristics of full flexibility and speed or a large-scale fixed plant with high capacity. Time ahead solutions and ideas will be presented how new processes and environmental friendliness can be married for the benefit of patient, supply reliability, and economics.

11:10 Applying Capacitance as a Process Analytical Technology to Vaccine Production: Exploring Process Automation and Enriching Process Understanding
Thomas Randolph Blanda, Scientist, Vaccine Process Development, Merck & Co., Inc.
Live virus vaccine production often requires the use of adherent cell lines, which necessitates considerable hands-on manipulation and operator staffing. Quantifying key process attributes in real-time without sampling presents opportunities for process automation and the enrichment of process understanding. We intend to show the wealth of detail that can be extracted from a single bioreactor batch and how to apply that detail to process decision-making.

11:40 Panel Discussion: Digitalized Biomanufacturing Facility – Considerations for An Integrated Approach
Moderator: Scott Clark, Associate Director, Data Intelligence & Analytics – Global Biologics, Bristol Myers Squibb Co.
There has been an ever-growing convergence occurring in the industry as we find more and more ways to leverage the data generated during biologics development and manufacturing! It is critical to have an integrated strategy to develop the appropriate data context to effectively use this data. Please join this engaging panel discussion on best practices, standardization, and challenges in building robust strategies and approaches to leverage our data.
Panelists:
Michael Sokolov, PhD, Co-Founder and COO, DataHow AG, Lecturer, ETH Zurich
Maximilian Krippl, PhD, Head of DSP Modeling, Novasign GmbH
Liliana Montano Herrera, Scientist, Digitalization Development Biologicals, Boehringer Ingelheim Pharma GmbH & Co. KG

12:10 pm LUNCHEON PRESENTATION: Evaluating Real-Time Spectroscopic and Particle Measurements to Address Common TFF Processing Issues of High Concentration mAbs METTLER TOLEDO
Yi Zhang, PhD, Purification Process Science, AstraZeneca
Subcutaneous injection of monoclonal antibodies (mAbs) and the use of devices are becoming more common options for delivering therapeutics. In order to reduce the volume being administered, desired antibody (mAb) concentrations can range from 100 mg/mL to over 300 mg/mL. High mAb concentrations, in traditional TFF processing, are problematic due to challenges that include: inaccurate mAb concentration measurements due to high viscosity retentate solutions, unrepresentative samples, limited dynamic range of common UV instruments and potentially variable excipient

or buffer concentrations arising from asymmetric distribution across the TFF membrane. These challenges can further complicate downstream formulation, the control strategy of the step and exacerbate mAb stability issues at high concentrations.
This presentation will highlight the use of integrated, real-time PAT techniques designed to address these new processing risks. We will provide examples demonstrating the use of real-time infrared and Raman spectroscopy to make accurate protein concentration measurements and monitor buffers and excipients change regardless of viscosity and protein concentration. In addition, we will also illustrate the use of *in situ* particle measurements to detect the onset of micron-sized particles during up-concentration.

12:40 **Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)**

MACHINE LEARNING APPROACHES

1:25 **Chairperson's Remarks**
Michael Sokolov, PhD, Co-Founder and COO, DataHow AG, Lecturer, ETH Zurich

1:30 **Intelligent Bioprocessing: Harnessing the Potential of Machine Learning and *in silico* Mechanistic Modeling for Advancing "Quality by Design" in Biomanufacturing**
Meiyappan Lakshmanan, PhD, Associate Staff Scientist, Bioinformatics, Bioprocessing Technology Institute
Ensuring consistent high yields and product quality are key challenges in biomanufacturing. Critical process parameters (CPPs) such as media and feed compositions, pH, and temperature can significantly affect product critical quality attributes (CQAs). Machine learning (ML) offers immense potential in identifying relevant CPPs that effectively predict the CQAs. ML techniques can also be synergized with mechanistic models as 'hybrid/white box ML', thus enabling rational design and control of bioprocesses.

2:00 **Machine Learning and Scientific Methods: New Insights into Cultivation Processes**
Liliana Montano Herrera, Scientist, Digitalization Development Biologicals, Boehringer Ingelheim Pharma GmbH & Co. KG
In this talk, we will highlight certain applications of machine learning for the holistic modeling of bioprocesses. As a new approach, we will shed light on the role of recurrent neural networks (RNNs) in predicting upstream cultivation processes. We will discuss the basics as well as the scientific implications for interpreting the results.

2:30 **Accelerating Drug Development through Scalable and Intelligent Computing** rescale
Derek McCoy, VP, Sales, Rescale
Whether it's research for new drug discovery or ways of expediting time to market, many healthcare and life sciences companies are looking to cloud-based scientific computing to accelerate research and development. The applications are countless: from faster drug approvals to improved collaboration, from more rapid vaccine development to helping genomics companies meet strict compliance and security standards. The goal in every case: faster time to market, cost efficiency, performance enhancements, more automation, improved agility and collaboration, ongoing security and compliance, and most importantly the safety and efficacy of new treatments to improve patient outcomes.

2:45 **Talk Title to be Announced**
Speaker to be Announced

3:00 **Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)**

ROOM LOCATION: Constitution A&B

PLENARY KEYNOTE: LEADING TO TOMORROW'S ADVANCES

3:50 **Plenary Introduction**
Nathalie Clément, PhD, CEO, Unicorn Consultations, LLC

4:00 **New Therapeutic Modalities and Moore's Law in Biomanufacturing**
Hari Pujar, PhD, Operating Partner, Flagship Pioneering; COO, Tessera Therapeutics
As we have embarked on newer, more complex modalities, biomanufacturing has appeared to stumble. The talk will chronicle the advancements in biomanufacturing of different therapeutic modalities, drawing parallels to semiconductor chip manufacturing, and establishing the rightful and bright future of biomanufacturing.

4:30 **Cell and Gene Therapy (R)evolution**
Mercedes Segura Gally, PhD, Vice President, Process Development, ElevateBio
The concept of gene therapy arose nearly half a century ago. Turning that concept into a therapeutic reality required years of scientific discovery, technological advances, and pioneering efforts, culminating in several regulatory approvals. These success stories paved the road for a second wave of advanced therapies. Compared with traditional biologics, cell and gene therapy products pose unique challenges. This presentation aims to summarize the progress made in recent years.

5:00 **Networking Reception in the Exhibit Hall with Poster Viewing (Grand Ballroom)**

6:00 **Close of Day**

THURSDAY, AUGUST 18

7:30 am **Registration and Morning Coffee (Grand Ballroom Foyer)**

ROOM LOCATION: Constitution A

PREDICTIVE SCALE-DOWN MODELS AND DIGITAL TWIN APPLICATIONS

7:55 **Chairperson's Remarks**
Christoph Herwig, PhD, Head of Research Area Bioprocess Technology, TU Vienna, Austria

8:00 **Successful Transfer of Lab Results into Industrial Scale *via* Scale-Down Devices That Filter Robust Production Cells**
Ralf Takors, PhD, Professor & Director, Institute of Biochemical Engineering, University of Stuttgart
After discussing commonly applied scale-down technologies, the talk presents a newly developed single multi-compartment bioreactor (SCMB) that mimics large-scale mixing times and mechanical power input in a simple manner. A workflow is presented that enables the quantitative prediction of small-scale performance by using particularly-designed discs in a 3.5L stirred tank. The procedure could be successfully applied for aerated and non-aerated cultivation conditions of CHO cells.

8:30 Digital Twins Deployment for Efficient Product Life Cycling and Optimized Productivity

Christoph Herwig, PhD, Head of Research Area Bioprocess Technology, TU Vienna, Austria

Digital twins are perceived to be the core enabler for Pharma 4.0. So, if we have one, what is the benefit, where and how to deploy them? We show two applications: 1) The sound definition of the control strategy using integrated digital twins, which analyze the interactivities between unit operations. 2) The real-time deployment for direct optimization of specific productivity in the upstream process. Both increase productivity and smooth life-cycling.

9:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

9:15 Poster Award Presented in the Exhibit Hall c-LEcta

9:30 Smart Process Development – Digital Twins in Early-Stage Downstream Process Development

Felix Wittkopp, PhD, Senior Scientist, Bioprocess Research, Roche Diagnostics GmbH

Detailed process-understanding is required to ensure high-quality products throughout the product development lifecycle. An innovative approach to achieving this balance is the application of digital twins in form of mechanistic modeling. This presentation shows the implementation of a digital twin concept in early-stage development including efficient workflows for fast model calibration and model application.

10:00 CO-PRESENTATION: Digital Twin: A Downstream Bioprocess Application

Antonio G. Cardillo, Senior Scientist, GSK Vaccines

Loredana Vagaggini, Senior Product Owner, Vaccines R&D IT, GSK

In this talk, we will present the approach to implement digital twin for development and control of the bioprocess with a focus on purification application. The need and capability of modeling and PAT integration are evaluated for Chromatography and tangential filtration. Using the digital twin approach we will try to reduce off-line release testing by online analytical testing and modeling.

10:30 Integrating Lab Instruments, Data Platforms, and Apps in the Sciences Ganymede

Nathan Clark, Co-Founder, Ganymede Bio

We present best practices for integrating lab instruments, apps, and data platforms, and building software to automate wet labs. This is the basis for Ganymede.bio, a modern cloud-based platform for wet lab integration and automation. We cover cases of common workflows such as flow cytometry, bioreactors, sequencing data, and LIMS/ELN tools, and share what approaches we've seen to how biotech companies build their data stack for bioprocessing, process development, and beyond.

11:00 Breakout Discussions

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: Smart Manufacturing and Bioprocessing Excellence

Robert F. Dream, PhD, Managing Director, HDR Co. LLC

- The rapid adoption of intelligent manufacturing and Industry 4.0/Pharma 4.0
- How to integrate digital tools in bioprocessing design and optimization?
- How to achieve smart manufacturing and contamination control strategies?
- How to integrate flexible networks, cutting-edge innovation in a quality culture?
- Continuous process verification and manufacturing (CPV-M) in a new paradigm shift (US-FDA vs EU-EMA)
- AI-enabled proactive quality response – Is it applicable?

IN-PERSON ONLY BREAKOUT: Vaccine/Bioprocess Development Opportunities from Digitalization

Antonio G. Cardillo, Senior Scientist, GSK Vaccines

- Digital twin: definition, application, and how to create one
- Monitoring and controlling Vaccine bioprocesses the challenge and opportunity to create a reliable data pipeline
- Process Analytical Technologies (PAT), how and when its use generate a return of investment

12:00 pm Enjoy Lunch on Your Own

12:30 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

DATA-DRIVEN APPROACHES

1:05 Chairperson's Remarks

Ralf Takors, PhD, Professor & Director, Institute of Biochemical Engineering, University of Stuttgart

1:10 Data Science and Digital Technologies in Biopharmaceutical Manufacturing: From Data Sourcing to Model Deployment

Francisca F. Gouveia, PhD, Innovation Data & Digital Lead, MS&T, Novartis Pharma SAS

Reproducing large molecules reliably at an industrial scale requires manufacturing capabilities with a high degree of sophistication as well as continued investments during commercialization. The talk will cover key topics related to data sourcing, data analysis, and deployment of visualization tools to make the output of advanced data & digital technologies accessible to relevant stakeholders within each department.

1:40 Modeling & Simulation as One Key Element of Next-Generation CMC

Johannes Scheiblauer, Fellow, Automation & Control, Innovation & Technology Sciences, Pharmaceutical Sciences, R&D, Takeda

Several enabling technologies change the way we do pharmaceutical development: automation and digitalization, modeling and simulation. But especially the latter are not silver bullets, and it is important to have a closer look: why and when one might use them, which factors support a successful and sustainable implementation, and what it means for an organization to embrace these new ways of work.

2:10 Deep Learning and Other Data-Driven Approaches to Bioprocess Optimization

Jordon Gilmore, PhD, Asst Professor, Bioengineering, Clemson University

Data-driven approaches offer a vast opportunity to leverage the large, rich datasets developed from bioprocessing systems. These approaches include many statistical and artificial intelligence-based methods, but deep learning algorithms are perhaps best suited to the modeling, classification, prediction, and visualization of complex multivariate datasets resulting

from bioprocesses. This presentation features an overview of the current opportunities, state-of-art, and barriers to the utilization of these approaches in bioprocess optimization.

2:40 Refreshment Break in the Exhibit Hall & Last Chance for Poster Viewing (Grand Ballroom)

ROOM LOCATION: Back Bay D

PAT AND HIGH-THROUGHPUT ANALYTICS

3:10 Analytical Challenges at the 11th Hour

Christina Vessely, PhD, Senior Consultant, CMC Analytics & Formulation Development, Biologics Consulting Group, Inc.

This session will focus on ways to accelerate analytical development to support faster development timelines. However, activities often performed during Phase 3, as assay validation, elucidation of structure and characterization of impurities, can sometimes generate surprises. This presentation is intended to provide recovery and bridging strategies when deficiencies are discovered late in the development lifecycle.

3:40 Implementing High-Throughput Analytics for Accelerated Biologics Development

Sophia Levitskaya-Seaman, PhD, Process Analytics Group Leader, Biopharmaceutical Development, MacroGenics, Inc.

Monoclonal antibodies and novel bispecific DART molecules are being developed for a variety of indications including immuno-oncology. Sensitive, accurate, and high-throughput analytical techniques enable faster development timelines for therapeutic molecules. An overview of different approaches, methodologies, and supporting software for high-throughput process analytics and related challenges will be presented as case studies.

4:10 Implementation of a Fully Automated Walk-Up Residual DNA qPCR Workflow

Michele Shannon, Investigator, GlaxoSmithKline

Clearance of residual host DNA is an important part of the biopharmaceutical process as host DNA can pose a potential risk to the patient. Using the KingFisher Presto integrated into a Hamilton liquid handling system, we have automated the entire residual DNA assay from sample preparation through qPCR plate preparation, significantly reducing FTE labor and allowing for a walk-up system for quicker turnaround and high-throughput for residual DNA results.

4:40 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 and complex molecules coming down the pipeline. Companies are looking toward strategies such as intensified perfusion, continuous chromatography, membrane separations, integrated up- and down-stream processes, as well as the latest trend in digitalization - digital twins, PAT, modeling and real-time monitoring - to gain better understanding of process design for downstream processing of next-generation biologics. The Downstream Processing stream will bring you on a voyage to discover these innovations driving the next wave of downstream development.

Conference Programs

AUGUST 15-16

Integrated Continuous Processing

View Program »

AUGUST 17-18

Advances in Purification & Recovery

View Program »

MONDAY, AUGUST 15

9:00 am Main Conference Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Back Bay C

THE CASE FOR NOVEL MODALITIES

9:55 Chairperson's Opening Remarks
Aaron Noyes, PhD, Vice President, Integrated Drug Substance Development, Codiak Biosciences

10:00 Evaluation of Continuous Chromatography Strategies to Enrich for Full AAV Capsids and Reduce COGS
Chao Huang, PhD, Associate Director, Pharmaceutical Development, Ultragenyx Pharmaceutical
The empty full capsid separation for recombinant adeno-associated virus (rAAV) products is a hot topic in the gene therapy community. By adopting weak portioning chromatography (WPC), the full-AAV percentage was improved in AEX eluate. By harnessing the power of multi-column chromatography (MCC) technology, the cost of goods (COGs) reduction was further decreased. Finally, the AEX-WPC-MCC operation demonstrated its superiority in both full-AAV percentage and gene of interest (GOI) recovery.

10:30 Evaluation of a Continuous Live Virus Vaccine Platform: Can It Be Done and What Would the System Look Like?
David Hesley, Scientist, Vaccine Process Research & Development, Merck & Co., Inc.
Continuous cell expansion of adherent cell lines could save considerable time and money if it can successfully be integrated into bioprocessing. We have demonstrated that a single N-1 reactor growing anchorage dependent cells on microcarriers is able to run for weeks, supplying adequate cell mass for multiple iterations of an LVV production process. This has the potential to accelerate processing time, scale back equipment requirements, and limit workforce demand.

11:00 Evaluation of the Use of Continuous Chromatography for Purification of Antisense Oligonucleotides
Armin Delavari, PhD, Scientist II, Technical Development, Biogen
With large potential demand for antisense oligonucleotides on the horizon, significant increases in downstream throughput are becoming extremely important. Continuous chromatography approaches, including CaptureSMB and Multicolumn Countercurrent Solvent Gradient Purification (MCSGP), can provide the throughput needed, while also maintaining desired purity. Utilizing two columns in a continuous mode for aqueous purification of antisense oligonucleotides demonstrated a near four-fold increase in productivity for the HIC step of a model process.

11:30 Combining Data-Based AI Optimization and Automated PAT Analysis to Enable Continuous Biomanufacturing with Quartic.ai
Bryan Pope, Solutions Engineer, Quartic.ai
The status quo in operational software for manufacturing is not designed to enable continuous manufacturing. Capturing data for the entire product lifecycle, building a data-based AI model and/or digital twin, capturing inline monitoring data, and then being able to quickly compute process health requires multiple, expensive, legacy software packages that do not inherently interoperate. Continuous biomanufacturing requires integrated software that leverages the latest technologies to do all the above; to quickly and accurately assess past, current, and future states; and to provide prescriptive recommendations or direct control to keep biomanufacturing processes running optimally.

12:00 pm Enjoy Lunch on Your Own

KEYNOTE SESSION

12:50 Chairperson's Remarks
Stefan R. Schmidt, MBA, PhD, COO & Head, Operations, BioAtrium AG

12:55 KEYNOTE PRESENTATION: A Truly Continuous Counter-Current Downstream
Jonathan L. Coffman, PhD, Senior Director, Bioprocess Technology & Engineering, AstraZeneca
We propose a truly continuous downstream without chromatography, based on tangential flow filtration. We have achieved a proof-of-concept for each stage of the mAb downstream. The downstream achieves acceptable levels of HCP and DNA, as well as reduces the Process Mass Intensity (PMI) from 3000 kg water per kg drug to below 1000. As a result of the decreased water use, the purification equipment is significantly smaller.

1:25 KEYNOTE PRESENTATION: Towards End-to-End Continuous Biomanufacturing for Exosomes and Bionanoparticles
Aaron Noyes, PhD, Vice President, Integrated Drug Substance Development, Codiak Biosciences
The supply of advanced medicinal therapies such as viral vectors and exosomes has been constrained by inadequate process and production technology. From its inception, Codiak has focused on debottlenecking the production of exosomes to enable ample clinical supply while reducing cost of goods. Employing case studies from Codiak, I will describe the evolution of exosome manufacturing technology from batch to continuous and highlight further opportunities for E2E continuous bioprocessing.

1:55 Small Molecules, Big Insights: Leveraging Metabolomic Solutions for Systems Optimization of Models during Bioprocessing-Focused DoE, QbD, and PAT
Rangaprasad (Ranga) Sarangarajan, PhD, CSO, Metabolon
The rapid growth and development of new therapeutic modalities from large molecules to cellular therapies have intensified pressure to increase processing throughput while decreasing cost and turnaround time. Multi-omic technologies including genes and metabolomics are critical to understanding the biological basis of new process development. Metabolomics is unique in providing systems-wide small molecule-derived biochemical insights for unbiased data-driven approaches for bioprocessing, from initial designs to scaling-associated quality. Optimizing bioproduction across the product development lifecycle using metabolomics will be discussed with specific case studies on optimization of cell bioprocess, data-driven DoE optimization, and integration with multi-omic technology for strain engineering.

2:25 Networking Refreshment Break (Grand Ballroom Foyer)

PATH TO CONTINUOUS BIOMANUFACTURING

2:40 Intensification Strategies: The Path to Continuous Processing
Stefan R. Schmidt, MBA, PhD, COO & Head, Operations, BioAtrium AG
Continuous processing is the holy grail for many industries and became popular for bioprocessing in the last decade, too. Intensification is a prerequisite to enable a step-wise transformation toward that goal. This presentation gives a comprehensive overview of strategies where and how to implement process intensification, quantifies the benefits like plant occupancy time, and optimizing capacity based on successful examples and case studies.

3:10 Process Intensification Measuring the Performance and Sustainability
Andrew Sinclair, President & Founder, BioPharm Services Ltd., United Kingdom
Understanding the impact of process intensification options in terms of sustainability and business efficiency. The latest process models evaluate facility efficiency (doses per unit volume of cleanroom), PMI, and total energy efficiency. Pre-release versions were used by Process Intensification team in NIIMBL to support sustainability assessments. In this talk, comparisons are made between standard fed-batch processes and intensified process options that include perfusion and continuous downstream operations.

3:40 Session Break and Transition to Plenary Keynote

ROOM LOCATION: Constitution A&B

PLENARY KEYNOTE: SOLVING TODAY'S CHALLENGES

4:20 Plenary Introduction
James Warren, PhD, Vice President, Pharmaceutical Development, Ultragenyx Pharmaceutical

4:30 Lessons Learned from the Pandemic: mRNA-LNP Vaccine Development
Nicholas Warne, PhD, Vice President, Pharmaceutical Research and Development, BioTherapeutics Pharmaceutical Sciences, Pfizer Inc.
The speed and scale of industry response to the COVID pandemic was unprecedented, ultimately leading to the availability of several vaccines in under a year. This presentation will discuss the approach taken by Pfizer, with their partner BioNTech, in the development, manufacture, and distribution of the vaccine drug product while reflecting on lessons that may, or may not, be applicable to future product development.

5:00 Advances in Vaccine Formulation and Stability
David B. Volkin, PhD, Distinguished Professor, Pharmaceutical Chemistry, University of Kansas, Lawrence
This presentation will provide an overview of analytical characterization and formulation development considerations for new vaccine candidates targeted for use in low- and middle-income countries (LMICs). Illustrative case studies with vaccine candidates (e.g., live-virus, adjuvanted recombinant protein) will highlight implementing state-of-the-art stability-indicating assays to enable development of stable formulations. Challenges with developing lower-cost formulations (e.g., multi-dose, combination, non-parenteral) to expand vaccine coverage in LMICs will also be discussed.

5:30 Welcome Reception in the Exhibit Hall with Poster Viewing (Grand Ballroom)

6:30 Close of Day

TUESDAY, AUGUST 16

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Back Bay C

PAT AND PREDICTIVE CONTROL

7:55 Chairperson's Remarks
Laura Crowell, PhD, Director, Research & Development, Sunflower Therapeutics PBC

8:00 Examining PAT Tools and Sensors for Continuous CHO Bioreactor Cultures
Casey L. Kohnhorst, MS, Contract Scientist, FDA/CDER/OPQ/OBP
Continuous biomanufacturing can intensify existing batch processes, potentially achieving greater efficiencies with a smaller footprint. However, successful execution of continuous biomanufacturing can be more complex than traditional batch processes because it requires maintaining a steady-state operation. Thus, continuous biomanufacturing processes may benefit from robust in-line PAT for real-time monitoring and control of culture state and product quality. This talk will explore current PAT for use in continuous bioreactor cultures.

8:30 Considerations on PAT for Continuous Biomanufacturing
Antonio R. Moreira, PhD, Vice Provost, Academic Affairs & Advanced Technology Center, University of Maryland, Baltimore County
Continuous Manufacturing (CM) strategies for the production of pharmaceutical drug substance and drug product are receiving increasing attention from manufacturing companies. Several small molecule products for which at least some CM steps have been implemented have recently received approval by regulatory authorities. This presentation will discuss the opportunities for CM in large molecule manufacturing, with a focus on the critical role that PAT implementation will play for these processes.

9:00 Development Approach for an Integrated Biologics Process Using a Digital Twin Supported Predictive Control Architecture
Martin Purtscher, PhD, Senior Fellow, Biologics Process Development, Takeda
Recent approaches in the biopharmaceutical industry are focusing on process intensification and digital transformation. Some of these new capabilities have the potential to significantly change process development. A showcase for a continuous/connected process and the use of a predictive control architecture utilizing digital twins will be presented. Some specific early benefits for process development, optimization, and control will be highlighted – as well as a forecast of future directions.

9:30 Requirements and Qualification of Single-Use Sensors for Continuous Bioprocessing
Nick Troise, R&D Engineer II, PendoTECH
Interest in continuous biomanufacturing has recently increased due to the potential for a more efficient process with economical advantages. However, there are new challenges to be solved before these processes are implemented in GMP environments. The implementation of PAT, especially with single-use sensors, is critical for continuous bioprocessing. This

presentation will review the requirements for utilizing single-use sensors in a continuous process using PendoTECH sensors as a case study for qualification.

10:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

10:45 Breakout Discussions

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: Process Intensification – When and Where Is It Appropriate?

Ricardo J.S. Silva, PhD, Senior Scientist, Downstream Process Development, Animal Cell Technology, iBET Instituto de Biologia Experimental Tecnologica

- Lessons learned from mAbs, can we use them for gene therapy products?
- What tools are out there to enable process integration? What are the current needs for USP, DSP, and process control?
- New opportunities and challenges along the road with new modalities

IN-PERSON ONLY BREAKOUT: PAT and Process Intensification – Finding the Tools to De-Risk the Process

Jennifer Zhang, Senior Engineer, Biogen

- In-line, at-line, off-line: What is the right fit, and where in the process?
- How to control/monitor bioreactor excursions for material diversion – characterizing and understanding residence time distribution to minimize impact on productivity
- Real-time release to alleviate analytical strain and sub-batching strategies

FROM BENCH TO SCALE-UP

11:30 Bench to Scale-Up of a Next-Gen High-Capacity Protein A Resin for Continuous Manufacturing

Maria Znidarsic, Engineer II, Downstream Development, Biogen

We will present development activities associated with the selection of a high-capacity Protein A resin for a biologics continuous manufacturing process. The work discusses screening of various resins, followed by optimization with considerations for continuous manufacturing. We will include data generated for resin lifetime under different regeneration conditions, as well as impact of bioburden control strategy on resin stability. The optimal conditions were implemented at 500L scale using continuous manufacturing.

12:00 pm POSTER HIGHLIGHT: Implementing Multi-Column Chromatography for Batch Mode Commercial Product

Yuanpu Wang, Manager, MS&T, Bristol Myers Squibb Co.

The commercialized batch-mode process is converted into a continuous MCC process on the laboratory scale. The steps of the MCC development, the required data packages, and the challenges and technical difficulties are illustrated in this presentation.

12:30 Enjoy Lunch on Your Own

1:30 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

INTEGRATED, AUTOMATED, AND INTENSIFIED PROCESSING

2:10 Chairperson's Remarks

Antonio R. Moreira, PhD, Vice Provost, Academic Affairs & Advanced Technology Center, University of Maryland, Baltimore County

2:15 N-1 Perfusion at Ambr250 Scale

Jared Franklin, Principal Research Associate, Medium & Bioprocess Technologies, Sanofi US

N-1 perfusion is one strategy for intensifying fed-batch processes. This talk will focus on the intensification of a fed-batch process using an ambr250HT to run N-1 perfusion cultures and inoculate fed-batch vessels from them.

2:45 Integration and Intensification of Biomanufacturing Processes Using Straight-Through Chromatography

Laura Crowell, PhD, Director, Research & Development, Sunflower Therapeutics PBC

Accessibility and affordability of biologics globally is poor. Significant changes in development, manufacturing, and distribution are required for the broad use of life-saving biologics in low- and middle-income countries. Disruptive innovations that could improve global access, such as small footprint biomanufacturing, are enabled by integrated, intensified, and automated processes. Here, we discuss the development and optimization of straight-through purification processes and demonstrate their utility in small footprint, automated, end-to-end manufacturing.

3:15 Downstream Process Intensification Solutions for Gene Therapy Products

Ricardo J.S. Silva, PhD, Senior Scientist, Downstream Process Development, Animal Cell Technology, iBET Instituto de Biologia Experimental Tecnologica

Process intensification has already been demonstrated as a tool for increasing the productivity of monoclonals. Nevertheless, several challenges remain in what concerns virus manufacturing. In this talk, we will highlight perfusion, periodic counter-current chromatography, and continuous filtration operations as enabling tools for a streamlined AAV manufacturing process, focusing on the caveats and pitfalls of the process development journey.

3:45 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

4:30 High-Throughput Solutions for an Intensified Continuous Process

Jennifer Pulkowski, Scientist, Upstream Process Development, Pfizer Inc.

The upstream process development team at Pfizer has developed a highly productive continuous bioreactor process. However, with the implementation of perfusion, traditional bench-scale models become highly labor intensive with a lowered throughput that makes practical process development and characterization challenging. An automated and high-throughput solution for efficient development of an intensified continuous process in the ambr250 HT perfusion system is presented.

5:00 **Design and Construction of a Truly Continuous and Automated Process Skid for the Production and Purification of a Monoclonal Antibody**

Bernhard Sissolak, PhD, Project Manager R&D, Innovation Management, Bilfinger Life Science GmbH

This work demonstrates a showcase of a truly continuous and automated process for the production and purification of a monoclonal antibody. It highlights, both, the potential of using protein precipitation as a capture step, and the issues and challenges in designing and constructing of such a process skid.

5:30 **Close of Integrated Continuous Processing Conference**

WEDNESDAY, AUGUST 17

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Back Bay C

CHALLENGING FORMATS AND EMERGING MODALITIES

7:55 Chairperson's Opening Remarks
David W. Wood, PhD, Professor, Chemical & Biomolecular Engineering, The Ohio State University

8:00 KEYNOTE PRESENTATION: A High-Throughput Method for the Purification of Secreted His-Tagged Proteins
Maria Lorenzo, Principal Scientific Researcher, Protein Chemistry, Genentech, Inc.
Adopting automation technologies facilitates the scalable and consistent production of proteins ranging from antibodies to various drug targets. I will discuss the development of a platform for the purification of secreted His-tagged proteins.

8:30 Early Downstream of a Multi-Armed Bispecific
Ambrose J. Williams, PhD, Scientist, Tech Development, Genentech, Inc.
Discussed is our strategy for assessing and developing a downstream process for complex IgGs. With a focus on product-related variants, quantitating and eliminating chain-mispaired species such as homodimers is a priority. Our approach leverages high-throughput screening and orthogonal analytics, and examples are presented from a few case studies.

9:00 Challenges and Adventures in Purification Development for a Novel Diabody
Jian Ren, PhD, Principal Scientist, AbbVie
A novel diabody-cytokine fusion protein presented unique impurity and stability challenges. To address these issues, purification process was developed via a tiered approach which consists of initial device/condition screening, end-to-end process confirmation, and key step robustness evaluation. The final process achieved 20-fold reduction in aggregate and nearly 6-log reduction in HCP. This exploration enabled high-quality purification of the novel diabody and expanded the purification development toolbox with new technologies.

9:30 Robust HCP and Viral Clearance Using a Novel Single-Use AEX Technology 3M Science. Applied to Life.
David Chau, PhD, Global Bioprocess Application Specialist, Biopharmaceutical Purification, Separation and Purification Sciences Division, 3m
In this study, we explored how a new downstream AEX single-use technology adds flexibility to a traditional downstream unit operation. This novel technology combines a guanidinium-functionalized membrane with Q-functionalized fibers to enhance process flexibility. Four feed streams were evaluated for HCP clearance, DNA reduction, and viral clearance at different buffer and salt conditions. These results conclude that it can be applied across multiple modalities, simplifying the downstream development and manufacturing process.

10:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

10:40 High Purity and Throughput Isolation of Exosomes and Other Vector Species *via* Capillary-Channeled Fiber Chromatography
R. Kenneth Marcus, PhD, Professor, Chemistry, Biosystems Research Complex, Clemson University
A bioprocess chromatographer once suggested that "new therapeutic modalities will require new modes of separation." To this end, this laboratory has developed a novel family of polymer fiber stationary phases to affect the separation of exosomes and viruses using a hydrophobic interaction chromatography elution scheme. The platform technology is operable from the microliter to tens of milliliter scale, with scale-up currently underway. Challenges, accomplishments, and paths forward will be presented.

11:10 The Evaluation of Environmentally Friendly Lysis Reagents for Gene Therapy Drug Production
Lu Wang, PhD, Lead, Downstream Process Development, Spark Therapeutics, Inc.
Triton X-100 (TX-100) is one of the most popular lysis reagents used in gene therapy industry. However, Triton X-100 has endocrine disrupting properties which elicit environmental concerns. Therefore, this chemical is listed as an emerging compound of concern by many European countries. This study evaluated a few environmentally friendly surfactants as lysis reagents for vector release. The lysis efficiency and filterability were first studied through bench-scale experiments.

11:40 POSTER HIGHLIGHT: Process Development for Extracellular Vesicle Purification Using Affinity Chromatography
Benjamin Barnes, Research Engineer, University College London
Despite the rapidly growing commercial interest in extracellular vesicles, manufacturing processes for EV production are still in the development phase. The use of the glycosaminoglycan, heparin, to capture extracellular vesicles is a relatively novel and unexplored method of purification. In this study, we demonstrated the use of heparin affinity chromatography to both purify and fractionate extracellular vesicles into subpopulations.

12:10 pm Luncheon Presentation: Key Buffer Considerations for Full Capsid Enrichment of Recombinant AAV TEKNOVA science matters
Bella Neufeld, PhD, Director of Research and Development, Teknova
Successful gene therapy using AAV vectors requires high titers of functional virus. A challenge for AAV production is enriching full capsids by anion-exchange chromatography. Each capsid and transgene modification requires specific chromatography buffers with discreet, critical parameters for optimized production. Teknova's ability to manufacture high-quality custom buffer formulations, combined with our in-house purification platform, allows us to identify the ideal buffer candidate quickly and effectively for use in scale-up and GMP production.

12:40 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

RISK MITIGATION STRATEGIES

1:25 Chairperson's Remarks
Lu Wang, PhD, Lead, Downstream Process Development, Spark Therapeutics, Inc.

1:30 CASPON – A Platform Process for Non-Platform Proteins
Gerald Striedner, PhD, University Professor, Biotechnology, University of Natural Resources and Life Sciences Vienna (BOKU), Austria

2:00 The Mitigation of Lipase Risk in a Bispecific Antibody Downstream Process

Haiying Bao, Principal Scientist, Bristol Myers Squibb Co.

Polysorbate 80 is commonly used as a surfactant in the therapeutic protein formulation. Lipase-mediated enzymatic polysorbate degradation is one of the challenges in the manufacturing and storage of the biologics. In this study, a series of the chromatographic approaches were evaluated to minimize the lipase risk in a bispecific antibody downstream process which has successfully been scaled up to the manufacturing scale.

2:30 Efficient Impurity Removal through Use of Novel Additives and Biodegradable Detergent

Jungmin Oh, Development Manager, Bioprocessing Research, Avantor

For cell-derived products, it is critical to demonstrate removal of viral contaminants, host cell DNA, and protein impurities. This presentation will discuss use of novel additives to drive downstream process efficiency. Case studies will show various processes from mAb to viral vector. Examples from a biodegradable detergent and a novel Protein A resin will be explored.

3:00 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

ROOM LOCATION: Constitution A&B

PLENARY KEYNOTE: LEADING TO TOMORROW'S ADVANCES

3:50 Plenary Introduction

Nathalie Clément, PhD, CEO, Unicorn Consultations, LLC

4:00 New Therapeutic Modalities and Moore's Law in Biomanufacturing

Hari Pujar, PhD, Operating Partner, Flagship Pioneering; COO, Tessera Therapeutics

As we have embarked on newer, more complex modalities, biomanufacturing has appeared to stumble. The talk will chronicle the advancements in biomanufacturing of different therapeutic modalities, drawing parallels to semiconductor chip manufacturing, and establishing the rightful and bright future of biomanufacturing.

4:30 Cell and Gene Therapy (R)evolution

Mercedes Segura Gally, PhD, Vice President, Process Development, ElevateBio

The concept of gene therapy arose nearly half a century ago. Turning that concept into a therapeutic reality required years of scientific discovery, technological advances, and pioneering efforts, culminating in several regulatory approvals. These success stories paved the road for a second wave of advanced therapies. Compared with traditional biologics, cell and gene therapy products pose unique challenges. This presentation aims to summarize the progress made in recent years.

5:00 Networking Reception in the Exhibit Hall with Poster Viewing (Grand Ballroom)

6:00 Close of Day

THURSDAY, AUGUST 18

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Back Bay C

ADVANCED TECHNOLOGIES AND APPROACHES

7:55 Chairperson's Remarks

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

8:00 High-Resolution Imaging to Aid Understanding of Liposome Sterilizing Grade Filtration

Thomas F. Johnson, PhD, Postdoc Research Assistant, Biochemical Engineering, University College London

Two imaging techniques were used to visualize and characterize sterilizing grade filtration of liposomes, examining a dual-layer polyethersulfone system. X-ray computed tomography resolved internal nanoporous structure that enabled pore size distributions to be calculated for both layers. Confocal microscopy identified the location where liposomes were retained within membranes after filtration, with three differential pressures compared for processing performance and retention profiles as a function of distance through each layer.

8:30 Mechanistic Modeling of a Hydrophobic Interaction Chromatography (HIC) for Protein Antigen Purification

Angela Li, PhD, Senior Scientist, Sanofi Pasteur

The current study explores the use of DSPX, a mechanistic modeling software, to simulate an HIC process used in the purification of a protein antigen. A mechanistic model is the basis for digital twin, allowing the simulation and prediction of chromatography performance, which may be applied to design space characterization, process monitoring, and control. In this study, following calibration of the initial model, its application for process optimization was explored.

9:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

9:15 Poster Award Presented in the Exhibit Hall

9:30 Quantitative Determination of Titer in Harvest for Bioprocess Using Real-Time FTIR Monitoring

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

A quantitative FTIR model was calibrated for real-time monitoring of mAb titer using clarified bulk harvests of three different mAbs. The challenge of the project was to minimize the interference of the IR signal from the host cell proteins. With proper calibration design and data pretreatment, the final model exhibited acceptable accuracy predicting titer in new clarified bulk harvest from fed batch and perfusion.

10:00 A Single Solution for Screening, Discovery, and Manufacturing of Biopharmaceuticals Based on a Self-Removing Affinity Tag

David W. Wood, PhD, Professor, Chemical & Biomolecular Engineering, The Ohio State University

We have developed a powerful purification platform based on a self-removing affinity tag that can serve both basic research and clinical manufacturing applications. In practice, a simple shift in buffer pH releases the purified tagless target from the affinity column, which can then be stripped and reused. The power and versatility of this system is demonstrated here using several case studies on proteins expressed in bacterial and mammalian host cells.

10:30 Rapid, Consistently-Scalable Protein A Membrane Chromatography

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

Lack of consistent, scalable Protein A membranes has limited membrane chromatography's potential in antibody-based therapies. Gore's high-throughput affinity capture devices surpass the limits of existing bead and membrane options. GORE® Protein Capture Devices achieve rapid cycling, high binding capacity at short residence times and low, consistent column pressure-drop. Our data demonstrate increased productivity and scalable performance across commercial sizes up to 232 mL and larger next-generation devices.

11:00 Breakout Discussions

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: Advances and Challenges in Protein Purification

Maria Lorenzo, Principal Scientific Researcher, Protein Chemistry, Genentech, Inc.

- Challenges in the high-throughput purification of intracellular proteins
- Elements of a robust protein purification platform
- High-throughput in protein stability determination
- New technologies in protein purification and processes

12:00 pm Luncheon Presentation: Intensifying Cell Clarification by a Streamlined Platform Approach Using Fluidized Bed Centrifugation

Martin Saballus, Scientist, BioProcessing DSP, Sartorius Stedim Biotech GmbH

A novel single-use based clarification platform was developed combining fluidized bed centrifugation with integrated filtration. The feasibility of this streamlined setup was investigated up to 200-liter bioreactor scale, where low harvest turbidity (2 NTU) and superior antibody recovery (95%) were achieved. A cost-of-goods (CoG) analysis, comparing conventional depth filtration with the developed unit operation, showed enormous potential to save more than half of the clarification costs in industrial biopharmaceutical manufacturing.

12:30 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

PURIFICATION OF AAVs

1:05 Chairperson's Remarks

Ohnmar Khanal, PhD, Senior Scientist, Technical Development, Downstream and Drug Product Development, Spark Therapeutics

1:10 Building a Reliable Workflow to Accelerate AAV Full-Capsid Enrichment Development

Ana De Castro, PhD, Scientist, Genomic Medicine Purification Process Development

Although AAVs can deliver genes in an efficient and targeted manner, the presence of empty capsids still raises safety concerns. Anion exchange chromatography (AEX), commonly used for full-capsid enrichment, often requires extensive development work. Hence, we established a systematic workflow for the AEX optimization of different AAV serotypes. Leveraging high-throughput screening and a methodical buffer evaluation, our workflow has demonstrated to be a powerful tool in AEX development.

1:40 The Impact of Capsid-Resin Interaction on Separation, Recovery, and Product Stability

Ohnmar Khanal, PhD, Senior Scientist, Technical Development, Downstream and Drug Product Development, Spark Therapeutics

Chromatography is becoming an increasingly popular tool for the purification of rAAV capsids. Base chromatographic matrices in the market are primarily designed for the purification of protein bio-therapeutics. Therefore, the purification of rAAV capsid with these resins can present challenges. In this talk, we share our investigation of how capsid-resin interaction impacts purification performance, product recovery, and product stability.

2:10 Improving AAV Purification across Multiple Constructs

Matthew Roach, Associate Director, AAV Production, BridgeBio

AAV continues to provide clinical success, but still needs significant improvements in the processes used to purify it. Alongside this, there remains a lack of information on the purification of various serotypes and transgenes. This presentation will describe our efforts to report the purification of multiple serotype and transgene combinations across platform purification steps.

2:40 Refreshment Break in the Exhibit Hall & Last Chance for Poster Viewing (Grand Ballroom)

ROOM LOCATION: Constitution B

3:10 Optimization of an AAV Purification Process to Accommodate Increased Upstream Yield and Reduce Manufacturing Bottlenecks

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

In recent years, significant resources have been invested into increasing the productivity of AAV manufacturing. Optimization of upstream processes has led to significant increases in AAV titer, and downstream purification strategies designed around lower-yielding production must be reevaluated to better accommodate the large increases in vector. This presentation highlights work done to modify downstream purification steps to handle a titer increase seen during the implementation of a next-generation upstream process.

3:40 Chromatographic Method Development for Enrichment of Full Capsids

Paul Greback-Clarke, Scientist, AAV Process Development, Asklepios BioPharmaceutical, Inc.

Variable empty/full distribution coming out of upstream unit ops coupled with the fact that empty particles co-purify with full particles are significant challenges in rAAV manufacturing. Modulating the mobile phase composition during the AEX load enables the flow-through or "partitioning" of empty capsids while selectively binding full rAAV particles. Minimizing empty capsid binding simplifies the elution strategy which can be a significant benefit during tech transfer and scale-up.

4:10 Recent Advances in Processing of AAV

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

This talk will discuss transformative technologies in the production, filtration, and chromatography-based purification of AAV with real-life examples of the challenges that have been overcome. Some real-life examples will include the development of depth filtration for suspension AAV production, which increased capacity and recovery by 4x and 2x respectively, and a new strategy for ensuring viral clearance that resulted in >85% recovery.

4:40 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 15-16

Gene Therapy CMC and Analytics

View Program »

AUGUST 17-18

Gene Therapy Manufacturing

View Program »

MONDAY, AUGUST 15

9:00 am Main Conference Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Constitution B

LATEST CHALLENGES IN GENE THERAPY ANALYSIS AND QUALITY

9:55 Chairperson's Opening Remarks
Mike Kelly, PhD, Senior Vice President, CMC, Atsena Therapeutics

10:00 KEYNOTE PRESENTATION: What Is in Those Capsids? AAV Capsid Content Is an Attribute with Many Measures
Herbert A. Runnels, PhD, Global CMC AAV Analytical Sponsor, Gene Therapy, Pfizer Inc.
In addition to the full-length vector genome, capsids of AAV vectors could be packed with partial transgene cassettes and other DNA impurities. The characterization of these capsid-delivered nucleic acids is a growing field of interest for both drug product safety and potency considerations. This presentation will describe tools for empty *vs* full capsid characterization and highlight how next-generation sequencing is enabling the elucidation of the nucleic acid payload.

10:30 Quality Control of Viral Vectors
Jerome Jacques, PhD, Principal Scientist, United States Pharmacopeia
Qualification of raw materials used in the manufacturing of viral vectors requires the use of risk assessment strategies to categorize the critical components of a manufacturing process. In addition to cell culture supplements, excipients and other formulation components must meet the required quality to ensure consistency in manufacturing, quality, and safety.

11:00 Viral Safety: The Need for a Wholistic Approach
Christopher Bravery, PhD, Consulting Regulatory Scientist, Advanced Biologicals Ltd.
Because there are limited options to apply viral reduction or elimination steps in the manufacture of viral vectors, viral control becomes more important. As a consequence, viral testing likely needs to be more comprehensive. Multiple orthogonal methods increase the chances of detection and examples of viral contamination events will be presented.

11:30 Enjoy Lunch on Your Own

ASSAY DEVELOPMENT AND VALIDATION

12:50 pm Chairperson's Remarks
Mike Kelly, PhD, Senior Vice President, CMC, Atsena Therapeutics

12:55 Development of a Potency Assay Matrix for the Characterization of AAV Gene Editing Products
Lauren M. Drouin, PhD, Director, Analytical Development LogicBio Therapeutics, Inc.
Potency is one of the most important critical quality attributes for gene therapy products but also the least well understood. This is primarily due to the complex mechanism of action which is inherent to the use of viral vectors for therapeutic gene delivery. Here we describe our approach to developing a potency assay matrix to satisfy the regulatory requirements for a clinical AAV gene editing product.

1:25 Evaluating Strategies for AAV Productivity and Efficacy
Susan D'Costa, PhD, Executive Vice President & Global Head Technology, Alcyone Therapeutics, Inc.
Part of the success of a gene therapy program is a robust manufacturing process that results in high productivity and highly functional vectors. Developing platform manufacturing processes and platform analytical assays is a CMC strategy to save time and resources. Alcyone will share updates on our efforts to develop a robust platform upstream process as well as a platform *in vitro* potency assay.

1:55 Analytical Assessment of AAV Capsid at Harvest
Yulia Ivanova, PhD, Principal Scientist, Bioanalytical R&D, Pfizer Inc.

2:10 A Simple, Rapid Method for Baculovirus Titer Determination and Detection of Residual Baculoviral DNA by Droplet Digital PCR
Mary M. Ng, Scientist, Analytical Development, Prevail Therapeutics
The baculovirus expression vector system (BEVS) to produce gene therapy vectors involves infecting insect cells with a recombinant baculovirus expressing a gene of interest. A sufficient amount of virus is needed for infection and expression of the GOI, and hence, an accurate titer would need to be measured. Presently, we developed a method using droplet digital PCR that offers better precision and accuracy without the reliance on a standard curve.

2:25 Networking Refreshment Break (Grand Ballroom Foyer)

CMC STRATEGIES FOR GENE THERAPIES

2:40 PANEL DISCUSSION: CMC Strategies for Gene Therapies
Moderator: James Richardson, PhD, Senior Director, Analytical Development, Interus BioTherapeutics
Panelists:
Svetlana Bergelson, PhD, Senior Director, Technology Development, Biogen
Marina S. Feschenko, PhD, Senior Director, Intergalactic Therapeutics
Susan D'Costa, PhD, Executive Vice President & Global Head Technology, Alcyone Therapeutics, Inc.
Lauren M. Drouin, PhD, Director, Analytical Development LogicBio Therapeutics, Inc.
Santoshkumar L. Khatwani, PhD, Director, Analytical Development, Sangamo Therapeutics

Panel Discussion3:40 Session Break and Transition to Plenary Keynote

ROOM LOCATION: Constitution A&B

PLENARY KEYNOTE: SOLVING TODAY'S CHALLENGES

4:20 Plenary Introduction
James Warren, PhD, Vice President, Pharmaceutical Development, Ultragenyx Pharmaceutical

4:30 Lessons Learned from the Pandemic: mRNA-LNP Vaccine Development
Nicholas Warne, PhD, Vice President, Pharmaceutical Research and Development, BioTherapeutics Pharmaceutical Sciences, Pfizer Inc.
The speed and scale of industry response to the COVID pandemic was unprecedented, ultimately leading to the availability of several vaccines in under a year. This presentation will discuss the approach taken by Pfizer, with their partner BioNTech, in the development, manufacture, and distribution of the vaccine drug product while reflecting on lessons that may, or may not, be applicable to future product development.

5:00 Advances in Vaccine Formulation and Stability
David B. Volkin, PhD, Distinguished Professor, Pharmaceutical Chemistry, University of Kansas, Lawrence
This presentation will provide an overview of analytical characterization and formulation development considerations for new vaccine candidates targeted for use in low- and middle-income countries (LMICs). Illustrative case studies with vaccine candidates (e.g., live-virus, adjuvanted recombinant protein) will highlight implementing state-of-the-art stability-indicating assays to enable development of stable formulations. Challenges with developing lower-cost formulations (e.g., multi-dose, combination, non-parenteral) to expand vaccine coverage in LMICs will also be discussed.

5:30 Welcome Reception in the Exhibit Hall with Poster Viewing (Grand Ballroom)

6:30 Close of Day

TUESDAY, AUGUST 16

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Constitution B

EVALUATING AND CHARACTERIZING PRODUCT-RELATED IMPURITIES

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

8:00 Expanding the Stability-Monitoring Toolbox for AAV
Kelly Walsh, Scientist I, Oxford Biomedica Solutions
To develop a liquid formulation for AAV we looked beyond the standard analytics and assessed colloidal behavior. Examination of the colloidal state using novel analytical technologies for subvisible particles and SLS/DLS complemented existing datasets supporting 2-8°C stability. We use these biophysical analytics to support the liquid formulation development for AAV products. This work confirms a stable AAV liquid formulation, supporting improved logistics and storage conditions for the gene therapy field.

8:30 Evaluation and Characterization of Residual DNA in AAV Gene Therapy Drug Products
Xiaozhu Sue Duan, Associate Director, Analytical Development, Astellas Gene Therapies
All rAAV products contain varying quantities of non-vector DNA derived from host cells and plasmids used in manufacturing process. In this study, DNA copy number and length of six residual genes, including HEK293 genomic and plasmid DNA, were evaluated in twenty-one lots by dual-color ddPCR assay. Study showed that all residual DNA amounts were low and close on a lot-to-lot basis.

9:00 Characterization of rAAV Genome Packaging with Orthogonal Methods
Wei Zhang, Associate Director, Analytical Development, Ultragenyx
Removing and characterizing empty and partial -genome-containing capsids are critical for safety and efficacy of rAAV products. Common characterization tools, such as AUC and TEM, are complicated and labor intensive, and not ideal for routine testing. Here we present different orthogonal methods in characterizing rAAV viral vectors genome. The methods are sensitive in detecting viral vector genome content, and are great additional characterization tools.

9:30 High-Throughput, Low-Volume Viral Vector Aggregation Characterization
halo labs
Bernardo Cordovez, CSO & Co-Founder, Halo Labs
Within early-stage development of gene therapeutics, there is a need to perform low volume subvisible particle imaging, counting, sizing and identification to characterize for product stability and impurities. Aura GT is the only tool that rapidly and accurately measures physical stability of viral vector samples and serotypes with just 5µL. Assess for AAV capsid integrity, DNA leakage and their impact on particle formation from early-stage development through drug commercialization.

10:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

10:45 Breakout Discussions
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: Analytical Strategies for Gene Therapies
Wei-Chiang Chen, PhD, Associate Director, BioProcess Analytics, Genomic Medicine Unit, Sanofi

11:30 FEATURED PRESENTATION: CMC Strategies for Process Intensification and Acceleration of IND Submission for Gene Therapies
Srinivas Chollangi, PhD, Director, Cell and Gene Therapy CMC, Sanofi

12:00 pm Identifying Stability-Indicating Assays for AAV Gene Therapy Products
Anna Pavlova, Scientist III, Sangamo Therapeutics
Identifying potential critical quality attributes of AAV gene therapy products is challenging due to their inherent complexity and insufficient understanding of the manufacturing process. This presentation will focus on unique properties of different AAV serotypes subjected to forced degradation conditions. For this purpose, various analytical methods and orthogonal approaches were used to evaluate the impact, including chromatography, electrophoresis, LC-MS, molecular biology methods, and effects on potency.

12:30 Take Command of Your AAV Process with Hassle-Free Tools for Quantification and Stability
UNCHAINED LABS
Kevin Lance, PhD, Director of Analytics Marketing, Unchained Labs
Getting almost any information on AAVs takes too much sample and time. But with Stunner's AAV Quant assay you can get the titer, empty/full ratio, and aggregation info you need to make your next in-process decision from 2 µL of sample and in less than a minute. When it's time to pressure test the stability

of your capsids, Uncle can take a full look at which capsids and conditions are the most stable by tracking DNA ejection from capsids, and protein unfolding and aggregation.

1:00 **CO-PRESENTATION: LUNCHEON PRESENTATION: Analytical Solutions to Accelerate AAV Based Gene Therapy**

Reiko Kiyonami, Senior Product Applications Specialist, Analytical Instrumentation Division, Thermo Fisher Scientific

Jonas Buege, Product Manager, Pharma Analytics, BioProduction, Thermo Fisher Scientific

Mass spectrometry and qPCR-based solutions will be presented for accurate and sensitive characterization of key attributes of AAV vectors and AAV manufacturing processes. Viral proteins are characterized at peptide and intact level for rapid confirmation of serotype and confident PTM identification, and accurate empty/full-ratio and mass measurement. Residual host cell and other DNA impurities are rapidly and accurately quantified, and size analysis performed on a single platform to meet regulatory requirements.

1:30 **Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)**

EVALUATING AND CHARACTERIZING PRODUCT-RELATED IMPURITIES

2:10 **Chairperson's Remarks**

Srinivas Chollangi, PhD, Director, Cell and Gene Therapy CMC, Sanofi

2:15 **Characterizing Host Cell Impurities in GT Products at Early/Late Phase Development**

Yiling Bi, PhD, Scientist III, Sangamo Therapeutics

The quantitation and control of Host Cell Proteins (HCPs) in gene therapy products face significant challenges as manufacturing processes not as well defined as those for traditional biologics, and upstream and downstream processes used by various manufacturers may differ widely. We will discuss a case study of HCP analysis for an adeno-associated virus (AAV)-based gene therapy product, also considerations for choosing specific approaches during different clinical development stages.

2:45 **Vector Genome Titer Method Optimization**

Jeffrey Gagnon, Manager, Analytical Development Lab, Oxford Biomedica Solutions

Establishing a robust AAV vector genome titering method early in product development is critical. The vector genome titer method is commonly utilized to make manufacturing, stability, and even clinical dosing decisions. This presentation will discuss the measures taken to develop and optimize a gene-of-interest vector genome titer method using ddPCR.

3:15 **CO-PRESENTATION: Column-Free Purification of AAVs and mAbs With New Jetted Resin Modalities**

Oleg Shinkazh, CEO, Chromatan

Fred Ghanem, Director, Business Development, Purolite

Chromatan team will present initial results for AAV capture and mock mAb perfusion using the CCTC platform. CCTC shows significant improvements in productivity as well as improved control of product quality, low residence time, and improved recovery vs status quo column capture. Purolite team will present on the developments of continuous jetted resin technology and its impact of improving consistency and product quality

3:45 **Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)**

4:30 **Orthogonal Measures of Lentiviral Particle Attributes Using DLS, NTA, and Micro Flow Cytometry**

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

Lentiviral vectors are utilized in cell and gene therapy programs for both *ex vivo* and *in vivo* delivery. Tracking the quantity, size distribution, charge, and protein content of viral particles during production, purification, and formulation development can aid developers in making process decisions. In this presentation, we will discuss the use of DLS, NTA, Flow Virometry, and other methods to characterize lentiviral particles.

5:00 **Sedimentation Velocity Analytical Ultracentrifugation (SV-AUC) as an Important Orthogonal Tool for Gene Therapy Characterization**

Ronald T. Toth, PhD, Senior Scientist, Characterization, Sanofi

Data are presented demonstrating the utility of SV-AUC with case studies covering characterization of AAV vectors and DNA drug substance. For an AAV vector with increased HMW by SEC we show presents as a low molecular weight peak on SV-AUC, with a higher DNA content than the full capsid. Use of pseudo-absorbance and software tools to increase throughput and ultra-low loading concentrations to reduce sample requirements are also discussed.

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

WEDNESDAY, AUGUST 17

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Constitution B

OPTIMIZING VIRAL VECTOR MANUFACTURING

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 Considerations of Manufacturability for AAV-Based Gene Therapy Products for Rare Diseases

Nripen Singh, PhD, Head, Process and Product Development, Passage Bio

There are numerous manufacturing challenges that need to be overcome for manufacturing AAV therapeutics, including low transient transfection yields, high empty capsid production, and subsequently poor recovery across the purification process. Passage Bio has established a platform process for AAV production based on transient transfection of human embryonic kidney (HEK) 293 cells. This contribution will discuss the improvements made for both upstream and downstream processes along with state-of-the-art analytical methods.

8:30 FEATURED PRESENTATION: Production of AAV Vectors: Process Optimization towards Commercialization

Barbara Kraus, PhD, Head, Gene Therapy Process Development, Takeda

One of the currently most used vectors in clinical trials for gene therapy is the adeno-associated virus. This presentation provides an overview of Takeda's strategies for scaling AAV production processes. Furthermore, a case study is presented showing how the process layout for the manufacturing of AAV vectors based on a wild-type capsid needs to be changed in order to realize the production of AAVs with a genetically engineered capsid.

9:00 KEYNOTE PRESENTATION: Phased Approach to Establishing Production Platforms

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

Biogen plans to support multiple waves of programs within our Gene Therapy pipeline by advancing an existing production technology while establishing next-generation systems for future use. Here we will review development to-date for leveraging suspension transient transfection (sTT) for phase-appropriate support of programs. Additionally, we present strategies for implementing producer cell line (PCL) production systems as a sustainable manufacturing platform.

9:30 Quality Attributes and Extended Characterization of Gene Vectors Using Light Scattering

John Champagne, PhD, Senior Application Scientist & Northeast Regional Manager, Wyatt Technology

The success of gene therapy owes greatly to the delivery vehicles used, such as adeno-associated viruses (AAV) and lipid nanoparticles (LNP). Quantifying quality attributes of gene vectors is important, as gene therapy products enter into the late development stages/QC environment. In this presentation, we demonstrate the use of three analytical techniques: batch dynamic light scattering (DLS), size exclusion chromatography coupled to multi-angle light scattering (SEC-MALS) and field-flow fractionation (FFF) with MALS.

10:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

10:40 New Approaches to Gene Therapy Manufacturing

Stephen Soltys, PhD, Vice President, Process Development, Kriya Therapeutics

Our cGMP production suites and single-use systems will allow the production of multiple products simultaneously at up to 3,000-liter bioreactor scale. We are developing reliable and robust production systems that can deliver higher amounts of AAV product per batch.

11:10 Comparison of Highly Pure rAAV9 Vector Stocks Produced in Suspension by PEI Transfection or HSV Infection Reveals Striking Quantitative and Qualitative Differences

Nathalie Clément, PhD, CEO, Unicorn Consultations, LLC

We will present our findings from an in-depth side-by-side comparison between PEI transfection HSV infection to produce rAAV9. Using a newly developed purification protocol compatible with both upstream platforms, we generated highly pure rAAV9 stocks, which were assessed for yields, quality attributes and residual impurities. We found notable qualitative and quantitative differences between PEI- or HSV-mediated production, which will be presented.

11:40 PANEL DISCUSSION: Manufacturing Strategies for Gene Therapies

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

Panelists:

Barbara Kraus, PhD, Head, Gene Therapy Process Development, Takeda

Stephen Soltys, PhD, Vice President, Process Development, Kriya Therapeutics

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

Rajiv Gangurde, PhD, CTO, SparingVision

12:10 pm LUNCHEON PRESENTATION: Full rAAV Capsid Enrichment in a Scalable Reproducible Viral Vector Manufacturing Platform

Mark Schofield, Senior R&D Manager, Pall Corporation

Scalable production of AAV gene therapies remains challenging with respect to yield and removal of "empty" capsids that do not contain the therapeutic DNA payload. To begin to address these challenges an end to end AAV production process has been developed that enables a complete AAV purification solution. Here we show our process development steps and how to improve yield, with a particular emphasis on the depletion of empty capsids

12:40 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

INCREASING YIELD, INTENSIFYING THE PROCESS

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 Development of an Intensified AAV Production Process

Jan Panteli, PhD, Associate Director, Upstream Process Development, Ultragenyx Pharmaceutical

There remains a significant need to improve AAV manufacturing platforms to achieve robust, high-yielding, scalable and cost-efficient processes. At Ultragenyx, we employ a HeLa producer cell line, helper-virus based infection process for AAV production which is reproducibly scalable to 2000L. We have

developed an intensified AAV production process, through implementation of perfusion to increase cell density, to achieve high titer, =3E11 GC/mL and high purity AAV suitable for clinical use.

2:00 Increased Yield of Adeno-Associated Viruses through Bioreactor Process Improvement

Kory Blocker, PhD, Director, Upstream Process Development, Vector Core, Gene Therapy Program & Orphan Disease Center, University of Pennsylvania

Through the delivery of recombinant adeno-associated virus (rAAV) vectors, gene therapy has the potential to cure and/or treat many genetic disorders. To lower manufacturing costs and enable the treatment of larger populations, it is critical to improve process yield. In this work, we describe the approach taken to develop, optimize, and scale-up triple transfection bioreactor process to improve per-batch productivity.

2:30 Versatile Platform Technologies for Intensified Viral Vector Production

Guodong (Javier) Jia, PhD, CEO, OBiO Technology

OVersatile™ platform enables flexible and high-quality process development and manufacturing solutions for a variety of gene and cell therapy products. The platform facilitates a high-titer, large-scale production capability and provides versatile development possibilities for various demands. The presentation will highlight research and process development of viral vector-based products with the application of novel manufacturing technologies.

3:00 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

ROOM LOCATION: Constitution A&B

PLENARY KEYNOTE: LEADING TO TOMORROW'S ADVANCES

3:50 Plenary Introduction

Nathalie Clément, PhD, CEO, Unicorn Consultations, LLC

4:00 New Therapeutic Modalities and Moore's Law in Biomanufacturing

Hari Pujar, PhD, Operating Partner, Flagship Pioneering; COO, Tessera Therapeutics

As we have embarked on newer, more complex modalities, biomanufacturing has appeared to stumble. The talk will chronicle the advancements in biomanufacturing of different therapeutic modalities, drawing parallels to semiconductor chip manufacturing, and establishing the rightful and bright future of biomanufacturing.

4:30 Cell and Gene Therapy (R)evolution

Mercedes Segura Gally, PhD, Vice President, Process Development, ElevateBio

The concept of gene therapy arose nearly half a century ago. Turning that concept into a therapeutic reality required years of scientific discovery, technological advances, and pioneering efforts, culminating in several regulatory approvals. These success stories paved the road for a second wave of advanced therapies. Compared with traditional biologics, cell and gene therapy products pose unique challenges. This presentation aims to summarize the progress made in recent years.

5:00 Networking Reception in the Exhibit Hall with Poster Viewing (Grand Ballroom)

6:00 Close of Day

THURSDAY, AUGUST 18

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Constitution B

OPTIMIZING THE PROCESS

7:55 Chairperson's Remarks

Michael Mercaldi, PhD, Senior Director, Downstream Process Development, Oxford Biomedica Solutions

8:00 A Robust and Scalable Platform Process for GMP Manufacturing of Lentiviral Vectors

Bojiao Yin, PhD, Director, Vector Process Development & Manufacturing, ElevateBio

We describe here a well-established platform process for LV production based on transient transfection of serum-free cells grown in suspension. Both upstream and downstream processes are highly optimized to achieve optimal vector yields and significant decrease in the impurities (host cell protein/DNA, plasmid DNA). The compatibility of this platform process has been evaluated with multiple CAR/TCR genes while the robustness is demonstrated in reproducible runs at pilot scale.

8:30 Manufacturing Lentiviral Vectors for *in vivo* CAR T Cell Therapy

Sarah Gould, PhD, Associate Director, Manufacturing Science & Technology, Umoja Biopharma

Umoja aims to transform cancer care by creating an off-the-shelf, direct injection lentiviral vector (LVV) drug product for *in vivo* CAR T cell generation and expansion. We will present our approach to reproducible and scalable manufacturing of high-quality LVV, with special focus on expediting process development with risk-based quality documentation and impurity clearance at key process unit operations to meet ambitious final specifications.

9:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

9:15 Poster Award Presented in the Exhibit Hall

9:30 Scaling-Up of a Suspension Packaging Cell Line for Lentiviral Vector Production: Upstream and Downstream Strategies

Aziza Manceur, PhD, Research Officer, National Research Council Canada

To streamline lentiviral vector (LV) manufacturing, we propose to use packaging cell lines. The cells were designed using molecular switches to control the production of LV cytotoxic proteins. They can be used to generate LV through a one-plasmid transfection corresponding to the gene of interest, or to generate stable producer cells. We will present a manufacturing process that circumvents the labile nature of the vectors and results in high yields.

10:00 Overcoming the Bottlenecks in the Manufacturing of Viral Vector-Based Therapies

Saurabh Gautam, PhD, Principal Scientist and Lab Head, Bioprocess Development, Viral Vectors, and Vaccines, ViraTherapeutics / Boehringer Ingelheim

A major gap with viral vectors is in our knowledge of the biology and morphology of the therapeutic. The work presented will focus on our efforts in development of novel chromatographic purification techniques complimented with extensive characterization of our virus using a suite of analytics.

10:30 Platform-Based Scalable Suspension Process for LVV Production

Sneha Rangarajan, PhD, Senior Scientist and Team Lead, IDT Biologika

Lentiviruses have been widely used for cell therapy based applications. This presentation will showcase IDT Biologika's platform based approach for Lentiviral Vector production. The strategies described here will enable a scalable and robust process that focuses on suspension transient transfection of serum free HEK cells.

11:00 Breakout Discussions

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: DSP Strategies for Gene Therapies

Meisam Bakhshayeshi, PhD, Senior Director and Head, Process Development, Intergalactic Therapeutics

12:00 pm Luncheon Presentation: Strategies to Propel Your Viral Vector Therapy from Lab to Clinic

Christine Ricci, Senior Scientist, Upstream Process Development, Viral Gene Therapy, Fujifilm Diosynth Biotechnologies

There are a number of challenges drug developers face on the journey to the clinic, including high costs and competitive timelines. FDB has solutions that help our partners navigate the challenges of development and material supply for clinical trials as well as propel their life changing medicines to commercialization. Our solutions include a flexible AAV platform and a wealth of regulatory experience taking medicines to the market for our partners.

12:30 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

ADVANCING PURIFICATION FOR GENE THERAPIES

1:05 Chairperson's Remarks

Meisam Bakhshayeshi, PhD, Senior Director and Head, Process Development, Intergalactic Therapeutics

1:10 Utilization of a Platform Approach toward AAV Purification Process Development

Ashish Sharma, PhD, Senior Scientist, Oxford Biomedica Solutions

A platform-based approach for AAV purification enables rapid scale-up of high-quality vector, into manufacturing and the clinic. This presentation will showcase how Oxford Biomedica Solutions' AAV platform was created by developing a deep understanding of chromatographic behavior, understanding the impact construct design and bioreactor performance have on purification, and focusing on process robustness throughout the development life cycle.

1:40 High-Throughput Purification and Mass Photometry Characterization of rAAV Viral Vectors

Dennis P. Chen, Scientist II, Downstream Process Development, Ultragenyx Pharmaceuticals

Recombinant adeno-associated virus (rAAV) has been broadly used as a vector for gene therapy applications. By combining the TECAN platform and light-scattering analytical methods, we demonstrated an integrated workflow to process clarified bioreactor material through affinity chromatography in a high-throughput manner to facilitate upstream process development. This methodology reduces material requirements and processing time, while providing readouts of product quality attributes that enable rapid and efficient process development.

2:10 Leveraging High-Throughput Ambr15 and Ambr250 Technologies to Advance Upstream Gene Therapy Process Development

Maria C Choi-Ali, Sr Engineer I, Gene Therapy, Biogen

2:20 Process Improvements for AAV Production by Transient Transfection of HEK293 Cells

Stephanie Doong, PhD, Scientist, Process Engineering, Vertex Pharmaceuticals Cell & Genetic Therapies

2:30 Optimizing Tangential Flow Filtration (TFF) Using Hollow Fiber for AAV Formulation

Xiaolong Lu, PhD, Director Downstream Process Development, Gene Therapy Program, University of Pennsylvania

2:40 Refreshment Break in the Exhibit Hall & Last Chance for Poster Viewing (Grand Ballroom)

3:10 Optimization of an AAV Purification Process to Accommodate Increased Upstream Yield and Reduce Manufacturing Bottlenecks

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

In recent years, significant resources have been invested into increasing the productivity of AAV manufacturing. Optimization of upstream processes has led to significant increases in AAV titer, and downstream purification strategies designed around lower-yielding production must be reevaluated to better accommodate the large increases in vector. This presentation highlights work done to modify downstream purification steps to handle a titer increase seen during the implementation of a next-generation upstream process.

3:40 Chromatographic Method Development for Enrichment of Full Capsids

Paul Greback-Clarke, Scientist, AAV Process Development, Asklepios BioPharmaceutical, Inc.

Variable empty/full distribution coming out of upstream unit ops coupled with the fact that empty particles co-purify with full particles are significant challenges in rAAV manufacturing. Modulating the mobile phase composition during the AEX load enables the flow-through or "partitioning" of empty capsids while selectively binding full rAAV particles. Minimizing empty capsid binding simplifies the elution strategy which can be a significant benefit during tech transfer and scale-up.

4:10 Recent Advances in Processing of AAV

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

This talk will discuss transformative technologies in the production, filtration, and chromatography-based purification of AAV with real-life examples of the challenges that have been overcome. Some real-life examples will include the development of depth filtration for suspension AAV production, which increased capacity and recovery by 4x and 2x respectively, and a new strategy for ensuring viral clearance that resulted in >85% recovery.

4:40 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. The stream is paired with the Gene Therapy CMC and Analytics track.

Conference Programs

AUGUST 15-16

Cell Therapy CMC and Analytics

View Program »

AUGUST 17-18

Cell Therapy Manufacturing

View Program »

MONDAY, AUGUST 15

9:00 am Main Conference Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Back Bay A

CMC CHALLENGES FOR CELL THERAPIES

9:55 Chairperson's Opening Remarks
Fouad Atouf, PhD, Vice President, Global Biologics, USP

10:00 Regulatory and Manufacturing Considerations for Next-Generation Cell Therapy Products
Mo Heidaran, PhD, Head, Translational and Regulatory Strategy, GC Therapeutics, Former FDA Reviewer
While the industry expects and anticipates approval of several products each year, there are signs that this pace of approval is not sustainable in view of deficiencies identified by the US FDA that are primarily focused on product manufacturing and consistency. This talk discusses the criticality of establishing phase-based CMC readiness, by developing a proactive plan to gain product knowledge iteratively while implementing a phase-appropriate approach to achieving full CGMPs.

10:30 KEYNOTE PRESENTATION: Current Challenges in Cell Therapy CMC
Bruce S. Thompson, PhD, Vice President and Technical Lead, Cell Therapy, National Resilience, Inc.
The cell and gene therapy field is a rapidly expanding mix of early-stage innovative programs and maturing therapies. This presents several challenges across CMC disciplines from acquisition and characterization of starting materials to manufacturing products in a closed, scalable and reproducible manner, to rapid testing and release of products for patient infusion. This discussion will focus on several of these issues and provide insights into how to prospectively approach them.

11:00 PANEL DISCUSSION: Latest Challenges in Cell Therapy CMC
Moderator: Fouad Atouf, PhD, Vice President, Global Biologics, USP
Panelists:
Bruce S. Thompson, PhD, Vice President and Technical Lead, Cell Therapy, National Resilience, Inc.
Sumona Sarkar, PhD, Biomedical Engineer, Biosystems and Biomaterials Division, Biomaterials Group, National Institute of Standards and Technology
Mo Heidaran, PhD, Head, Translational and Regulatory Strategy, GC Therapeutics, Former FDA Reviewer

11:30 Optimisation of High-Throughput GMP-Compatible Workflow for Efficient Single-Cell Cloning
ADVANCED INSTRUMENTS
Camilla Domeneghetti, Biology Manager – Cell Line Development, Advanced Instruments
We will demonstrate how you can achieve high single-cell seeding efficiencies and clonal outgrowth whilst conforming to the needs of regulatory bodies through proof of monoclonality. We will outline how to optimise and develop a robust GMP-compatible process by utilising a recombinant matrix, GMP media and dissociation reagents and outline some potential new GMP compatible consumables that will be available in combination with the Advanced Instruments technologies.

12:00 pm Enjoy Lunch on Your Own

CELL ANALYSIS AND ENSURING QUALITY CONTROL

12:50 Chairperson's Remarks
Fouad Atouf, PhD, Vice President, Global Biologics, USP

12:55 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 characterization and testing of cellular therapeutic products (CTPs) is a critical aspect of product development, translation and release. Here I will describe recent efforts in the standardization of the characterization and testing of CTPs. I will also describe recently published standards on cell counting as well as NIST technical programs for increasing confidence in cell count and viability assays.

1:25 Replacing Manual Gating of Flow Cytometry Data in Cell Therapy Manufacturing
Ryan Brinkman, Managing Director, Cytapex Bioinformatics, Inc; Distinguished Scientist, BC Cancer; Professor, Medical Genetics, UBC
Flow cytometry data analysis is extremely time-consuming and the principal source of variation in the application of the technology. While many unsupervised approaches have been developed, poor performance has limited their application to discovery applications. As an alternative, we have developed the best performing approach for cell population identification. It consistently achieves up to 94% accuracy, and is in use by pharma for clinical studies and cell therapy manufacturing.

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

2:25 Networking Refreshment Break (Grand Ballroom Foyer)

2:40 Raw Material Qualification for Cell Therapies
Ben Clarke, PhD, Senior Scientist, USP
Qualification of raw materials used in the manufacturing of cell therapies requires the use of risk assessment strategies to categorize the critical components of a manufacturing process. In addition to cellular starting materials, cell culture supplements, excipients, and other components must meet the required quality criteria to ensure consistency in manufacturing, quality, and safety.

3:10 Quality Systems for Large-Scale Manufacturing of Cell-Based Commercial and Clinical Products
Zorina Pitkin, Senior Vice President, Quality Systems, Organogenesis, Inc.
Diverse product lines require compliant, robust, and versatile quality systems. We present the quality requirements of cell-based products produced by Organogenesis and partners. The gold standard quality systems developed for the commercial large-scale manufacturing of Apligraf, a cell-based product regulated as a Class III medical device to treat diabetic foot and venous leg ulcers, are adapted for the specific regulatory requirements of the clinical and commercial products in Organogenesis's portfolio.

3:40 Session Break and Transition to Plenary Keynote

ROOM LOCATION: Constitution A&B

PLENARY KEYNOTE: SOLVING TODAY'S CHALLENGES

4:20 Plenary Introduction
James Warren, PhD, Vice President, Pharmaceutical Development, Ultragenyx Pharmaceutical

4:30 Lessons Learned from the Pandemic: mRNA-LNP Vaccine Development
Nicholas Warne, PhD, Vice President, Pharmaceutical Research and Development, BioTherapeutics Pharmaceutical Sciences, Pfizer Inc.
The speed and scale of industry response to the COVID pandemic was unprecedented, ultimately leading to the availability of several vaccines in under a year. This presentation will discuss the approach taken by Pfizer, with their partner BioNTech, in the development, manufacture, and distribution of the vaccine drug product while reflecting on lessons that may, or may not, be applicable to future product development.

5:00 Advances in Vaccine Formulation and Stability
David B. Volkin, PhD, Distinguished Professor, Pharmaceutical Chemistry, University of Kansas, Lawrence
This presentation will provide an overview of analytical characterization and formulation development considerations for new vaccine candidates targeted for use in low- and middle-income countries (LMICs). Illustrative case studies with vaccine candidates (e.g., live-virus, adjuvanted recombinant protein) will highlight implementing state-of-the-art stability-indicating assays to enable development of stable formulations. Challenges with developing lower-cost formulations (e.g., multi-dose, combination, non-parenteral) to expand vaccine coverage in LMICs will also be discussed.

5:30 Welcome Reception in the Exhibit Hall with Poster Viewing (Grand Ballroom)

6:30 Close of Day

TUESDAY, AUGUST 16

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

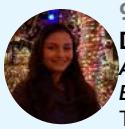
ROOM LOCATION: Back Bay A

COMPARABILITY, POTENCY ASSAYS, AND RELEASE TESTING

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

8:00 Comparability Strategies: Bridging Process Changes
Scott R. Burger, Principal, Advanced Cell & Gene Therapy LLC
Manufacturing process changes are inevitable, but present risk to CQAs. Comparability mitigates this risk. Changes with greater risk to CQAs or later in development require more stringent comparability. Parallel study designs using split starting material are preferable. Assays are not limited to release tests, and an assay matrix, using orthogonal methods, increases certainty. Acceptance criteria are narrower than for release and should be pre-defined, based on robust statistical analysis.

8:30 FEATURED PRESENTATION: Potency Assay Development for Cell-Based Therapies
Therese Choquette, PhD, Director, Analytics, Tigen
There are several challenges with potency assays for cell therapies such as a complex mechanism of action, product heterogeneity, assay controls, lack of reference standards, etc. Difficulties in finding the perfect assay occur depending on the type of cell therapy and it may be that a complicated assay or a matrix of assays is the best solution to determine potency. This presentation discusses challenges around potency assays for cell therapies.



9:00 FEATURED PRESENTATION: Method Development Strategy for Release Methods
Akshata Ijantkar, Senior Scientist, Analytical Development, Bristol Myers Squibb Co.
This presentation will go through method development, optimization, robustness and qualification strategy for release for infusion methods in a phase appropriate manner. Method development strategy will be presented in context of Flow Cytometry methods used to assess quality attributes such as purity and strength.

9:30 Robust Plasmid Manufacturing Process for Cell and Gene Therapeutics
Lan Tang, PhD, Director of Scientific Solutions, GenScript Probio USA Inc
Many advanced therapies rely on plasmid DNA to generate viral vectors and RNA vaccines; however, manufacturing pDNA poses an array of challenges. Learn how GenScript ProBio's multidisciplinary plasmid team tackles technical challenges around plasmid production and developed a well-established plasmid manufacturing process producing high quality and purity, with faster turn-around time and significant cost-savings compared to other CDMOs.

10:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

10:45 Breakout Discussions
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: CMC Challenges for Cell Therapies
Christopher Bravery, PhD, Consulting Regulatory Scientist, Advanced Biologicals Ltd.

11:30 GMP-Compliant Analytics and Processing for Clinical-Grade IPS Cell Banking
Ruud Hulspas, PhD, Technical Director, Process Development, Dana-Farber Cancer Institute
Considering that the first human-induced pluripotent stem cells were generated only 15 years ago, there is currently an impressive number of clinical trials to test their therapeutic value. However, tumorgenicity and heterogeneity remain major challenges to be addressed in manufacturing

of clinical-grade iPS cells. Here, we discuss risk-based and process-stage-appropriate quality considerations for consistent manufacturing of uniform iPS cells to maximize patient safety in clinical trials.

12:00 pm Identification of Predictive Critical Quality Attributes for Manufacturing Cardiomyocytes from Pluripotent Stem Cells
Sean P. Palecek, PhD, Professor, Chemical & Biological Engineering, University of Wisconsin, Madison
Differentiation of human pluripotent stem cells (hPSCs) to cardiomyocytes (CMs) is a lengthy process prone to batch failures. Currently, in-process monitoring of differentiation progression is rarely utilized. We performed a longitudinal multi-omics analysis of successful and failed batches of hPSC-derived CMs. I will describe gene, protein, and metabolite multivariate parameters that when measured early in differentiation will allow prediction of CM purity in the end product.

12:30 Validation and Approval for Lot-Release Testing of a Rapid Mycoplasma Assay
Darren J Bauer, Field Applications Specialist, Pharmaceutical Analytics, Thermo Fisher Scientific

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

1:30 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

ANALYTICAL STRATEGIES

2:10 Chairperson's Remarks
Ruud Hulspas, PhD, Technical Director, Process Development, Dana-Farber Cancer Institute

2:15 Cell Sorting for GMP Manufacture of Cell Therapies
Harish Adoni, Scientist, Analytics Cell Therapy, ElevateBio
Over the past decade, the development of cell therapies has gained significant momentum. Processes executed in a hospital setting such as the isolation of tumor-infiltrating lymphocytes (TILs), transformed clones of hematopoietic stem cells (HSCs), or other rare cells, are challenging to replicate in a cGMP environment. We will discuss the cell sorting process and analytical development considerations when implementing fluorescence-activated cell sorting (FACS) in a cGMP environment.

2:45 Analytical Characterization Approach of Novel Red Cell Therapeutic Product Candidates
Paul Liebesny, PhD, Senior Scientist, Rubius Therapeutics, Inc.
Rubius Therapeutics is developing a novel cell therapy platform by genetically engineering donor-derived precursor cells to express therapeutic proteins inside or on the cell surface and culturing them into erythroid cells, called Red Cell Therapeutics. Developing analytical tools to characterize the details of this maturation is key to understanding process robustness and Critical Quality Attributes. Approaches to understand in-process cellular phenotype, cellular impurities, and overall cell health will be discussed

3:15 Cell Banking Considerations for Allogeneic Products
Christopher Bravery, PhD, Consulting Regulatory Scientist, Advanced Biologicals Ltd.
The business model for allogeneic cell therapies can appear similar to that of traditional biotech products, however with the exception of some stem cells, banked cells from a single donor are unlikely to last the whole product lifecycle. This means various aspects of banking will differ between products. This talk will consider some of these factors, their regulatory implications, and how to address them.

3:45 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

ROOM LOCATION: Constitution B

4:30 Orthogonal Measures of Lentiviral Particle Attributes Using DLS, NTA, and Micro Flow Cytometry
James Richardson, PhD, Senior Director, Analytical Development, Interus BioTherapeutics
Lentiviral vectors are utilized in cell and gene therapy programs for both *ex vivo* and *in vivo* delivery. Tracking the quantity, size distribution, charge, and protein content of viral particles during production, purification, and formulation development can aid developers in making process decisions. In this presentation, we will discuss the use of DLS, NTA, Flow Virometry, and other methods to characterize lentiviral particles.

5:00 Sedimentation Velocity Analytical Ultracentrifugation (SV-AUC) as an Important Orthogonal Tool for Gene Therapy Characterization
Ronald T. Toth, PhD, Senior Scientist, Characterization, Sanofi
Data are presented demonstrating the utility of SV-AUC with case studies covering characterization of AAV vectors and DNA drug substance. For an AAV vector with increased HMW by SEC we show presents as a low molecular weight peak on SV-AUC, with a higher DNA content than the full capsid. Use of pseudo-absorbance and software tools to increase throughput and ultra-low loading concentrations to reduce sample requirements are also discussed.

5:30 Close of Cell Therapy CMC and Analytics Conference

WEDNESDAY, AUGUST 17

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Back Bay A

ADVANCES IN AUTOLOGOUS CELL THERAPY MANUFACTURING

7:55 Chairperson's Opening Remarks
Dominic Clarke, PhD, ISCT Process & Product Committee Co-Chair & CTO, Cell and Gene Therapy, Discovery Life Sciences

8:00 Making the Case for Autologous Manufacturing
Knut Niss, PhD, CTO, Mustang Bio, Inc.
Autologous manufacturing has long been thought of as resource-intense and therefore leading to high cost. As a result, autologous therapies are coming with a high price that is justified by exceptional efficacy. However, with the emergence of allogeneic therapies based on gene editing technologies, there is significant pressure on autologous therapies to become more cost efficient. This presentation will take a look at all aspects of potential cost savings.

8:30 KEYNOTE PRESENTATION: Advances in Cell Therapy Manufacturing
Julie G. Allickson, PhD, Michael S. and Mary Sue Shannon Family Director, Mayo Clinic Center for Regenerative Medicine, Member, ISCT

9:00 Process Development for TCR T Cell Therapies: Begin with the End in Mind
Mamta Kalra, PhD, Director, CMC and Process Development, Immatics US, Inc.
Manufacturing autologous TCR-engineered T cell products requires multiple manipulations, specialized equipment, critical raw material, and supply chain logistics. Successful manufacturing with variable patient material warrants well-defined and thoroughly optimized process from the beginning. Key considerations such as quality of starting material, expansion platform, media and cytokine selection, optimized gene delivery, and manufacturing duration are critical for success in early stages and laying the foundation for advancement to pivotal and commercialization.

9:30 Metabolic Sensing Artificial Intelligence & Control, Base for Efficient Autologous Manufacturing, the ADVA X3 Case Study
Ohad Karnieli, PhD, CEO & Founder, Adva Biotechnology
Maintaining high cell quality is still the challenge in autologous cell therapies manufacturing, Manual manufacturing process is labour-intensive & oblivious to occur within the cell culture.
The ADVA-X3 platform learns the cell's metabolic behavior using Multiparameter sensing and Artificial Intelligence, adapts, controls & optimizes the physical conditions to reach maximal potential from each donor, based on pre-set parameters and protocol.

10:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

10:40 NCI Advances Cancer Cell Therapy by Targeting Manufacturing Hurdles
Rachelle Salomon, PhD, Program Director, National Cancer Institute
Recently the NCI has invested in establishing centralized manufacturing for cell therapy products to support multi-center clinical trials. This presentation will highlight the challenges of developing manufacturing and CMC support for autologous cell therapy products including: tech transfer, use of closed manufacturing systems, aseptic validations, process qualification, QC/QA rapid release, cryopreservation, and chain of custody logistics.

11:10 NK and CAR-NK Processing Development
Dongfang Liu, PhD, Associate Professor, Director Immunoassay Development, Pathology & Immunology & Lab Medicine, Rutgers University

11:40 PANEL DISCUSSION: Latest Challenges in Cell Therapy Manufacturing
Moderator: Dominic Clarke, PhD, ISCT Process & Product Committee Co-Chair & CTO, Cell and Gene Therapy, Discovery Life Sciences
- What have we learned from first-generation CAR T therapies?
- Improvements in autologous therapies
- Reducing costs
- Is allogeneic the next step, or *in vivo*?

Panelists:
Julie G. Allickson, PhD, Michael S. and Mary Sue Shannon Family Director, Mayo Clinic Center for Regenerative Medicine, Member, ISCT
Mamta Kalra, PhD, Director, CMC and Process Development, Immatics US, Inc.
Dongfang Liu, PhD, Associate Professor, Director Immunoassay Development, Pathology & Immunology & Lab Medicine, Rutgers University
Rachelle Salomon, PhD, Program Director, National Cancer Institute
Ohad Karnieli, PhD, CEO & Founder, Adva Biotechnology

12:10 pm Enjoy Lunch on Your Own

12:40 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

ENABLING AND IMPROVING PROCESS DEVELOPMENT: ISCT SESSION

1:25 Chairperson's Remarks
Dominic Clarke, PhD, ISCT Process & Product Committee Co-Chair & CTO, Cell and Gene Therapy, Discovery Life Sciences

1:30 Panel Discussion: ISCT Sponsored Session: Enabling Process Development for Scalable Manufacturing: Getting It Right from the Start
Moderator: Dominic Clarke, PhD, ISCT Process & Product Committee Co-Chair & CTO, Cell and Gene Therapy, Discovery Life Sciences
Developing a consistent and scalable manufacturing platform for cell & gene therapies has proven to be challenging. Whether autologous or allogeneic, the process is the product. Implementing the right reagents and equipment coupled with the appropriate process analytics early is critical, but presents

ongoing questions given the abundance of options and still evolving industry maturity. This panel session of industry experts will discuss lessons learned from collective process development experience.

Panelists:

Julie G. Allickson, PhD, Michael S. and Mary Sue Shannon Family Director, Mayo Clinic Center for Regenerative Medicine, Member, ISCT

Shannon Eaker, PhD, Chief Technology Officer, Xcell Biosciences

John Fink, Co-Chair PPD ISCT, General Manager, Liquid Handling, Perkin Elmer

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

Ohad Karnieli, PhD, CEO & Founder, Adva Biotechnoogy

2:30 CO-PRESENTATION: Critical Challenges in Manufacturing and Commercialization of Cell Therapies

Nikhil Tyagi, Director, Cell Therapy Process Development, Process Development, Center for Breakthrough Medicines

John Lee, PhD, Head of Cell Therapy Manufacturing, Cell Therapy, Center for Breakthrough Medicines

Despite promising clinical results and some commercial success, cell therapy remains a nascent industry facing supply chain, manufacturing, and delivery challenges that hamper broad adoption.

Advanced therapies CDMOs can help cell therapy developers overcome these hurdles through scalable "first-time right" process development that considers flexible sourcing, automated manufacturing, and integrated release testing to unlock the commercial and therapeutic value of this paradigm-changing modality.

3:00 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

ROOM LOCATION: Constitution A&B

PLENARY KEYNOTE: LEADING TO TOMORROW'S ADVANCES

3:50 Plenary Introduction

Nathalie Clément, PhD, CEO, Unicorn Consultations, LLC

4:00 New Therapeutic Modalities and Moore's Law in Biomanufacturing

Hari Pujar, PhD, Operating Partner, Flagship Pioneering; COO, Tessera Therapeutics

As we have embarked on newer, more complex modalities, biomanufacturing has appeared to stumble. The talk will chronicle the advancements in biomanufacturing of different therapeutic modalities, drawing parallels to semiconductor chip manufacturing, and establishing the rightful and bright future of biomanufacturing.

4:30 Cell and Gene Therapy (R)evolution

Mercedes Segura Gally, PhD, Vice President, Process Development, ElevateBio

The concept of gene therapy arose nearly half a century ago. Turning that concept into a therapeutic reality required years of scientific discovery, technological advances, and pioneering efforts, culminating in several regulatory approvals. These success stories paved the road for a second wave of advanced therapies. Compared with traditional biologics, cell and gene therapy products pose unique challenges. This presentation aims to summarize the progress made in recent years.

5:00 Networking Reception in the Exhibit Hall with Poster Viewing (Grand Ballroom)

6:00 Close of Day

THURSDAY, AUGUST 18

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Constitution B

LENTIVIRAL MANUFACTURING

7:55 Chairperson's Remarks

Michael Mercaldi, PhD, Senior Director, Downstream Process Development, Oxford Biomedica Solutions

8:00 A Robust and Scalable Platform Process for GMP Manufacturing of Lentiviral Vectors

Bojiao Yin, PhD, Director, Vector Process Development & Manufacturing, ElevateBio

We describe here a well-established platform process for LV production based on transient transfection of serum-free cells grown in suspension. Both upstream and downstream processes are highly optimized to achieve optimal vector yields and significant decrease in the impurities (host cell protein/DNA, plasmid DNA). The compatibility of this platform process has been evaluated with multiple CAR/TCR genes while the robustness is demonstrated in reproducible runs at pilot scale.

8:30 Manufacturing Lentiviral Vectors for *in vivo* CAR T Cell Therapy

Sarah Gould, PhD, Associate Director, Manufacturing Science & Technology, Umoja Biopharma

Umoja aims to transform cancer care by creating an off-the-shelf, direct injection lentiviral vector (LVV) drug product for *in vivo* CAR T cell generation and expansion. We will present our approach to reproducible and scalable manufacturing of high-quality LVV, with special focus on expediting process development with risk-based quality documentation and impurity clearance at key process unit operations to meet ambitious final specifications.

9:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

9:15 Poster Award Presented in the Exhibit Hall

ROOM LOCATION: Back Bay A

MANUFACTURING NOVEL CELL THERAPIES

9:25 Chairperson's Remarks

Patrick J. Hanley, PhD, Assistant Research Professor, Pediatrics & Director, GMP for Immunotherapy & Cellular Lab Therapy, Children's National Health System

9:30 The Facility Evolution of Small-Scale Cell Therapy
Peter Walters, Director of Advanced Therapies, CRB
This presentation will provide: Overview on the evolution of autologous facility design: the history, what's happening right now, and what's next; effective and efficient design considerations for scaling out autologous cell therapy; and facility strategies to save on square footage and cost

10:00 Optimizing Antigen-Specific T Cells for Off-the-Shelf Use
Patrick J. Hanley, PhD, Assistant Research Professor, Pediatrics & Director, GMP for Immunotherapy & Cellular Lab Therapy, Children's National Health System
Allogeneic, off-the-shelf antigen-specific T cells have demonstrated promise by several academic and industry groups. However, manufacturing considerations for antigen-specific T cells vary from small-scale autologous or donor-directed T cells to large-scale off-the-shelf antigen-specific T cells. Here we will outline a number of considerations for manufacturing off-the-shelf T cells such as donor selection, scale, methods of characterization and assessing potency, and product testing and release.

10:30 Universal OmniCAR T Cells and CellPryme-M, Two Methods to Optimize CAR T Manufacturing, Cost Economics, and Performance.
Daniel A Shelly, PhD, VP Bus Dev & Alliances, Bus Dev & Alliances, Prescient Therapeutics Pty Ltd
CellPryme-M enhances adoptive cell therapy performance by shifting immune cells to a central memory phenotype, improving persistence, and increasing the ability to find and penetrate tumors. CellPryme-M is applied during the final 24 hours of CAR-T manufacture. The OmniCAR Universal Immune Receptor (UIR) platform significantly reduces CMC costs through creation of a single master CAR-Immune cell for ANY indication/target.

11:00 Breakout Discussions
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 Challenges for Cell-Based Therapies
Patrick J. Hanley, PhD, Assistant Research Professor, Pediatrics & Director, GMP for Immunotherapy & Cellular Lab Therapy, Children's National Health System

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

12:30 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

AI, MACHINE LEARNING, AND DIGITALIZATION

1:05 Chairperson's Remarks
Patrick J. Hanley, PhD, Assistant Research Professor, Pediatrics & Director, GMP for Immunotherapy & Cellular Lab Therapy, Children's National Health System

1:10 Optimizing Cell Therapy Manufacturing
Chase D. McCann, MSPH, PhD, Cell Therapy Lab Lead, Manufacturing, Children's National Hospital

1:40 FEATURED PRESENTATION: AI, Digitalization in Cell Therapy Manufacturing
Krishnendu Roy, PhD, Robert A. Milton Chair & Director, Center for Cell Manufacturing & Center for Immuno-Engineering, Georgia Institute of Technology
This presentation examines state-of-the-art PAT implementation on standard physicochemical parameters in biopharmaceutical operations and considers potential cell therapy-related parameters that may be instrumental in overcoming the challenges of the current cell therapy manufacturing landscape. Current innovations applied to the field, such as high-throughput and high-dimensional analyses, machine learning, and novel sensor technologies, are also discussed.

2:10 Stem Cell Biology, Laser Physics, and Machine Learning: A Multidisciplinary Approach to Personalized Cell Therapy Production
Marinna Madrid, PhD, Chief Product Officer & Co-Founder, Cellino Biotech
Cellino's vision is to make personalized regenerative medicines viable at large scale. Cellino's platform combines label-free imaging and high-speed laser editing with machine learning to automate cell reprogramming, expansion, and differentiation in a closed cassette format, enabling thousands of patient samples to be processed in parallel in a single facility.

2:40 Refreshment Break in the Exhibit Hall & Last Chance for Poster Viewing (Grand Ballroom)

3:10 CO-PRESENTATION: Opportunities of Hybrid Model-Based Reinforcement Learning for Cell Therapy Manufacturing Process Control
Wei Xie, PhD, Assistant Professor, Mechanical & Industrial Engineering, Northeastern University
Zheng Li, PhD, Senior Manager, Manufacturing Science and Technology, Genentech
Driven by the key challenges of cell therapy manufacturing, including high complexity, high uncertainty, and very limited process observations, we propose a hybrid model-based reinforcement learning (RL) to efficiently guide process control.

3:40 Applying Lean Manufacturing Techniques in Bioprocessing Settings
Daniel Fleming, Continuous Improvement Manger, Lean Consulting, GBMP
The LEAN manufacturing and management model, developed from the Toyota Production System (TPS), has triggered major transformations in various manufacturing industries. In this presentation, we provide practical examples to highlight the areas where such benefits can be achieved.

4:40 Close of Summit

STREAM #5
ANALYTICAL & QUALITY

The Summit's 2022 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 15-16

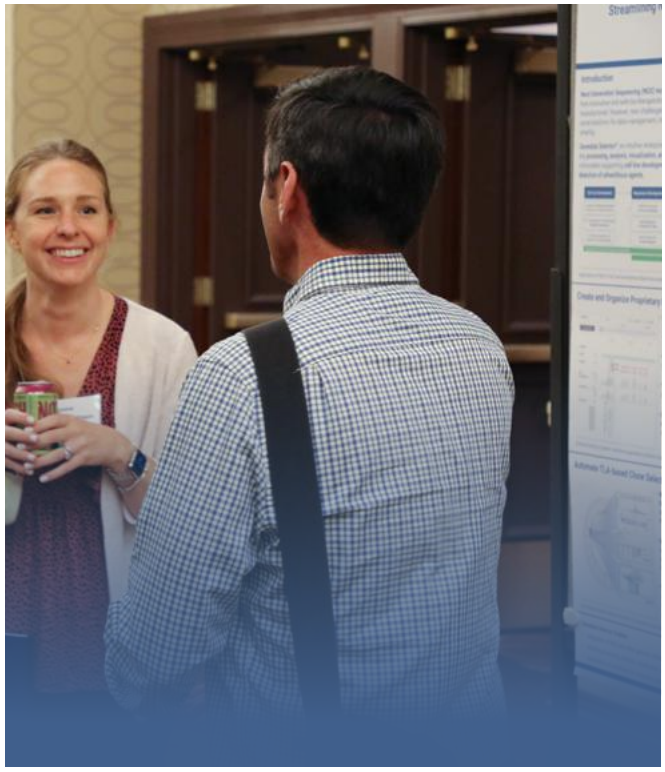
Host Cell Proteins

View Program »

AUGUST 17-18

Accelerating Analytical Development

View Program »



MONDAY, AUGUST 15

9:00 am Main Conference Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Back Bay D

HIGH-RISK HOST CELL PROTEINS

9:55 Chairperson's Opening Remarks
Gang Xiao, MSc, Senior Scientist, Process Development, Amgen, Inc.

10:00 KEYNOTE PRESENTATION: High-Risk Host Cell Proteins: A Multi-Company Collaborative View
Nisha Palackal, PhD, Director, Protein Biochemistry, Regeneron Pharmaceuticals, Inc.
Host cell proteins (HCPs) are process-related impurities that may co-purify with drug products and can be problematic. Problematic HCPs can be considered high-risk and can include those that are immunogenic, difficult to purify, and/or degrade product molecules and excipients. These HCPs can be classified into different risk categories. Recommendations for monitoring and/or eliminating the problematic impurity from the process that would be beneficial to the biopharmaceutical industry are provided.

10:30 Assessing Methods for Low Abundant HCP Detection
Thomas Waerner, PhD, Senior Principal Scientist & Laboratory Director, Analytical Development & Quality Control, Boehringer Ingelheim Pharma GmbH & Co. KG, Germany
It is well known that some HCPs with very little abundance have a strong impact on product quality. One example is the degradation of the formulation component polysorbate accompanied by increased product aggregation. The detection of those HCPs is a prerequisite for developing a suitable HCP depletion process. Here we assess our research on methods for detection of low-abundant HCPs by MS/MS and enrichment by hexapeptides or antibodies (IAC).

11:00 Characterization of a Host Cell Protein that Causes Product Cleavage by Nano LC-MS
Gang Xiao, MSc, Senior Scientist, Process Development, Amgen, Inc.
Host cell proteins are process-related impurities, and for those that are immunogenic, enzymatically, or biologically active, they are high-risk and often impact patient safety and product stability. A case study is presented to demonstrate that such an HCP was identified and confirmed as the cause of product protein cleavage in one of the processes aimed at high yield, and an effective mitigation strategy was implanted based on our finding.

11:30 Strategies for Successful Host Cell Protein Monitoring
Charlotte Coenders, Manager Business Development, Business Development, BioGenes GmbH
The ELISA is still the gold standard to monitor HCP reduction during drug purification and for product release testing. The performance of a HCP ELISA strongly depends on the respective polyclonal antibodies, which needs to match the HCP composition in terms of absolute abundance and affinity for each individual HCP. A sophisticated ELISA development strategy supported by orthogonal methods for reagent characterization is essential for robust and reproducible ELISA-based HCP monitoring.

12:00 pm Enjoy Lunch on Your Own

LC-MS IN HCP DETECTION AND CONTROL

12:50 Chairperson's Remarks
Feng Yang, PhD, Principal Scientist & MS Core Group Leader, Protein Analytical Chemistry, Genentech, a member of Roche Group

12:55 Versatile LC–MS-Based Workflow with Robust 0.1 ppm Sensitivity for Identifying Residual HCPs in Biotherapeutic Products
Feng Yang, PhD, Principal Scientist & MS Core Group Leader, Protein Analytical Chemistry, Genentech, a member of Roche Group
Highly active hydrolytic enzymes at sub-ppm levels can negatively impact the shelf life of drug products but are challenging to identify by LC-MS/MS due to the high dynamic range between HCPs and biotherapeutic proteins. We have employed strategies that increased the sensitivity for HCP identification by 10- to 100-fold over previous reports and shown the robustness as low as 0.1 ppm for identifying HCPs (34.5 to 66.2 kDa MW).

1:25 HCP Analysis by LC-MS and ELISA in Process Development
Roger Liu, PhD, Scientist, Bristol Myers Squibb Co.
Host cell proteins (HCPs) are impurities in the biological process development. HCP ELISA has been widely used in process development for detecting and quantifying HCPs owing to its high sensitivity and throughput. This presentation describes the implementation of a mass spectrometry-based HCP workflow in the process development of antibody-based biologics and demonstrates the strength and weakness of HCP analysis by mass spectrometry-based methods.

1:55 Next Gen Tools for Bioprocessing: Better Data Faster - New Tools for Biologics Characterization and QA/QC Testing
Jerry Peng, PhD, Senior Product Specialist, PerkinElmer
Characterization and quantification of biologics, is essential to ensure that critical quality attributes are achieved. It is valuable to utilize strong analytical methods and instrumentation to help researchers monitor biologic production and accurately characterize samples. PerkinElmer's LabChip GXII Touch provides high throughput characterization of proteins and nucleic acids in as little as 42 seconds, delivering data comparable to other methods of quantitation with as much as 70x increase in throughput.

2:25 Networking Refreshment Break (Grand Ballroom Foyer)

ROOM LOCATION: Back Bay B

2:40 Detection and Quantitation of Host Cell Proteins in Monoclonal Antibody Drug Products Using Automated Sample Preparation and Data-Independent Acquisition LC-MS/MS
Jonathan Bones, PhD, Principal Investigator, Characterisation and Comparability Laboratory, National Institute for Bioprocessing Research and Training (NIBRT), Ireland
Data from LC-MS analyses of host cell proteins focusing on quantitation aspects and application to recombinant proteins and AAV-based gene therapy will be presented. Additionally, combination with ribosomal footprint profiling (Ribo-seq) to improve the depth and quality of the database used for MS data searching will also be discussed. Using this approach, we identified microprotein-based HCPs present in commercial drug products and investigated their behaviour during cell culture.

3:10 Advanced LC-MS/MS Workflows for HCP Quantification, Including Optimized Standards and Data Independent Acquisition (DIA) MS Combined with Ion Mobility Separation

Christine Carapito, PhD, Co-Head of the BioOrganic Mass Spectrometry Laboratory, CNRS and University of Strasbourg, France

The implementation of new standards for robust and accurate quantification of Host Cell Proteins (HCP) will be presented. The potentialities of Data Independent Acquisition for HCP profiling will be benchmarked against commonly used targeted and Data Dependent Acquisition methods. Finally, benefits of an additional ion mobility separation in the LC-MS/MS workflow will be assessed on various instrumental platforms (High-Field Asymmetric-Waveform Ion-Mobility Spectrometry (FAIMS) and Trapped Ion Mobility Spectrometry (TIMS)).

3:40 Session Break and Transition to Plenary Keynote

ROOM LOCATION: Constitution A&B

PLENARY KEYNOTE: SOLVING TODAY'S CHALLENGES

4:20 Plenary Introduction

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

4:30 Lessons Learned from the Pandemic: mRNA-LNP Vaccine Development

Nicholas Warne, PhD, Vice President, Pharmaceutical Research and Development, BioTherapeutics Pharmaceutical Sciences, Pfizer Inc.

The speed and scale of industry response to the COVID pandemic was unprecedented, ultimately leading to the availability of several vaccines in under a year. This presentation will discuss the approach taken by Pfizer, with their partner BioNTech, in the development, manufacture, and distribution of the vaccine drug product while reflecting on lessons that may, or may not, be applicable to future product development.

5:00 Advances in Vaccine Formulation and Stability

David B. Volkin, PhD, Distinguished Professor, Pharmaceutical Chemistry, University of Kansas, Lawrence

This presentation will provide an overview of analytical characterization and formulation development considerations for new vaccine candidates targeted for use in low- and middle-income countries (LMICs). Illustrative case studies with vaccine candidates (e.g., live-virus, adjuvanted recombinant protein) will highlight implementing state-of-the-art stability-indicating assays to enable development of stable formulations. Challenges with developing lower-cost formulations (e.g., multi-dose, combination, non-parenteral) to expand vaccine coverage in LMICs will also be discussed.

5:30 Welcome Reception in the Exhibit Hall with Poster Viewing (Grand Ballroom)

6:30 Close of Day

TUESDAY, AUGUST 16

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Back Bay D

EMERGING CHARACTERIZATION METHODS

7:55 Chairperson's Remarks

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

8:00 Single-Protein Immunoassays for Detection of Hitchhiker HCPs

Cullen Mason, PhD, Associate Director, Technical Development, Biogen

Custom reagents that are host-strain and process-specific can help ensure good characterization and clearance of HCP impurities. However, some non-immunogenic proteins will not be covered by the anti-HCP antibodies. Initial detection and identification of these HCPs requires the use of LC-MS, but protein-specific immuno-based methods are sometimes needed to support process development. A case study will be presented detailing the development and application of a single-protein immunoassay method.

8:30 Strategies for Controlling HCP Impurities

Erika M. Friedl, PhD, Quality Expert, Haematology & Transfusion Medicine, Paul Ehrlich Institute, Germany

The efficient removal of HCP impurities is a challenging objective during product development. For patient safety, the development and adaption of suitable HCP control strategies throughout the product life cycle are a measure to guarantee high-quality medicines on the market. The choice of the most appropriate HCP assay and the life cycle management of the critical reagents are important decision points during product development and for continuous product monitoring.

9:00 Optimization and Lessons Learned for HCP ELISA Coverage Using Affinity Extraction, Mass Spectrometry, and 2D-PAGE

Martha Stapels, PhD, Associate Director, Sanofi

For each platform-specific ELISA, it is beneficial to show that the ELISA antibodies recognize the most abundant HCPs in the purified protein drug as well as in upstream samples. The combination of affinity enrichment along with 2D PAGE and mass spectrometry is useful to demonstrate good coverage as well as individual abundant HCPs understanding. Practical tips and lessons learned in the development of these methods will also be discussed.

9:30 Advanced Orthogonal Methods to Fully Characterize Process HCPs

CYGNUS TECHNOLOGIES
part of Maravai LifeSciences

Jared Isaac, PhD, Associate Director Chromatography and Mass Spectrometry, Cygnus Technologies

In recent years we've learned that Host Cell Proteins (HCP) can cause problems with respect to patient safety, Drug Substance (DS) stability, and DS efficacy. It's more important than ever to fully understand the HCPs in your process. This talk will focus on using advanced methods immunoaffinity chromatography and mass spectrometry to fully characterize the Host Cell Protein ELISA as well as the individual HCPs in in-process and DS samples.

10:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

10:45 Breakout Discussions

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: New Directions for HCP Detection, Analysis, and Control
Abraham M. Lenhoff, PhD, AP Colburn Professor, Chemical & Biomolecular Engineering, University of Delaware

11:30 **Development of Custom HCP Immunoassays for Both Product Release and Process Support**
Emily Menesale, Scientist II, Analytical Development, Ultragenyx
Having an appropriate assay to detect HCP is critical in biopharmaceutical development, including the development of gene therapy products. To ensure accurate quantitation of HCP from your bioproduction process, process- or platform-specific HCP assays are preferred over generic assays. We describe the development of custom reagents for process-specific HCP assays, as well as the development of custom HCP assays on two different immunoassay platforms for product release and in-process support.

12:00 pm **Enhancing Host Cell Protein Detection in Protein Therapeutics Using HILIC Enrichment and Proteomic Analysis**
Qingyi Wang, PhD, Scientist, Bristol Myers Squibb Co.
We developed a new method for improving HCP detection in biotherapeutics, which relies on hydrophilic interaction chromatography for HCP enrichment followed by *in situ* concentration and digestion prior to LC-MS. Our results indicate that HILIC is able to separate most HCPs from the therapeutic proteins tested, and also improve sensitivity for a population of HCPs which are difficult to observe when using other LC-MS approaches, such as the native digestion method.

12:30 **POSTER HIGHLIGHT: Assessing the Immunogenic Potential of CHO Host Cell Proteins by Combining Native Fractionation, LC-MS and *In Vitro* Cell Assay**
Sherin Panikulam, Graduate Student, University of Applied Sciences & Arts, Northwestern Switzerland

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

1:30 **Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)**

PROBLEMS AND SOLUTIONS

2:10 **Chairperson's Remarks**
Stefano Menegatti, PhD, Assistant Professor, Chemical & Biomolecular Engineering, North Carolina State University

2:15 **Role of Particulates in HCP Persistence in mAb Bioprocessing**
Abraham M. Lenhoff, PhD, AP Colburn Professor, Chemical & Biomolecular Engineering, University of Delaware

2:45 **Analysis of Fouling and Breakthrough of Process-Related Impurities during Depth Filtration Using Confocal Microscopy**
Maria Parau, Researcher, University College London, United Kingdom
Antibody titre improvement has led to process intensification, which has challenged DSP with high cell densities and associated process-related impurities. Breakthrough studies and a novel confocal imaging technique have been applied to Millistak+ HC (cellulose) and SP (synthetic) depth filters to show differences in performance and foulant distribution at different feed viability and loading. Protein A resin lifetime studies show higher fouling, particularly DNA, when SP filters are used.

3:15 **LigaGuard: A Novel Adsorbent for Host Cell Protein (HCP) Removal *via* Flow-Through Affinity Chromatography**
Stefano Menegatti, PhD, Assistant Professor, Chemical & Biomolecular Engineering, North Carolina State University
Residual HCP titer is a critical quality attribute of biotherapeutics – from mAbs to viral vectors for gene therapy and vaccines. Specific HCPs, however, resist removal by chromatography and persist through the purification pipeline. Responding to this challenge, we developed LigaGuard, an adsorbent designed to remove HCPs in flow-through mode: CHO HCP removal in mAb manufacturing, HEK293 HCP removal in viral vector manufacturing, and VERO HCP removal in vaccine manufacturing.

3:45 **Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)**

4:30 **Identification of Active Enzymes for Polysorbate Degradation in Biotherapeutics by Activity-Based Protein Profiling**
Shawn Li, PhD, Principal Scientist, Analytical Research and Development (AR&D) Mass Spectrometry, Merck & Co., Inc.
Enzymatic activity from residual host cell enzymes such as lipases and esterases plays a major role in polysorbate degradation. Their high activity, often at very low concentration, constitutes a major analytical challenge in the biopharmaceutical industry. In this study, we evaluated and optimized the activity-based protein profiling (ABPP) approach to identify active enzymes responsible for polysorbate degradation, which enables more meaningful polysorbate degradation investigations for biotherapeutic development.

5:00 **Best Practices for Internal Development of HCP Monitoring Kits**
Olivier Ducoudret, Senior Development Specialist, Quality Control, Macrogenics
Host cell proteins (HCPs) are a class of process-related impurities during the manufacturing of biologics. HCPs must be removed in biologics manufacturing to ensure final product purity, manufacturing robustness, and safety. HCP analysis is often performed using an enzyme-linked-immunosorbent-assay (ELISA) due to method sensitivity and ease of use. When using an ELISA kit for HCP monitoring best practices involve determining kit sensitivity (quantification and coverage), product dilutional linearity, and robustness.

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

WEDNESDAY, AUGUST 17

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Back Bay D

PLATFORMS AND WORKFLOWS

7:55 Chairperson's Opening Remarks
Elena A. Smith, PhD, Deputy Director and Quality Analytical Expert, Quality, Sanofi

8:00 Navigating International Requirements for Analytical Testing Strategies
Elena A. Smith, PhD, Deputy Director and Quality Analytical Expert, Quality, Sanofi
Advancing a product, from its conception in research laboratories to commercialization, requires a series of analytical activities that need to evolve as the product goes through various stages and expands to various markets globally. This presentation intersects and balances Science, Quality and Regulation, and aim to help navigate complexity of the international environment.

8:30 Analytical Characterization of Novel Modalities: Strategy, Methodology, and Case Studies
Guodong Chen, PhD, Scientific Director, Bristol Myers Squibb Co.
Since the introduction of the first recombinant DNA-derived insulin, biotherapeutics market has shown a healthy growth. Novel modalities such as multi-specific antibodies, fusion proteins, and gene therapy have gained significant momentum as innovative therapies. Integrated analytical strategy is required to advance attribute sciences for such complex molecules. This presentation will discuss recent developments in protein analytics for characterizing key attributes, including phase-appropriate analytical strategy, orthogonal methodology, and case studies.

9:00 KEYNOTE PRESENTATION: Analytical Support for Accelerated Development Timelines
Stephan O. Krause, PhD, Executive Director, Analytical Science and Technology, Cell Therapy Quality, Bristol Myers Squibb Co.
Strategies for lifecycle steps for APT methods are conceptually reviewed within the framework of supporting CMC development acceleration. Reduced method qualification, transfer, and validation studies could be performed, provided that the initially-validated test method remains unchanged. A detailed case study is used to illustrate considerations for the initial method validation and subsequent APT verification studies. Suggestions are provided for the submission of APT information in regulatory filings.

9:30 Tackling the Glycan Isomer Challenge for Your BioTherapeutics with MOBIE
James Atwood, Vice President of Omics Business, MOBILion Systems
N-linked glycosylation is a critical influencer of pharmacological function, safety and efficacy. As minor changes to production processes can influence both glycan structure and composition, glycans must be monitored. However, characterization of N-linked glycosylation is analytically challenging due to innate glycan microheterogeneity. Herein we report the use of the MOBIE™ high-resolution ion mobility system from MOBILion, to improve released N-linked glycan analysis and demonstrate applicability for profiling complex glycan isomeric mixtures.

10:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

EMERGING METHODS AND INSTRUMENTS

10:40 Multiplexing Antibody Potency Assays by Converting Nanoparticles
Jin-Hee Han, PhD, Associate Principal Scientist, Merck & Co., Inc.
We developed a homogeneous particle-based immunoassay using upconverting nanoparticles (UNCPs) for multiplexing potency of two different monoclonal antibodies (mAb). In this study, the recombinant human protein for each target mAb was conjugated to a corresponding UCNP. Two different UCNPs-immunocomplexes transferred energy for creating detection fluorescent signals separately with signal excitation at 980 nm. Harvested fluorescent signals at two different wavelengths were used for creating dose-response curves for the target mAb.

11:10 qPCR Titer Assay Miniaturization and Automation Method Development
William Beyer, Research Associate, Ultragenyx
This presentation describes the development of an automation workflow for qPCR titer assay using traditional liquid handler and contactless liquid dispensing technology. Combination of these two technologies allowed the cost reduction of the assay by 5-fold via miniaturization, improving assay performance and overall improvement in the operation efficiency by 4-fold in comparison to the manual assay. Case examples will be provided with results from validation runs of the assay.

11:40 USP Standards to Support Multi-Attribute Methods
Diane McCarthy, PhD, Senior Director, Science & Standards, Global Biologics, US Pharmacopeia
Use of multi-attribute methods (MAM) for analytical testing of biotherapeutics is increasing due to their potential to improve efficiency and provide detailed information on quality attributes. Based on stakeholder input, USP established an expert panel to develop a chapter on best practices for MAM. This presentation will provide an overview of the proposed chapter contents and an update on other efforts to support biopharmaceutical quality and consistency using MAM.

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 (Grand Ballroom)

AUTOMATION

1:25 Chairperson's Remarks
Young-ok You, PhD, Scientist, Analytical Sciences, Macrogenics

1:30 Miniaturization Enables a Quick & Concise Comparison of Multiple Assay Parameters
Richard Rodriguez, Senior Systems Specialist, Genentech, Inc.
A biological assay for use in repetitive testing must be robust. Multiple parameter assays (MPAs), which can curtail assay development time, are achieved by miniaturizing volumes, utilizing denser formatted 384-well plates and managing conditions in a concurrent manner rather than consecutive.

Automated methods capable of running distinctive MPAs can be developed as "tools." A library of these tools can then be used for future assay development projects.

2:00 Developing of Peptide Mapping Method for High-Throughput Analysis Using an Automated Liquid Handler System

Young-ok You, PhD, Scientist, Analytical Sciences, Macrogenics

Peptide mapping is an indispensable tool for monitoring post-translational modifications (PTM) of biological drugs. The traditional manual methods for peptide mapping are tedious and time-consuming. We have developed peptide mapping methods for monoclonal antibodies and novel bispecific DART molecules using an automated Liquid Handler system. The robustness, reliability, and reproducibility of these methods will be presented. These results will contribute to implementing the multi-attribute monitoring (MAM) workflows.

2:30 Magnetic Resonance Multi-Attribute Methods for Biotherapeutics Characterization, PAT and QC

Kate Holub, Market Sales Manager, Pharma – North America, Bruker

Nuclear magnetic resonance is a technique recognized for being information rich and more sensitive towards changes when compared to other biophysical techniques used to address higher order structure of biologics1,2. The introduction of benchtop flow NMR, integrated into PAT software, makes NMR important for (bio)process monitoring and control3. We show examples of magnetic resonance for biologics/biosimilars analysis, from NMR in R&D4 to benchtop EPR, FT-NMR and TD-NMR in bioPAT and QC.

3:00 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

ROOM LOCATION: Constitution A&B

PLENARY KEYNOTE: LEADING TO TOMORROW'S ADVANCES

3:50 Plenary Introduction

Nathalie Clément, PhD, CEO, Unicorn Consultations, LLC

4:00 New Therapeutic Modalities and Moore's Law in Biomanufacturing

Hari Pujar, PhD, Operating Partner, Flagship Pioneering; COO, Tessera Therapeutics

As we have embarked on newer, more complex modalities, biomanufacturing has appeared to stumble. The talk will chronicle the advancements in biomanufacturing of different therapeutic modalities, drawing parallels to semiconductor chip manufacturing, and establishing the rightful and bright future of biomanufacturing.

4:30 Cell and Gene Therapy (R)evolution

Mercedes Segura Gally, PhD, Vice President, Process Development, ElevateBio

The concept of gene therapy arose nearly half a century ago. Turning that concept into a therapeutic reality required years of scientific discovery, technological advances, and pioneering efforts, culminating in several regulatory approvals. These success stories paved the road for a second wave of advanced therapies. Compared with traditional biologics, cell and gene therapy products pose unique challenges. This presentation aims to summarize the progress made in recent years.

5:00 Networking Reception in the Exhibit Hall with Poster Viewing (Grand Ballroom)

6:00 Close of Day

THURSDAY, AUGUST 18

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Back Bay D

DIGITAL METHODS IN ANALYTICAL DEVELOPMENT

7:55 Chairperson's Remarks

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

8:00 Predicting Antibody Developability Profiles through Early-Stage Discovery Screening

Yao Yu, Senior Scientist, Protein Sciences, Merck Research Laboratories

The current race to develop better biologics faster has led biopharmaceutical companies into optimizing all drug discovery and development processes. As part of this effort, machine learning algorithms are being developed to identify correlations between amino acid sequences and physicochemical properties. The talk will focus on the platform Merck MSD is currently developing to serve as the interface between our scientists and machine learning algorithms for *in silico* developability assessments.

8:30 Process Analytical Technologies – Advances in Bioprocess Integration to Transition to Digital Manufacturing

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

Process Analytical Technology (PAT) instruments include analyzers capable of measuring physical and chemical process parameters and key attributes with the goal of optimizing process controls. PAT probes and sensors are intended for understanding bioprocesses with the goal to control quality and consistency at all stages of product manufacturing to achieve quality-by-design (QbD) and real-time release. The advantages, challenges, and future development will be discussed.

9:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

9:15 Poster Award Presented in the Exhibit Hall

PROCESS CHARACTERIZATION & CONTROL

9:30 Glycosylation Profiling Using a Novel High-Throughput Method and Highly-Sensitive Derivatives

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

Glycosylation is a critical quality attribute of biopharmaceuticals. Production from bioprocesses results in heterogenous glycan profiles that necessitate rapid, sensitive, and high-throughput analysis. Liquid chromatography (HPLC or UPLC) has become a standard method of separation and analysis, often with a time-consuming workflow for glycan

derivatization. We present an innovative, streamlined, 96-well-plate-based platform of derivatization that is rapid and sensitive. Applications and advantages over existing methods will be shown.

10:00 High-Throughput Peptide Mapping for Process Characterization and Product Control

Adam Evans, PhD, Principal Scientist, Analytical Development, Janssen Research and Development

Product quality attributes of protein therapeutics that impact safety or efficacy must be monitored to ensure product quality. Post-translational modifications, sequence variants, and glycosylation can be monitored by peptide mapping using mass spectrometry. We have developed a high-throughput peptide mapping strategy that characterizes multiple attributes to support process development, process characterization, and commercial product release/ stability testing in QC.

10:30 CO-PRESENTATION: Implementation of ELISA-Alternative Assays to Support Upstream and Downstream Gene Therapy

GYROS PROTEIN Technologies

Adam Hejmowski, Team Leader of BioProcess Analytics, PALL Corporation

Ellen Lee Lee, PhD, Field Application Sceintist, Gyros Protein Technologies

Implementation of the Gyrolab xPlore platform for R&D gene therapy was used as a valuable training opportunity for a newly assembled team working with monoclonal antibodies (mAbs), adeno-associated virus (AAV), and lentivirus (LV) focusing on host cell protein, p24 content, and IgG titer analysis, all ready-made kits for the xPlore. The recent assays Adam's team has performed, and lessons learned when transitioning from manual counterpart assays are presented.

11:00 Breakout Discussions

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: Opportunities and Challenges in Analytical Method Lifecycle Management (AMLM)

Chengdong (Jason) Xu, PhD, Senior Scientist, Merck & Co., Inc.

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

12:30 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

1:05 Chairperson's Remarks

Chengdong (Jason) Xu, PhD, Senior Scientist, Merck & Co., Inc.

1:10 Development of Robust and QC Friendly Compact Mass Spectrometer-Based Peptide Mapping Platform for Biologics Process Control

Chengdong (Jason) Xu, PhD, Senior Scientist, Merck & Co., Inc.

Increased use of quality-by-design strategies creates the need to quantify post-translational modifications in the primary structure of a biological therapeutic protein. We have developed a compact mass spectrometer-based platform for monitoring both specific and multiple attributes within a monoclonal antibody. By using "QC-friendly" MS-technologies, the method can analyze >250 samples per week and be easily used by a non-MS expert, demonstrating its potential in both GMP and development environments.

1:40 Modeling for rAAV Process Characterization and Design

Tam Nguyen, PhD Candidate, Massachusetts Institute of Technology

We constructed a mechanistic model based on the published understanding of the underlying biology and existing data to elucidate the mechanisms and bottlenecks of rAAV synthesis in HEK293 suspension-adapted cells. Through model analysis, we designed a multi-stage transfection method that successfully increased the ratio of full to total capsids in the viral harvest without compromising the viral titer.

PROBLEMS AND SOLUTIONS

2:10 Critical Quality Attributes Risk Assessment for Recombinant Adeno-Associated Virus Vector

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. In-depth characterization methods were developed to assess recombinant Adeno-Associated Virus vector quality attributes. A case study of the risk assessment approach to define appropriate critical quality attributes will be presented.

2:40 Refreshment Break in the Exhibit Hall & Last Chance for Poster Viewing (Grand Ballroom)

3:10 Analytical Challenges at the 11th Hour

Christina Vessely, PhD, Senior Consultant, CMC Analytics & Formulation Development, Biologics Consulting Group, Inc.

This session will focus on ways to accelerate analytical development to support faster development timelines. However, activities often performed during Phase 3, as assay validation, elucidation of structure and characterization of impurities, can sometimes generate surprises. This presentation is intended to provide recovery and bridging strategies when deficiencies are discovered late in the development lifecycle.

3:40 Implementing High-Throughput Analytics for Accelerated Biologics Development

Sophia Levitskaya-Seaman, PhD, Process Analytics Group Leader, Biopharmaceutical Development, MacroGenics, Inc.

Monoclonal antibodies and novel bispecific DART molecules are being developed for a variety of indications including immuno-oncology. Sensitive, accurate, and high-throughput analytical techniques enable faster development timelines for therapeutic molecules. An overview of different approaches, methodologies, and supporting software for high-throughput process analytics and related challenges will be presented as case studies.

4:10 Implementation of a Fully Automated Walk-Up Residual DNA qPCR Workflow

Michele Shannon, Investigator, GlaxoSmithKline

Clearance of residual host DNA is an important part of the biopharmaceutical process as host DNA can pose a potential risk to the patient. Using the KingFisher Presto integrated into a Hamilton liquid handling system, we have automated the entire residual DNA assay from sample preparation through qPCR plate preparation, significantly reducing FTE labor and allowing for a walk-up system for quicker turnaround and high-throughput for residual DNA results.

4:40 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 15-16

Rapid Methods to Assess Stability and Impurities in Biologics

View Program »

AUGUST 17-18

Formulation and Delivery of Biologics and New Modalities

View Program »

MONDAY, AUGUST 15

9:00 am Main Conference Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Back Bay B

9:55 Chairperson's Opening Remarks
Alex Dow, PhD, Associate Principal Scientist, Merck & Co., Inc.

10:00 FEATURED PRESENTATION: Impact of Stress Factors in Primary Packaging, Transportation, and Handling of Protein Drug Products
Twinkle Christian, MS, Senior Scientist, Amgen, Inc.
A biotherapeutic is exposed to a variety of stress factors throughout its production. The drug manufacturer is responsible to deliver a safe and efficacious product to the patient by maintaining the critical quality attributes of a drug product during manufacture, transport, and use. The scope of this talk is to assess the impact of packaging, transportation, and handling of drug product quality and discuss appropriate mitigations.

IMPROVING PREDICTION AND SCREENING

11:00 Autonomous Pipeline for Characterization of Biotherapeutics: Integrating Rapid Analytics with Rapid Informatics
Harsha Gunawardena, PhD, Senior Scientist, Mass Spectrometry, Janssen Pharmaceutical Companies of Johnson & Johnson
High-throughput screening tools are needed to triage product quality attributes of molecules and clones. We demonstrate high-throughput RapidFire-mass spectrometry analysis (i.e., 96-samples in ~ 20 mins) integrated with cloud-based protein informatics (~ 30 min/96 samples), to report and aggregate product quality attributes. This to our knowledge is the first demonstration of a complete end-to-end and scalable mass analysis solution that is hands-off, automated, and rapid (96 samples < 1 hr.).

11:30 Utilizing CHO-Endogenous Retrovirus Like Particles and Non-Infectious Minute Virus of Mice VLP's to Predict Viral Clearance During Process Development
David Cetlin, Senior Director, MockV Products, Cygnus Technologies
To determine viral clearance efficacy of biomanufacturing steps, viruses are "spiked" into in-process solutions, processed and analyzed for reduction. Due to the infectivity of these viruses, studies are conducted in BSL-2 facilities. Costs and logistics limit analysis during process development. Discussed in the presentation are results from several studies that utilized CHO-endogenous RVLP and non-infectious MVM VLP's as viral surrogates. The results demonstrate the feasibility and value of adding viral clearance predication to downstream process development and optimization.

12:00 pm Enjoy Lunch on Your Own

IMPROVING PREDICTION AND SCREENING, CONT.

12:50 Chairperson's Remarks
Alex Dow, PhD, Associate Principal Scientist, Merck & Co., Inc.

12:55 Machine Learning-Based Prediction of Single and Multiple Point Protein Mutations Stability Changes
Andrzej Kloczkowski, PhD, Professor, Pediatrics, Nationwide Children's Hospital
Accurate prediction of protein stability changes resulting from amino acid substitutions is of utmost importance in medicine to better understand which mutations are deleterious, leading to diseases, and which are neutral. Since conducting wet-lab experiments to get a better understanding of protein mutations is costly and time-consuming, computational tools based on machine learning are becoming promising alternatives to tedious and highly costly mutagenic experiments. We will review them here.

1:25 PANEL DISCUSSION: Formulation Development during the Pandemic: What Did We Learn?
Moderator: Björn Boll, Fellow Drug Product Design – Senior Expert for Particle Characterization and Analytics, ten23 health
Panelists:
Andrzej Kloczkowski, PhD, Professor, Pediatrics, Nationwide Children's Hospital
Sanket Patke, PhD, Associate Director, Sanofi
Harsha Gunawardena, PhD, Senior Scientist, Mass Spectrometry, Janssen Pharmaceutical Companies of Johnson & Johnson

1:55 Sponsored Presentation *(Opportunity Available)*

2:25 Networking Refreshment Break (Grand Ballroom Foyer)

LC-MS IN HCP DETECTION AND CONTROL

2:40 Detection and Quantitation of Host Cell Proteins in Monoclonal Antibody Drug Products Using Automated Sample Preparation and Data-Independent Acquisition LC-MS/MS
Jonathan Bones, PhD, Principal Investigator, Characterisation and Comparability Laboratory, National Institute for Bioprocessing Research and Training (NIBRT), Ireland
Data from LC-MS analyses of host cell proteins focusing on quantitation aspects and application to recombinant proteins and AAV-based gene therapy will be presented. Additionally, combination with ribosomal footprint profiling (Ribo-seq) to improve the depth and quality of the database used for MS data searching will also be discussed. Using this approach, we identified microprotein-based HCPs present in commercial drug products and investigated their behaviour during cell culture.

3:10 Advanced LC-MS/MS Workflows for HCP Quantification, Including Optimized Standards and Data Independent Acquisition (DIA) MS Combined with Ion Mobility Separation
Christine Carapito, PhD, Co-Head of the BioOrganic Mass Spectrometry Laboratory, CNRS and University of Strasbourg, France
The implementation of new standards for robust and accurate quantification of Host Cell Proteins (HCP) will be presented. The potentialities of Data Independent Acquisition for HCP profiling will be benchmarked against commonly used targeted and Data Dependent Acquisition methods. Finally, benefits of an additional ion mobility separation in the LC-MS/MS workflow will be assessed on various instrumental platforms (High-Field Asymmetric-Waveform Ion-Mobility Spectrometry (FAIMS) and Trapped Ion Mobility Spectrometry (TIMS)).

3:40 Session Break and Transition to Plenary Keynote

ROOM LOCATION: Constitution A&B

PLENARY KEYNOTE: SOLVING TODAY'S CHALLENGES

4:20 Plenary Introduction
James Warren, PhD, Vice President, Pharmaceutical Development, Ultragenyx Pharmaceutical

4:30 Lessons Learned from the Pandemic: mRNA-LNP Vaccine Development
Nicholas Warne, PhD, Vice President, Pharmaceutical Research and Development, BioTherapeutics Pharmaceutical Sciences, Pfizer Inc.
The speed and scale of industry response to the COVID pandemic was unprecedented, ultimately leading to the availability of several vaccines in under a year. This presentation will discuss the approach taken by Pfizer, with their partner BioNTech, in the development, manufacture, and distribution of the vaccine drug product while reflecting on lessons that may, or may not, be applicable to future product development.

5:00 Advances in Vaccine Formulation and Stability
David B. Volkin, PhD, Distinguished Professor, Pharmaceutical Chemistry, University of Kansas, Lawrence
This presentation will provide an overview of analytical characterization and formulation development considerations for new vaccine candidates targeted for use in low- and middle-income countries (LMICs). Illustrative case studies with vaccine candidates (e.g., live-virus, adjuvanted recombinant protein) will highlight implementing state-of-the-art stability-indicating assays to enable development of stable formulations. Challenges with developing lower-cost formulations (e.g., multi-dose, combination, non-parenteral) to expand vaccine coverage in LMICs will also be discussed.

5:30 Welcome Reception in the Exhibit Hall with Poster Viewing (Grand Ballroom)

6:30 Close of Day

TUESDAY, AUGUST 16

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Back Bay B

STABILITY & DEVELOPABILITY

7:55 Chairperson's Remarks
Dhanashri Bagal, Principal Scientist, Discovery Attribute Sciences, Amgen, Inc.

8:00 High-Throughput Transformation of Biologics Formulation Development Workflows
Hanlin Ouyang, Senior Scientist, Merck & Co., Inc.
The HTS group collaborated with formulation and analytical departments to build from scratch platform HT formulation screening workflows that cover study design, sample analytical plan, and data visualization. This ongoing workflow development synergizes cutting-edge robotic liquid handling system, HT study setups, HT analytical instruments, DOE JMP design, and data analysis to deliver richer information for a faster and robust decision-making with less material requirement.

8:30 Assessing New and Sensitive Mass Spectrometry-Based Techniques to Rapidly Characterize Protein Therapeutics
Dhanashri Bagal, Principal Scientist, Discovery Attribute Sciences, Amgen, Inc.
Low material consumptive and rapid analytics performed on early-stage engineering panels of protein therapeutic candidates can inform on protein stability and developability. Herein we examine sensitive and robust mass spectrometry-based methods using online capillary SEC and HIC – LC/MS to inform on protein attributes such as aggregation and mispairing. We will also discuss a sensitive N-terminal chemical tagging and rpLC-MS/MS-based approach to accurately identify low-level proteolytic clips.

9:00 Challenges and Opportunities in Cell Therapy Drug Product Development
Bharathi Vellalore, PhD, Senior Scientist, Biotherapeutics Drug Product Development, Janssen
- Overview of drug product development from formulation, fill-finish, storage, to delivery
- Formulation and process considerations to improve end-to-end drug product stability
- Integrated drug product design to suit clinical and commercial supply chain needs

9:30 Sponsored Presentation *(Opportunity Available)*

10:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

10:45 Breakout Discussions
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: Stability and Developability of Novel Modalities
Bharathi Vellalore, PhD, Senior Scientist, Biotherapeutics Drug Product Development, Janssen
- Analytical methods for high concentration biologics and cell and gene therapies
- Phase-appropriate methods for process monitoring and control
- Analytical methods for drug products characterization
- Stability and impurities in novel modalities

SURFACTANT AND AGGREGATION

11:30 Identification and Rapid Characterization of Impurities Resulting in PS-80 Degradation by Proteomics and Charge-Reduced Mass Spectrometry
Shannon Rivera, PhD, Senior Scientist, Merck & Co., Inc.
Polysorbates are commonly used excipients in biotherapeutic formulations. These molecules may be degraded by process-related impurities, ultimately resulting in negative impacts on quality, efficacy, safety, and stability of the biopharmaceutical. Here we present work using two mass spectrometry techniques – 1) LCMS proteomics for identification of impurities and 2) RP-HPLC coupled with charge-reduced HRMS for rapid characterization of PS-80 degradation – used to identify sources of and track PS-80 degradation.

12:00 pm Surfactants – Cause or Cure for Aggregates
Björn Boll, Fellow Drug Product Design – Senior Expert for Particle Characterization and Analytics, ten23 health
This presentation will highlight surfactants, e.g. polysorbate or poloxamer, as an important part of drug formulations responsible for stabilizing the active ingredient against interfacial stresses caused by shaking, stirring, or freezing. The stability of the surfactants within the shelf-life of the drug product is critical to ensure the quality of the final drug product since their degradation could lead to aggregation and even compromise the stability of the active ingredient.

12:30 Sponsored Presentation *(Opportunity Available)*

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

1:30 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

CHARACTERIZATION & CONTROL OF IMPURITIES IN BIOLOGICS: HCPs, SURFACTANTS, AND MORE

2:10 Chairperson's Remarks
Kevin Zen, PhD, Executive Director, Chemistry Manufacturing and Controls, AnaptysBio, Inc.

2:15 FEATURED PRESENTATION: Control Strategy of Biotech Product Impurities
Kevin Zen, PhD, Executive Director, Chemistry Manufacturing and Controls, AnaptysBio, Inc.
The impurities in therapeutic protein drug products can originate from raw material, bioprocess, product itself, and during patient dosing. This presentation will highlight potential impurities of therapeutic biotech products during upstream process and downstream purification and share the best strategy to mitigate and control such impurities. Some common queries from health authorities will be exemplified as case studies.

2:45 Impact of Four Inorganic Impurities on the Quality Attributes of a Fc-Fusion Protein
Alessandra Pistacchio, PhD, Biotech Pharmaceutical Development, Drug Product Development, Merck KgaA
Regulatory guidelines limit those impurities considered having a higher potential safety risk. However, one of the problems of these limits is that they account for the safety risk, while alterations of certain Quality Attributes of a biologic may also take place. To understand how certain impurities could affect the physicochemical properties of biotherapeutics, we had examined how Ni2+, Cu2+, Zn2+, Fe3+ could alter an Fc-fusion protein, under different conditions.

3:15 Informing Downstream Control of Polysorbate Degradation Using a High-Throughput, Lipolytic Activity Assay
Alex Dow, PhD, Associate Principal Scientist, Merck & Co., Inc.
Polysorbate, a stabilizer within biologics formulations, is known to degrade via active, low-concentration lipases and esterases. The need for a rapid assay probing this specific HCP mechanism is required to improve the use of proper control strategies. A high-throughput, rapid activity assay using a fluorescent substrate allows for quantitation of lipolytic activity. This enabled an improved approach for the measurement of impurity impact and control.

3:45 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

4:30 Identification of Active Enzymes for Polysorbate Degradation in Biotherapeutics by Activity-Based Protein Profiling
Shawn Li, PhD, Principal Scientist, Analytical Research and Development (AR&D) Mass Spectrometry, Merck & Co., Inc.
Enzymatic activity from residual host cell enzymes such as lipases and esterases plays a major role in polysorbate degradation. Their high activity, often at very low concentration, constitutes a major analytical challenge in the biopharmaceutical industry. In this study, we evaluated and optimized the activity-based protein profiling (ABPP) approach to identify active enzymes responsible for polysorbate degradation, which enables more meaningful polysorbate degradation investigations for biotherapeutic development.

5:00 Best Practices for Internal Development of HCP Monitoring Kits
Olivier Ducoudret, Senior Development Specialist, Quality Control, Macrogenics
Host cell proteins (HCPs) are a class of process-related impurities during the manufacturing of biologics. HCPs must be removed in biologics manufacturing to ensure final product purity, manufacturing robustness, and safety. HCP analysis is often performed using an enzyme-linked-immunosorbent-assay (ELISA) due to method sensitivity and ease of use. When using an ELISA kit for HCP monitoring best practices involve determining kit sensitivity (quantification and coverage), product dilutional linearity, and robustness.

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

WEDNESDAY, AUGUST 17

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Back Bay B

7:55 Chairperson's Opening Remarks
Sanket Patke, PhD, Associate Director, Sanofi

8:00 KEYNOTE PRESENTATION: Developing Drug/Device Combination Products – Things to Consider
Hanns-Christian Mahler, PhD, CEO, ten23 health
Biologics are applied parenterally. Product for SC administration are often leveraging a device to ensure administration convenience and compliance. However, drug/device combination products present many technical challenges, related to stability, usability, and manufacturing of such products. This presentation aims to discuss how to integrate design thinking for a drug/device combination product into early stage molecule selection, product characterization and development.

9:00 A Systematic Approach to Evaluating Closed System Drug-Transfer Devices During Drug Product Development
Sanket Patke, PhD, Associate Director, Sanofi

PREDICTING PROTEIN AGGREGATION

9:30 Machine Learning Prediction of Antibody Aggregation and Viscosity for High-Concentration Formulation
Pin-Kuang Lai, PhD, Assistant Professor, Department of Chemical Engineering and Materials Science, Stevens Institute of Technology
Predictive models that evaluate aggregation and viscosity behaviors at high concentrations during early-stage design can facilitate drug development. This talk describes a rational feature selection framework to develop accurate models for high concentration aggregation and viscosity at 150 mg/mL for 20 preclinical and preclinical antibodies with few features. In addition, this talk will present an efficient convolutional neural network model that predicts high concentration viscosity.

10:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

FORMULATION AND DELIVERY OF NOVEL MODALITIES

10:40 Drug Product Development of an Allogeneic NK-Cell Therapy
Randall Mauldin, PhD, Associate Director, Cell Therapy Drug Product, Sanofi
Allogeneic cell therapies present a unique challenge for drug product process development by combining aspects of autologous cell therapy and conventional biologic products. In addition, production scale may increase significantly as the process evolves towards commercial manufacturing. Technical challenges in cell process development, considerations for tech transfer, and the analytical assessment of stability-indicating attributes to guide development will be discussed.

11:10 Effects of 6-His Tag Inclusion on Assembly and Stability of a Universal Flu Vaccine Nanoparticle
Rajoshi Chaudhuri, PhD, Senior Scientist, Vaccine Production Lab, National Institutes of Health/National Institute of Allergy and Infectious Diseases
Mosaic Flu nanoparticles were designed to present multiple arrays of antigens to induce a stronger immune response. Preliminary formulation development utilized proteins containing a histidine tag. The nanoparticle developed for clinical trials consisted of antigens without a histidine tag. Differences in stability profile were observed between the His-tagged and non-His-tagged nanoparticle mainly after freeze-thaw stress. This talk discusses the approach taken to optimize the formulation to mitigate the freeze-thaw instability.

11:40 FEATURED PRESENTATION: Optimizing Drug Product Presentation and Manufacturing Processes for Gene Therapy Products
Sandeep Yadav, PhD, Senior Director, Drug Product Formulation & Fill/Finish, Sangamo Therapeutics
This talk focuses on developing and optimizing DP formulation/presentations and manufacturing strategies to support patient safety, healthcare provider convenience, and reducing cost of goods to enable patient accessibility.

12:40 pm Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

FORMULATION AND DELIVERY OF NOVEL MODALITIES, CONT.

1:25 Chairperson's Remarks
Amey Bandekar, PhD, Associate Director, Drug Product Development, Sanofi

1:30 Assessment of Structural Integrity of Biotherapeutics through Simulated Subcutaneous Environment Using SCISSOR
Deep Bhattacharya, PhD, Senior Scientist, Formulation & Process Development Biotherapeutics, Pfizer Inc.
- Structural assessment of simulated *in vitro* conditions of biologics using the SCISSOR
- Understanding of destabilizing mechanisms of biologics in the subcutaneous environment
- Deeper dive into peptide hotspots to identify potential sites for destabilization and reiterative formulation development

2:00 Novel Modified Albumin Blocks Glycocalyx Inflammatory Cascade
William Norberg, CEO & Pediatric Cardiac Intensivist, SysteMedical, Inc.
A New concept of the Inflammatory Cascade of 2022:
The body organ, the endothelial glycocalyx lining, the entire vascular interior, the entire inflammatory reaction damage begins. The complex coagulation and thrombotic responses, the entities that control fever, blood pressure, fluid volumes, and cellular inflammatory responses are activated.
A novel modified albumin solution initially created to prevent fluid losses prevented the hallmarks of sepsis.
Sepsis was first stopped here.

2:30 Formulation Considerations and Challenges for Non-Viral Gene Delivery
Amey Bandekar, PhD, Associate Director, Drug Product Development, Sanofi
In LNP Design, choice of each component determines the *in vivo* behavior and efficacy of nucleic acid encapsulation and delivery. These parameters can be modulated to maneuver the biodistribution of LNPs depending on the organ of interest and type of nucleic acid. Hence, choice of the correct lipid systems based on the nucleic acid, manufacturing process, and intracellular trafficking tools are important to enable improved screening prior to *in vivo* studies.

3:00 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

ROOM LOCATION: Constitution A&B

LEADING TO TOMORROW'S ADVANCES

3:50 Plenary Introduction
Nathalie Clément, PhD, CEO, Unicorn Consultations, LLC

4:00 New Therapeutic Modalities and Moore's Law in Biomanufacturing
Hari Pujar, PhD, Operating Partner, Flagship Pioneering; COO, Tessera Therapeutics
As we have embarked on newer, more complex modalities, biomanufacturing has appeared to stumble. The talk will chronicle the advancements in biomanufacturing of different therapeutic modalities, drawing parallels to semiconductor chip manufacturing, and establishing the rightful and bright future of biomanufacturing.

4:30 Cell and Gene Therapy (R)evolution
Mercedes Segura Gally, PhD, Vice President, Process Development, ElevateBio
The concept of gene therapy arose nearly half a century ago. Turning that concept into a therapeutic reality required years of scientific discovery, technological advances, and pioneering efforts, culminating in several regulatory approvals. These success stories paved the road for a second wave of advanced therapies. Compared with traditional biologics, cell and gene therapy products pose unique challenges. This presentation aims to summarize the progress made in recent years.

5:00 Networking Reception in the Exhibit Hall with Poster Viewing (Grand Ballroom)

6:00 Close of Day

THURSDAY, AUGUST 18

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Back Bay B

HIGH-CONCENTRATION PROTEIN FORMULATIONS

7:55 Chairperson's Remarks
Shantanu V. Sule, PhD, Principal Scientist, Amgen, Inc.

8:00 Strategies in Development and Manufacturing of Low Viscosity, Ultra-High-Concentration Formulation for IgG1 Antibody
Vaibhav Deokar, Principal Scientist, Formulation Development, Lupin Ltd. (Biotechnology Division)
Present research provides comparative evaluation of scalable manufacturing strategies to develop low viscosity (<20cps), ultra-high-concentration (>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 comparative evaluation of manufacturing strategies and their impact on 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).

8:30 Caffeine as a Viscosity Reducer for Highly-Concentrated Monoclonal Antibody Solutions
Yuhong Zeng, PhD, Director, Formulation, Comera Life Sciences
Monoclonal antibody solutions usually exhibit high viscosity at elevated concentrations, which prevents manufacturing and injecting of products at the small volumes needed for subcutaneous administration. Here we show that caffeine effectively reduces the viscosity of infliximab and ipilimumab formulated at high concentrations and small volumes required for subcutaneous injection. Further studies show that caffeine has no impact on accelerated stability or *in vitro* bioactivities of the two model proteins.

9:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

9:15 Poster Award Presented in the Exhibit Hall

9:30 Developing High Dose Biologics – Scaling the Cliffs and Beyond
Shantanu V. Sule, PhD, Principal Scientist, Amgen, Inc.
Large-molecule parenteral drugs often require high concentration formulations, large volume delivery systems, or a combination of both. This poses major challenges in quality, manufacturability, delivery, and patient convenience. Here we present comprehensive approaches to address these challenges during developability assessment, formulation development for optimal viscosity and stability, integration with delivery device, process design, and tech transfer. We also discuss novel approaches in predicting them in early development.

10:00 Novel Excipients for Enhancing Physical and Chemical Stability of Spray Dried Proteins
Purnendu Kumar Nayak, Scientist, Pharmaceutical Development, Genentech, Inc.

10:30 Redefining High-Concentration Formulations: Beyond the Aqueous Limit
Deborah Bitterfield, PhD, CEO & Founder, Lindy Biosciences, Inc.
A major challenge in formulating high-dose biologics for subcutaneous injection is overcoming the viscosity and stability limitations of high-concentration (>100 mg/mL) solutions. Suspensions, however, have reduced viscosity, enabling concentrations of 400 mg/mL and higher. Microglassification™ is a dehydration technology that results in stable, dense, spherical particles of amorphous, solid protein. We will present current studies on the stability and injectability of suspensions using these particles.

11:00 Breakout Discussions
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: Advantages and Disadvantages of Machine Learning Approach for Antibody Stability Prediction
Pin-Kuang Lai, PhD, Assistant Professor, Department of Chemical Engineering and Materials Science, Stevens Institute of Technology
- Machine learning algorithms for small data
- Feature creation and selection for antibody
- Apply the machine learning protocol to a new platform

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

12:30 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

HIGH-CONCENTRATION PROTEIN FORMULATIONS, CONT.

1:05 Chairperson's Remarks
Shantanu V. Sule, PhD, Principal Scientist, Amgen, Inc.

1:10 Leveraging Automation to Enable High-Concentration Formulation Development
Peter Soler, PhD, Senior Research Investigator, Bristol Myers Squibb Co.
Biologics drug development has experienced rapid growth in recent years. To meet the need biologics formulation development has quickly acquired a set of automation tools and analytical techniques to provide robust drug products for patients. The push for more effective therapeutics and better patient-care options has demanded new types of studies. This has motivated adaptation of our tools to meet the increases in process complexity to benefit of patients globally.

1:40 Succinate Buffer in Biologics Products: Real-World Formulation Considerations, Processing Risks, and Mitigation Strategies
Anvay Ukidve, PhD, Scientist, Formulation and Process Development, Sanofi
Succinate acid/succinate system has an excellent buffering capacity at acidic pH values (4.5-6.0). However, its use in formulating drug products is largely limited due to risk of its components crystallizing and the consequent pH shifts. Physicochemical behavior of succinate system was characterized under pharmaceutically representative conditions. mAbs formulated in de-risked succinate buffer maintained a good stability profile during typical pharmaceutical processing and upon storage, bolstering their wider use in drug products.

2:10 Panel Discussion: Drug-Device Combination Products and High-Concentration Protein Formulations
Moderator: Shantanu V. Sule, PhD, Principal Scientist, Amgen, Inc.
What are the obstacles when integrating protein formulations with parenteral administration devices?
Panelists:
Sandeep Yadav, PhD, Senior Director, Drug Product Formulation & Fill/Finish, Sangamo Therapeutics
Yuhong Zeng, PhD, Director, Formulation, Comera Life Sciences
Peter Soler, PhD, Senior Research Investigator, Bristol Myers Squibb Co.
Vaibhav Deokar, Principal Scientist, Formulation Development, Lupin Ltd. (Biotechnology Division)

2:40 Refreshment Break in the Exhibit Hall & Last Chance for Poster Viewing (Grand Ballroom)

4:40 Close of Summit

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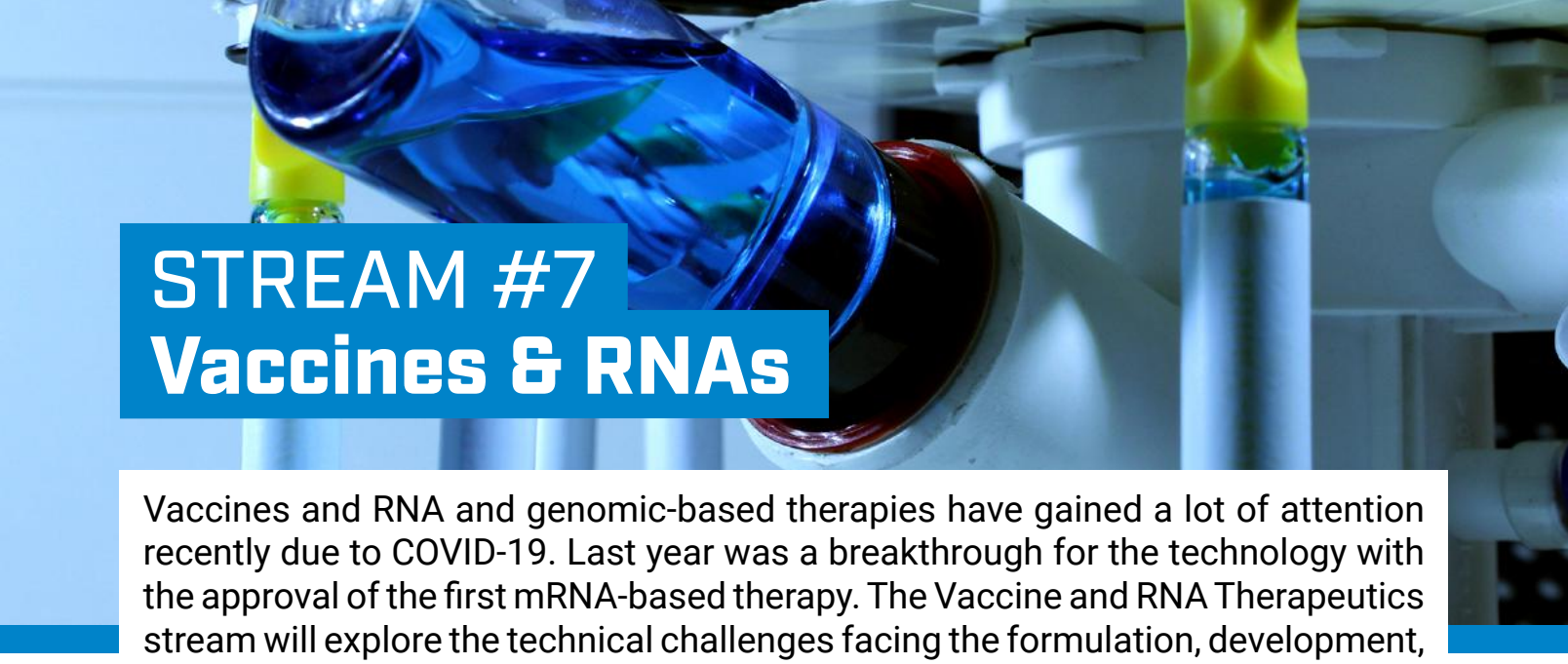


STREAM #7
Vaccines & RNAs

Vaccines and RNA and genomic-based therapies have gained a lot of attention recently due to COVID-19. Last year was a breakthrough for the technology with the approval of the first mRNA-based therapy. The Vaccine and RNA Therapeutics stream will explore the technical challenges facing the formulation, development, manufacturing, and supply of next-generation vaccines, mRNA vaccines, mRNA gene therapies, RNAi, gene therapies, and cell 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 15-16

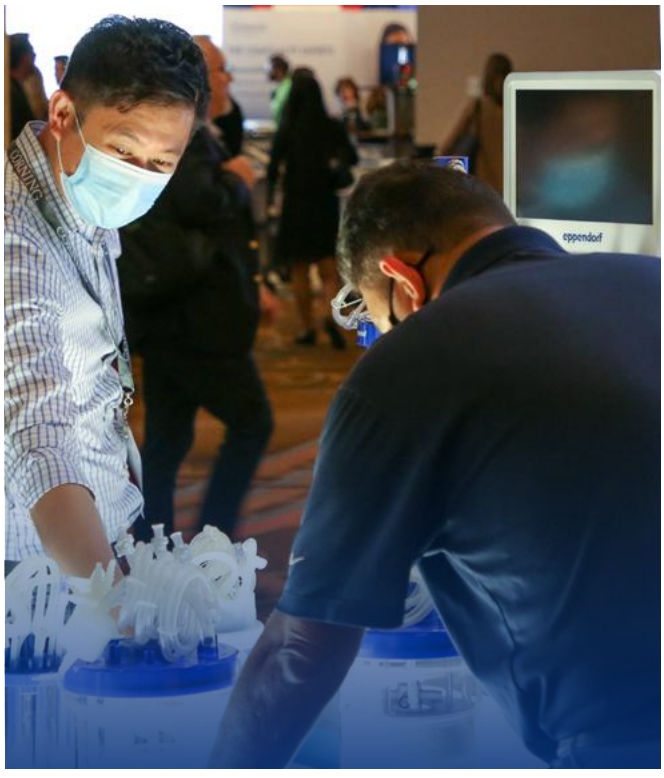
Vaccine Development and Manufacturing

View Program »

AUGUST 17-18

RNA and Genomic-Based Therapeutics

View Program »



MONDAY, AUGUST 15

9:00 am Main Conference Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Gardener

UPDATES ON COVID-19 VACCINES AND BOOSTERS

9:55 Chairperson's Opening Remarks
Alexander Rumyantsev, PhD, Therapeutic Area Head, Infectious Diseases, Vaxxinity

10:00 The Progress with UB-612, a Phase III Stage Heterologous COVID-19 Vaccine Booster
Alexander Rumyantsev, PhD, Therapeutic Area Head, Infectious Diseases, Vaxxinity
Vaxxinity develops a heterologous COVID-19 vaccine, UB-612, aimed to boost the immunity stimulated after the primary vaccination. To date, clinical evidence supports UB-612 favorable safety profile and high immunogenicity across multiple SARS-CoV2 variants, including Omicron. Currently, UB-612 is tested in a Phase 3 pivotal study designed to compare head-to-head its safety and immunogenicity to several authorized COVID-19 vaccines based on mRNA, virus-vectored, and inactivated manufacturing platforms, administered as 3rd-dose boosters.

10:30 Lessons Learned from Rapid Process Development of a VSV-SARS-CoV-2 Vaccine Candidate
Laura Kuczynski, Senior Scientist, Vaccine Process Development, Merck & Co., Inc.
This discussion will focus on how Merck was able to rapidly advance an investigational SARS-CoV-2 vaccine based on the vesicular stomatitis virus platform used for the Ebola vaccine, ERVEBO. In this discussion, we detail the development of the purification process for this vaccine candidate. We will highlight areas where the platform was successfully adopted as well as additional measures that were needed for the SARS-CoV-2 vaccine candidate.

11:00 High-Throughput Viral Infection Imaging Methods for Enhancing the Speed of Vaccine Development
Carl Hofmann, Senior Scientist, Analytical Research and Development, Merck & Co., Inc.
High-throughput (HTP) microscopy's ability to measure cellular phenomenon in multiple cell culture models has advanced the utility of viral imaging with the advent of automation-friendly multi-modal imagers. The ability to automate cellular staining, and subsequent imaging, paired with the use of integrated liquid handling, has led to the development of faster HTP approaches that can supplement slower traditional virologic methods during clinical development.

11:30 Evolution of Vaccine Processing to Accelerate Innovation MilliporeSigma
Oliver Prince, PhD, Senior Consultant Bioprocessing, Traditional Modalities, Americas, MilliporeSigma
The COVID pandemic and Industry 4.0 acceleration have highlighted the need for concrete solutions for agile vaccine manufacturing. For this talk, we will discuss recent trends, solutions and innovations that allow vaccine processing acceleration, including closed processing, modular facilities, and vaccine platform manufacturing.

12:00 pm Enjoy Lunch on Your Own

ANALYTICAL STRATEGIES AND TOOLS

12:55 Structure and Compositional Analysis of Aluminum Oxyhydroxide Adsorbed Pertussis Vaccine
Marina Kirkitadze, PhD, Head Bioprocess Support & PAT Platform, Analytical Sciences, Sanofi Pasteur
Characterization of an acellular pertussis vaccine (Tdap) containing genetically modified pertussis toxin (gdPT) and TLR agonist adsorbed to AlOOH adjuvant will be discussed in this presentation. The results obtained using several analytical tools including nanoDSF, FTIR, LD, as well as in-line IR and FBRM probes report conformation of novel gdPT, as well as the composition of AlOOH adjuvant formulations adsorbed to pertussis vaccine.

1:25 Raman Deployment for Vaccines
Christopher Mahoney, PhD, Scientist, Advanced Process Control, Johnson & Johnson Pharmaceutical R&D
Raman deployment in vaccine manufacturing is viewed as an advanced technology, aimed to increase overall efficiency, quality, and reliability. With Raman-based regression models, efficiency increases with inline process characterization of metabolites, cell density, and titer in real time which can be viewed from any laptop/desktop assuming the proper network capabilities. Raman models increase quality by enabling proactive issue resolution and reducing overall risk during new product introduction and tech transfer.

1:55 Upstream Process Intensification for Virus Vaccine Manufacturing Case Study REPLIGEN
Shelly Parra, Senior Director of Technology Positioning and Product Solutions, Repligen
Many viral manufacturing processes for animal health have been transferred from the time consuming and higher risk adherent culture systems to more scalable suspension cell cultures. Growing demand and the need for efficient and economic processes drive the continuous search for innovations. This talk describes studies conducted at MSD Animal Health to evaluate the XCell ATF Technology for intensification of virus propagation on suspension cells.

2:10 Sponsored Presentation *(Opportunity Available)*

2:25 Networking Refreshment Break (Grand Ballroom Foyer)

ROOM LOCATION: Back Bay C

PATH TO CONTINUOUS BIOMANUFACTURING

2:40 Intensification Strategies: The Path to Continuous Processing
Stefan R. Schmidt, MBA, PhD, COO & Head, Operations, BioAtrium AG
Continuous processing is the holy grail for many industries and became popular for bioprocessing in the last decade, too. Intensification is a prerequisite to enable a step-wise transformation toward that goal. This presentation gives a comprehensive overview of strategies where and how to implement process intensification, quantifies the benefits like plant occupancy time, and optimizing capacity based on successful examples and case studies.

3:10 Process Intensification Measuring the Performance and Sustainability
Andrew Sinclair, President & Founder, BioPharm Services Ltd., United Kingdom
Understanding the impact of process intensification options in terms of sustainability and business efficiency. The latest process models evaluate facility efficiency (doses per unit volume of cleanroom), PMI, and total energy efficiency. Pre-release versions were used by Process Intensification team in

NIIMBL to support sustainability assessments. In this talk, comparisons are made between standard fed-batch processes and intensified process options that include perfusion and continuous downstream operations.

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

ROOM LOCATION: Constitution A&B

PLENARY KEYNOTE: SOLVING TODAY'S CHALLENGES

4:20 **Plenary Introduction**
James Warren, PhD, Vice President, Pharmaceutical Development, Ultragenyx Pharmaceutical

4:30 **Lessons Learned from the Pandemic: mRNA-LNP Vaccine Development**
Nicholas Warne, PhD, Vice President, Pharmaceutical Research and Development, BioTherapeutics Pharmaceutical Sciences, Pfizer Inc.
The speed and scale of industry response to the COVID pandemic was unprecedented, ultimately leading to the availability of several vaccines in under a year. This presentation will discuss the approach taken by Pfizer, with their partner BioNTech, in the development, manufacture, and distribution of the vaccine drug product while reflecting on lessons that may, or may not, be applicable to future product development.

5:00 **Advances in Vaccine Formulation and Stability**
David B. Volkin, PhD, Distinguished Professor, Pharmaceutical Chemistry, University of Kansas, Lawrence
This presentation will provide an overview of analytical characterization and formulation development considerations for new vaccine candidates targeted for use in low- and middle-income countries (LMICs). Illustrative case studies with vaccine candidates (e.g., live-virus, adjuvanted recombinant protein) will highlight implementing state-of-the-art stability-indicating assays to enable development of stable formulations. Challenges with developing lower-cost formulations (e.g., multi-dose, combination, non-parenteral) to expand vaccine coverage in LMICs will also be discussed.

5:30 **Welcome Reception in the Exhibit Hall with Poster Viewing (Grand Ballroom)**

6:30 **Close of Day**

TUESDAY, AUGUST 16

7:30 am **Registration and Morning Coffee (Grand Ballroom Foyer)**

ROOM LOCATION: Gardener

VACCINE BIOPROCESSING AND MANUFACTURING

7:55 **Chairperson's Remarks**
Gerald Striedner, PhD, University Professor, Biotechnology, University of Natural Resources and Life Sciences Vienna (BOKU), Austria

8:00 **A Scalable, Integrated Downstream Process for the Production of Recombinant Measles Virus-Vectored Vaccines**
Alois Jungbauer, PhD, Professor & Head, Biotechnology, Institute of Bioprocess Science and Engineering, University of Natural Resources and Life Sciences (BOKU)
Purification of measles vector vaccines based on restricted access chromatography and ultrafiltration has all relevant elements for a platform process. MV vectors remain in the process stream and impurities bound to the stationary phase or depleted based on their smaller size during ultrafiltration. The purification strategy can be adapted to other MV vectors because we avoid a bind-elute step, allowing fast process development for vaccines and adaption for new applications.

8:30 **ambr250 HT System: A Key Bioreactor Process Development Tool for Industrial Vaccine and Oncolytic Virus Production**
Marena Trauger, Scientist, Vaccine Process Development, Merck & Co., Inc.
The ambr250 HT is an important tool for bioreactor process development of vaccines and oncolytic viruses, allowing for expedited experimentation due to ability to automate, reduced resourcing and staffing needs, and smaller footprint. Through this poster, a series of case studies from live virus/microcarriers, fermentation, or suspension mammalian cells are examined where the ambr250 HT was applied in an industrial setting to expand capabilities, increase efficiency, and shorten timelines.

9:00 **Production of High-Quality Plasmid DNA: A Key Ingredient in Vaccine Production Processes**
Gerald Striedner, PhD, University Professor, Biotechnology, University of Natural Resources and Life Sciences Vienna (BOKU), Austria
The provision of high-quality plasmid DNA is a requisite for the production of different vaccines like mRNA vaccines or protein-based vaccines. For this purpose, a generic approach for the fermentation process and the DSP steps has been developed which allows the production of the corresponding plasmid variants in high quality in a very flexible manner and in a short time.

10:00 **Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)**

10:45 **Breakout Discussions**
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: Production of Vaccines in a Closed System
Alois Jungbauer, PhD, Professor & Head, Biotechnology, Institute of Bioprocess Science and Engineering, University of Natural Resources and Life Sciences (BOKU)
- Production technologies for large viruses and vaccines
- Sterile interconnection of unit operations
- Testing/validation of sterility of the production system

11:30 FEATURED PRESENTATION: Disruptive Technologies for Formulation, Manufacturing, and Delivery: Enabling Advancements for Biologics and Vaccines Products into the Future

Jeff Blue, Executive Director, Vaccine Drug Product Development, Merck & Co., Inc.

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

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

1:30 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

VACCINE FORMULATION AND DELIVERY

2:10 Chairperson's Remarks

Lee Christopher Smith, Principal Consultant, GreyRigge Associates Ltd.

2:15 KEYNOTE PRESENTATION: Microneedle Development and Manufacturing for the Vaccines

Philippe-Alexandre Gilbert, PhD, Senior Program Officer, Vaccine Development and Surveillance, Bill and Melinda Gates Foundation

Microneedle array patches (MAPs) are being developed as a new delivery system for vaccines. MAPs are offering many advantages over traditional vaccine delivery technologies and could be an asset for LMIC immunization campaigns. Mass production of MAP will have to be demonstrated for vaccines at an acceptable cost and output while meeting all the regulatory authority's safety standards. MAP industrialization will be key for a rapid adoption of this technology.

3:15 QbD Analytics for Vaccines

Lee Christopher Smith, Principal Consultant, GreyRigge Associates Ltd.

Quality by Design (QbD) guidance in vaccine development continues to be limited and fragmented in its application. Furthermore, with the imminent publication of ICH Q14, QbD approaches will extend to analytical methods. The approaches to be taken to generate suitable documentation, risk assessments and design of experiments to provide both process and analytical control strategies will be discussed.

3:45 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

4:30 Multidetector Field Flow Fractionation (MD-FFF) for Comprehensive Quality Assessment of mRNA-LNP Vaccines

Sven Even F. Borgos, PhD, Senior Research Scientist, Biotechnology & Nanomedicine, SINTEF

mRNA-based vaccines show a revolutionary prophylactic and therapeutic potential. However, both the mRNA itself and the nanocarrier used for its delivery place unique demands on the analytical methods used to ensure vaccine quality. We will show that MD-FFF is a very powerful, high-data-content methodology that nicely complements and extends beyond batch-phase techniques for physical, chemical, and stability analysis of these next-generation vaccines in the industry setting.

5:00 PANEL DISCUSSION: Lessons Learned from COVID-19 and Preparing for the Next Pandemic

Moderator: Lee Christopher Smith, Principal Consultant, GreyRigge Associates Ltd.

Panelists:

Philippe-Alexandre Gilbert, PhD, Senior Program Officer, Vaccine Development and Surveillance, Bill and Melinda Gates Foundation

Jeff Blue, Executive Director, Vaccine Drug Product Development, Merck & Co., Inc.

Gerald Striedner, PhD, University Professor, Biotechnology, University of Natural Resources and Life Sciences Vienna (BOKU), Austria

Sven Even F. Borgos, PhD, Senior Research Scientist, Biotechnology & Nanomedicine, SINTEF

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

5:30 Close of Vaccine Development and Manufacturing Conference

WEDNESDAY, AUGUST 17

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Gardener

7:55 Chairperson's Opening Remarks
Christina Schier, PhD, Senior Scientist, Merck & Co., Inc.

8:00 Dysregulated RNA Processing – A New Senotherapeutic Target
Lorna Harries, Diabetes & Vascular Medicine, Peninsula Medical School UK
Interventions targeting aging hallmarks treat the causes, not the consequences of age-related disease. Dysregulated RNA processing is emerging as a new hallmark of aging. Drugging this new hallmark in senescent human primary cells using small molecules or targeted genetic interventions is capable of rescuing multiple features of cellular senescence. Drugs that target the regulation of splicing factors may therefore represent promising novel anti-degenerative therapies in the future.

8:30 KEYNOTE PRESENTATION: Living in the World of RNA Therapeutics Enabled by Lipid Nanoparticles
Mano Manoharan, PhD, Distinguished Scientist & Senior Vice President, Innovation Chemistry, Alnylam Pharmaceuticals
- Chemistry of Lipid Nanoparticles
- Ionizable Lipids *vs* Cationic Lipids
- Mechanism of endosomolytic release by LNPs
- Historical approval of ONPATTRO
- LNP-mediated delivery, starting from siRNAs to mRNAs

9:30 Strategies and Key Considerations for the Purification of Plasmid DNA and Production of mRNA
Laurens Vergauwen, Process Development Scientist, Technical & Scientific Solutions EMEA, MilliporeSigma
Production of pDNA suffers from the low productivity of microbial fermentation and the purification process remains challenging. This presentation describes an end-to-end platform where each of the steps will be explored, along with strategies to optimize and streamline the purification workflow. In addition, considerations for an mRNA template process are discussed.

10:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

ANALYTICAL GUIDANCE AND TOOLS FOR mRNA THERAPIES

10:40 Analytical Procedures to Support mRNA Vaccine Quality: Draft USP Guidelines
Diane McCarthy, PhD, Senior Director, Science & Standards, Global Biologics, US Pharmacopeia
The development and approval of mRNA-based vaccines for COVID-19 revealed the potential of this platform for both preventative and therapeutic purposes. A standard set of analytical methods to assess mRNA quality would support product developers, manufacturers, regulators, and control laboratories worldwide. In collaboration with vaccine experts, USP has developed draft guidelines with analytical procedures for quality assessment of mRNA products. Results of this work and stakeholder input will be shared.

11:10 High-Throughput Definition and Characterization of a Cell-Based Assays for mRNA-LNP Vaccine Potency
Christina Schier, PhD, Senior 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.

11:40 Panel Discussion: Advances in Characterization Approaches and Analytical Tools for mRNA-Based Therapies
Moderator: Christina Schier, PhD, Senior Scientist, Merck & Co., Inc.
Panelists:
Mano Manoharan, PhD, Distinguished Scientist & Senior Vice President, Innovation Chemistry, Alnylam Pharmaceuticals
Amy Glekas, PhD, Global Head, Innovation & Product Characterization, MilliporeSigma

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 (Grand Ballroom)

EMERGING APPLICATIONS AND INDICATIONS

1:30 Reducing Therapeutic mRNA Dose 100-Fold by Engineering Translation Enhancers
Wendy V. Gilbert, PhD, Associate Professor, Molecular Biophysics & Biochemistry, Yale University
High-throughput screening technology developed in my lab has uncovered elements encoded in the 5' UTRs of mRNAs that can modulate the translation output of mRNAs across a 1,000-fold range (Niederer et al. Cell Systems 2022). We are leveraging this technology to design new translation-enhancing features that can be engineered into chemically modified, therapeutic mRNAs to control protein production.

2:00 Silencing mRNA by RNA Interference: Skin Delivery of RNAi-Based Therapeutics for the Treatment of Autoimmune Skin Diseases
Qi Tang, PhD, Postdoctoral Associate, Department of Dermatology, University of Massachusetts Chan Medical School
siRNAs are a new class of therapeutic moieties that harness endogenous RNAi process to enable specific and sustained silencing of mRNAs, thus reducing the protein synthesis of a disease target. We chemically engineer siRNAs for various therapeutic applications, and here we present the preclinical development of fully chemically-modified siRNAs targeting IFN-gamma pathway to establish a path toward the treatment of autoimmune skin diseases.

2:30 A Multi-Scale Digital Twin to Predict and Control the Real-Time Performance of a Bioreactor Announced
Kurt Svihla, Senior Application Engineer, Ansys
In the present study, three-dimensional steady-state CFD simulations for varying sparge rates and impeller agitation rates are used to develop a reduced-order model of bioreactor performance. This model is coupled to

a zero-dimension transient model representing various cellular metabolic processes. A coupled system-level control model is then developed using Ansys TwinBuilder and exported as a digital twin.

3:00 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

ROOM LOCATION: Constitution A&B

PLENARY KEYNOTE: LEADING TO TOMORROW'S ADVANCES

3:50 Plenary Introduction
Nathalie Clément, PhD, CEO, Unicorn Consultations, LLC

4:00 New Therapeutic Modalities and Moore's Law in Biomanufacturing
Hari Pujar, PhD, Operating Partner, Flagship Pioneering; COO, Tessera Therapeutics
As we have embarked on newer, more complex modalities, biomanufacturing has appeared to stumble. The talk will chronicle the advancements in biomanufacturing of different therapeutic modalities, drawing parallels to semiconductor chip manufacturing, and establishing the rightful and bright future of biomanufacturing.

4:30 Cell and Gene Therapy (R)evolution
Mercedes Segura Gally, PhD, Vice President, Process Development, ElevateBio
The concept of gene therapy arose nearly half a century ago. Turning that concept into a therapeutic reality required years of scientific discovery, technological advances, and pioneering efforts, culminating in several regulatory approvals. These success stories paved the road for a second wave of advanced therapies. Compared with traditional biologics, cell and gene therapy products pose unique challenges. This presentation aims to summarize the progress made in recent years.

5:00 Networking Reception in the Exhibit Hall with Poster Viewing (Grand Ballroom)

6:00 Close of Day

THURSDAY, AUGUST 18

7:30 am Registration and Morning Coffee (Grand Ballroom Foyer)

ROOM LOCATION: Constitution B

OPTIMIZING THE PROCESS FOR CELL & GENOMIC THERAPIES

7:55 Chairperson's Remarks
Michael Mercaldi, PhD, Senior Director, Downstream Process Development, Oxford Biomedica Solutions

8:00 A Robust and Scalable Platform Process for GMP Manufacturing of Lentiviral Vectors
Bojiao Yin, PhD, Director, Vector Process Development & Manufacturing, ElevateBio
We describe here a well-established platform process for LV production based on transient transfection of serum-free cells grown in suspension. Both upstream and downstream processes are highly optimized to achieve optimal vector yields and significant decrease in the impurities (host cell protein/DNA, plasmid DNA). The compatibility of this platform process has been evaluated with multiple CAR/TCR genes while the robustness is demonstrated in reproducible runs at pilot scale.

8:30 Manufacturing Lentiviral Vectors for *in vivo* CAR T Cell Therapy
Sarah Gould, PhD, Associate Director, Manufacturing Science & Technology, Umoja Biopharma
Umoja aims to transform cancer care by creating an off-the-shelf, direct injection lentiviral vector (LVV) drug product for *in vivo* CAR T cell generation and expansion. We will present our approach to reproducible and scalable manufacturing of high-quality LVV, with special focus on expediting process development with risk-based quality documentation and impurity clearance at key process unit operations to meet ambitious final specifications.

9:00 Coffee Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

9:15 Poster Award Presented in the Exhibit Hall c-LEcta

9:30 Scaling-Up of a Suspension Packaging Cell Line for Lentiviral Vector Production: Upstream and Downstream Strategies
Aziza Manceur, PhD, Research Officer, National Research Council Canada
To streamline lentiviral vector (LV) manufacturing, we propose to use packaging cell lines. The cells were designed using molecular switches to control the production of LV cytotoxic proteins. They can be used to generate LV through a one-plasmid transfection corresponding to the gene of interest, or to generate stable producer cells. We will present a manufacturing process that circumvents the labile nature of the vectors and results in high yields.

10:00 Overcoming the Bottlenecks in the Manufacturing of Viral Vector-Based Therapies
Saurabh Gautam, PhD, Principal Scientist and Lab Head, Bioprocess Development, Viral Vectors, and Vaccines, ViraTherapeutics / Boehringer Ingelheim
A major gap with viral vectors is in our knowledge of the biology and morphology of the therapeutic. The work presented will focus on our efforts in development of novel chromatographic purification techniques complimented with extensive characterization of our virus using a suite of analytics.

10:30 Sponsored Presentation *(Opportunity Available)*

11:00 Breakout Discussions
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: DSP Strategies for Gene Therapies
Meisam Bakhshayeshi, PhD, Senior Director and Head, Process Development, Intergalactic Therapeutics

12:00 pm Luncheon Presentation: Strategies to Propel Your Viral Vector Therapy from Lab to Clinic

Christine Ricci, Senior Scientist, Upstream Process Development, Viral Gene Therapy, Fujifilm Diosynth Biotechnologies

There are a number of challenges drug developers face on the journey to the clinic, including high costs and competitive timelines. FDB has solutions that help our partners navigate the challenges of development and material supply for clinical trials as well as propel their life changing medicines to commercialization. Our solutions include a flexible AAV platform and a wealth of regulatory experience taking medicines to the market for our partners.

12:30 Refreshment Break in the Exhibit Hall with Poster Viewing (Grand Ballroom)

ROOM LOCATION: Gardener

NEXT-GENERATION NANOPARTICLES: PRECLINICAL AND CLINICAL CASE STUDIES

1:10 Next-Generation Lipid Nanoparticles – Paving the Road to Patients

Sven Even F. Borgos, PhD, Senior Research Scientist, Biotechnology & Nanomedicine, SINTEF

LNPs have proven their value for delivery of RNA medicines. We argue that some critical needs should be addressed to facilitate and accelerate the next-generation of LNPs for nucleic acid delivery, for the benefit of patients. These needs include i) automated platforms for combinatorial synthesis linked to high-throughput *in vitro* and *in vivo* screening systems; ii) mRNA and/or LNP reference materials, and iii) robust test methods and protocols.

1:40 Lipid Nanoparticles for Overcoming Biological Barriers to mRNA Delivery

Michael J. Mitchell, PhD, Skirkanich Assistant Professor, Innovation Bioengineering, University of Pennsylvania

In this talk, I will discuss our efforts towards the development of lipid and polymer-based nanoparticles that enable the delivery of nucleic acid therapeutics to target cells and tissues *in vivo*. Furthermore, I will describe new therapeutic strategies utilizing these nanoparticles to (i) reprogram immune cells for cancer immunotherapy applications, (ii) in utero mRNA delivery for treating disease before birth.

2:10 Double-Encapsulated mRNA Vaccine

Trevor P. Castor, PhD, President & CEO, Aphios Corp.

We are developing a single-shot, room temperature stable mRNA vaccine by double nanoencapsulating the mRNA construct in phospholipid nanosomes and biodegradable polymer nanospheres. We are using continuous flow, solvent-free processes that minimize loss of potency, preserve antigenicity of the nanoencapsulated mRNA, and eliminate residual organic solvents. The impact of this development would be significant to the US and worldwide vaccination for current and future coronavirus pandemics and other infectious diseases.

2:40 Refreshment Break in the Exhibit Hall & Last Chance for Poster Viewing (Grand Ballroom)

4:40 Close of Summit

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- Corporate logo with link within the conference proceedings denoting Corporate Support Sponsorship
- Two (2) main conference registrations – excludes access to short courses and training seminars
- Two (2) booth-only registrations
- Additional main conference registrations available at a discount for your staff – limited to 5
- Pre-& post-conference mailing lists for onetime usage through a third-party mail house
- On-site signage designating your company as a Corporate Support Sponsor
- 50-word company description in the conference materials
- Corporate logo link on the conference website
- Conference discount email for your clients & prospects

For more information regarding exhibit and sponsorship, please contact:

Companies A-K

Sherry Johnson
Senior Business Development Manager
781-972-1359
sjohnson@healthtech.com

Companies L-Z

Jon Stroup
Senior Business Development Manager
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jstroup@healthtech.com

Join Us in Boston!

Hotel and Travel

Conference Venue and Hotel:
Sheraton Boston
39 Dalton Street
Boston, MA 02199

Discounted Room Rate: $239
Discounted Room Rate Cut-off Date: July 18, 2022

BOOK TODAY »

Top Reasons to Stay at the Sheraton Boston

- No Commute, since meeting takes place at hotel!
- Steps away from the Prudential Center & Copley Place with shopping and restaurants
- Local attractions including Newbury Street, Eataly, Boston's South End and Boston Public Gardens within walking distance
- Boston Duck Tours departure location is outside the Huntington Avenue entrance for the Prudential Center.

Boston was recently named America's Favorite City by readers of Travel and Leisure Magazine and boasts a wealth of historic landmarks in a dynamic cultural setting. The Freedom Trail, Old North Church, Paul Revere's House, and Faneuil Hall Marketplace are just a few examples of this city's rich and varied history. After a full day of presentations and networking, you can saunter out from the hotel and visit nearby Boston attractions, including the famed "North End" for a fabulous Italian meal, the New England Aquarium, the lively Faneuil Hall, historic sites, and much more.

Can't Make it to Boston?

Join via Our Robust Virtual Platform

- INTUITIVE INTERFACE
- COMPANY BRANDING (LOGO)
- VIRTUAL BOOTH
- PRODUCT VIDEOS
- ROUNDTABLES
- GAMIFICATION (98 90)
- DOWNLOADS
- LIVE CHAT
- 1:1 NETWORKING
- MATCHMAKING
- PRODUCT DIRECTORY
- LIVE SESSIONS
- RECORDED SESSIONS
- INTEGRATED SCHEDULER
- POSTER SESSIONS

LEARN MORE »

Pricing & Registration

The 14th Annual BIOPROCESSING SUMMIT

Flexible Registration Policy

Seamlessly switch between in-person and/or virtual registration

Select an in-person or virtual option, and you have the flexibility to switch your preferred event experience at any time leading up to the conference.

PREMIUM PACKAGE

(Includes access to all conferences, training seminars, and networking events. Plus, On-Demand access. You are allowed to move between conference sessions to attend presentations taking place at the same time.)

	COMMERCIAL	ACADEMIC, GOVERNMENT, HOSPITAL AFFILIATED
STANDARD PRICING AFTER JULY 8 AND ONSITE	$3,199	$1,549

BASIC PACKAGE

(Includes access to ONE conference and/or training seminar, and networking events. Plus, On-Demand access. You are allowed to move between conference sessions to attend presentations taking place at the same time.)

STANDARD PRICING AFTER JULY 8 AND ONSITE	$2,149	$1,099

GROUP DISCOUNTS

HAVE YOUR COLLEAGUES OR ENTIRE TEAM ATTEND!

Purchase a full price registration and participants from the same organization will receive a 25% discount when registering through the Group Registration page.

For more information on group discounts contact Bill Mote at 781-972-5479.

LEARN MORE

VIRTUAL REAL-TIME OPTIONS

Includes access to virtual conferences and event features including the virtual exhibit hall, poster presentations, interactive breakout groups, facilitated networking, on-demand access and more!

LEARN MORE

POST-EVENT ON-DEMAND ONLY

Includes post-event recorded access to ALL conferences. Does not include access to live Q&A or networking.

LEARN MORE

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

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