Program, Abstracts & Conference Information

7 – 12 OCTOBER 2018 | OMNI GROVE PARK INN | ASHEVILLE, NC

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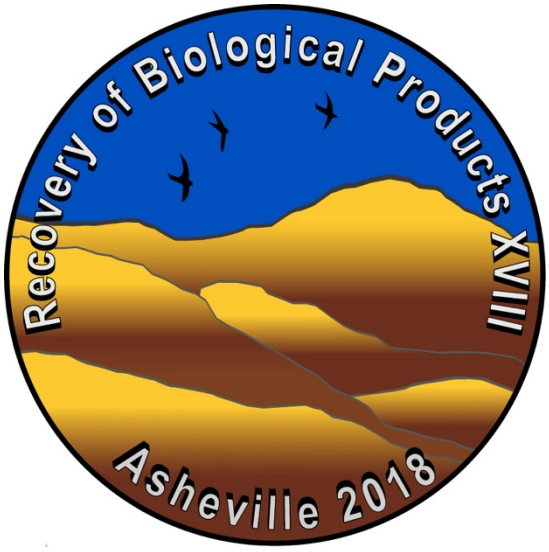
Recovery of Biological Products XVIII

Omni Grove Park Inn
Asheville, North Carolina

7 - 12 October 2018



An International Conference
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Division of Biochemical Technology

Conference Management Provided by:
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Hotel and Conference Information

Saturday 6 October Renaissance Hotel, Asheville

Reception and Dine-Around

LOCATION: Top of the Plaza of the Asheville Renaissance Hotel

On Saturday 6 Oct, participants and registered guests arriving early to RXVIII can enjoy the hospitality of the Renaissance Hotel to enable them to tour Asheville and its surroundings. To heighten their experience, all are invited to a welcome reception at the Hotel, where they can meet old friends and new RXVIII delegates while enjoying drinks and appetizers. Then join a group at a pre-reserved table at your choice of top restaurants in Asheville, the dining capital of the South.

RXVIII Meeting 7 - 12 October Omni Grove Park Inn

Omni Select Guest® Loyalty Program

We encourage all delegates and guests of the RXVIII meeting to join the Omni Select Guest® Loyalty Program prior to attending the meeting. The Loyalty Program is free to join at www.omnihotels.com/loyalty/join-now and will provide you important benefits, including express check-in/check-out and free WiFi in your room.

Guest Hospitality Suite

LOCATION: Coolidge DE

From Noon – 3 PM, participants and registered guests of RXVIII are welcome to gather to meet and enjoy a drink and light snacks.

Conference Registration

LOCATION: Vanderbilt Wing Registration Area

Conference Registration will be open Sunday 7 October from noon to 6:00 PM. The Conference Staff will be there and throughout the meeting to assist you with anything you need. Please do not hesitate to contact a staff member if you have a question regarding the schedule, activities, attire or any other aspect of the program.

Wear Your Official RXVIII ID

Please wear your RXVIII ID (name badge) to all Technical Sessions and other official events. Badges will be checked upon entrance to all technical sessions and social events.

Meeting Locations

Daily General Sessions

LOCATION: Grand Ballroom A & B (Vanderbilt wing)

Poster Sessions

LOCATION: Grand Ballroom C (Vanderbilt wing)

Daily Breakfast Buffet

LOCATION: Grove Park Inn Pavilion

Other Meals and Events

Consult the Daily Conference Program in this document to find the locations of all remaining meals and events. Lunch and dinner locations vary, so please do check your schedule for locations. There will also be staff present to direct you.

Poster Presentations

LOCATION: Grand Ballroom C (Vanderbilt wing)

Posters must be in place no later than 6:00 PM Sunday 7 October, and removed by 4:30 pm Thursday 11 Oct. Consult the Scaling New Heights in Bioprocessing subsection of this program to identify the numbered poster board that has been assigned to display your poster. **Monday Session** - All Even Numbered Posters. **Wednesday Session** - All Odd Numbered Posters. Posters remaining after 9 AM on Friday October 12 will be discarded.

Speaker Instructions

Speakers, you should upload and preview your presentations prior to your scheduled talk time. Please make every attempt to hand in your presentation to the conference desk staff via a flash drive no later than the session before your scheduled presentation so that it may be loaded onto the presentation computer in advance. Should you need to transfer your presentation another way, please let the staff know and they will provide other options. Note that a dedicated presentation computer will be provided and use of your own device is therefore discouraged.

Messages

There will be a message board located in the Vanderbilt wing Registration area. Please check the board during breaks. Messages will not be personally delivered and technical sessions will not be interrupted.

Recording and Photography

Both audio and visual recording of any oral session during the Conference is strictly prohibited. Likewise, photographic documentation of posters is not permitted unless expressly permitted by the presenting author. Delegates should directly ask presenters if they wish to have copies of slides, posters, or other materials.

Networking Activities Info

If you have pre-registered for Networking Activities, your activity tickets will be included in your registration materials. Please be sure to bring your assigned ticket(s) with you to each activity. If you have not pre-registered or would like to make changes to your reservation, you will have the opportunity to do so at the registration desk.

Attire

Dress during the conference is casual. Typical high temperatures in Asheville in October are in the low to mid 70's (°F)/low 20s (°C). Typical low temperatures are in the low to mid 50's (°F)/ 5 - 10 (°C.) Please check the weather forecast for Asheville before packing and bring appropriate attire (e.g. sunscreen, hats, rain gear, etc).

Grove Park Inn Activities

As a 5-star resort, the historic Grove Park Inn offers a multitude of activities that can be enjoyed separately from the activities offered in the RXVIII conference program. These include

- **Golf** on a Donald Ross designed course (please reserve your tee times before you travel to the meeting)
- The **Resort Pools** and the associated Cabana Grill and Bar
- A day at the 43,000 sq. ft. **Spa at the Grove Park Inn**, recently named one of the top 5 spas in North America by Conde Nast Travel. (Please make your reservations before you travel to the meeting)
- **Tennis or a Gym Work-Out** at the Grove Park Inn Sports Complex
- **Guided History Tours of the Grove Park Inn** resort grounds. Offered Wed to Sat

Hotel Check-Out and Payment

Hotel accommodations from Sunday, 7 Oct to Friday, 12 Oct are included in your registration fee. For those staying additional nights prior to and/or after the conference whose reservations were made through Precision Meetings & Events, Inc. (the RXVIII conference management company), please present your credit card to the front desk clerk upon arrival (please note that the charges for those additional nights will appear on your personal folio). Any personal expenses incurred at the hotel, such as telephone, fax, Internet access fees, bar bills, use of recreational facilities, and food (other than scheduled conference meals), are the responsibility of each attendee and/or their guest(s) and must be paid upon check-out.

Conference Program Overview

Time	SUNDAY, OCT 7
	BioProcessing 3.0 - A Vision for Future Biologics Manufacturing
8:00 – 11 AM	**Check Out** **Asheville Renaissance** (Saturday arrivals wishing to see Asheville before the start of RXVIII)
Noon – 6:00 PM	**RXVIII Registration Desk Open** (Grove Park Inn Check-In as well) Vanderbilt Wing Registration Area **Guest Hospitality Suite** Cooledge DE Room
3:00 PM	**Welcome to RXVIII (Opening Remarks)** Grand Ballroom A & B
3:10 – 5:00 PM	**Oral Session 1: Revolutionary Products & Process Development** CHAIRS: Nina Bauer, Brian Kelley Grand Ballroom A & B
5:00 PM	**Adapting Gene Editing Technologies for Therapeutic Uses** Ed Rebar Sangamo Therapeutics Grand Ballroom A & B
6:00 PM	**RXVIII Conference Welcome Reception** Vanderbilt Terrace
7:00 PM 8:00 PM	**RXVIII Opening Banquet** Heritage Ballroom **Gene Therapy – A Long Time Coming** Olivier Danos RegenXBio, Inc. Heritage Ballroom
9:15 PM	**Early Posters Viewing** Grand Ballroom C

Time	MONDAY, OCT 8
	Downstream Processing of an Expanding Biologics Portfolio
6:30 - 8:30 AM	**Breakfast Buffet** Grove Park Inn Pavilion
8:30 - 10:15 AM	**Oral Session 2: Bioprocessing New mAb Modalities** CHAIRS: Nihal Tugcu, Marcel Ottens Grand Ballroom A & B
10:15 AM	**Coffee and Social Break** Grand Ballroom Pre-Function Hall
10:40 AM to Noon	**Oral Session 3: Emerging Biological Classes** CHAIRS: Kent Goklen, Anne Kantardjieff Grand Ballroom A & B
Noon – 12:45 PM	**The Case for Continuous Manufacturing to Improve Global Access to Therapeutics** Lisa Connell-Crowley Just Biotherapeutics Grand Ballroom A & B
12:45 PM	**Boxed Lunch Pick-Up** Grand Ballroom Pre-Function Hall
1:30 – 6:00 PM	**Networking Activities** (Optional) Chartered buses to activities depart from the 7th Floor Exit (Vanderbilt Wing) of the Grove Park Inn. Please arrive 15 minutes before the scheduled start of your activity.
6:00 PM	**Low Country Supper** Skyline Mountainview Terrace
7:30 - 10:00 PM	**Poster Session I: Scaling New Heights in Bioprocessing** (Even Numbered Posters) CHAIRS: Michelle Butler, Sanchayita Ghose, Bramie Lenhoff Grand Ballroom C

Conference Program Overview

Time	TUESDAY, OCT 9
Downstream Processing in the Age of Big Data and Automation	
6:30 - 8:30 AM	**Breakfast Buffet** Grove Park Inn Pavilion
8:30 - 9:50 AM	**Oral Session 4: Quantifying Manufacturability** CHAIRS: Jurgen Hubbuch, Ranga Godavarti Grand Ballroom A & B
9:50 AM	**Morning Social Break** Vanderbilt Terrace
10:10 – 11:30 AM	**Oral Session 5: Big Data and the Fully Realized QbD Process** CHAIRS: Jayme Franklin, Karol Lacki Grand Ballroom A & B
11:30 AM - 1:00 PM	**Southern BBQ Lunch Buffet** Skyline Mountainview Terrace
1:00 – 2:45 PM	**Oral Session 6: Integrated and Continuous Bioprocessing** CHAIRS: Suzanne Farid, Konstantin Konstantinov and Aaron Noyes Grand Ballroom A & B
2:45 PM	**Afternoon Social Break** Vanderbilt Terrace
3:15 – 4:40 PM	**Oral Session 7: The Need for Speed - Automated Bioprocess Design** CHAIRS: Jennifer Pollard, Benjamin Tran Grand Ballroom A & B
5:15 PM	**Buses Depart for Highland Brewery** (Meet at Entrance of the Grove Park Inn)
6:00 – 10:00 PM	**Special Session: The Highland Games** *In silico predictions from gene sequence data* • Beer and Appetizers • The Highland Games • Tapas Dinner CHAIRS: Bruno Marquez, Richard Willson
9:45 PM	**Busses depart for GPI every 15 minutes**

Time	WEDNESDAY, OCT 10
Next Generation Tools and Operations for ProcessDesign, Control and Optimization	
6:30 - 8:30 AM	**Breakfast Buffet** Grove Park Inn Pavilion
8:30 - 9:50 AM	**Oral Session 8: Disruptive Downstream Processing Tools and Technologies** CHAIRS: Jorg Thommes, Nooshafarin Sanaie Grand Ballroom A & B
9:50	**Coffee and Social Break** Vanderbilt Terrace
10:15 – 11:00 AM	**Human Plasma Fractionation: A 75-year Journey of Innovation, Ingenuity and a Degree of Serendipity** Nigel Titchener-Hooker University College London Grand Ballroom A & B
11:00 AM – 1:00 PM	**Lightning Round – Next Gen Downstream Bio-Process Development** CHAIRS: Hanne Bak, Glen Bolton Grand Ballroom A & B
1:00 PM	**Boxed Lunch Pick-Up** Grand Ballroom Pre-Function Hall
1:30 – 5:45 PM	**Networking Activities** (Optional) Chartered buses to activities depart from the 7th Floor Exit (Vanderbilt Wing) of the Grove Park Inn. Please arrive 15 minutes before the scheduled start of your activity.
6:00 PM	**French Broad Dinner** Blue Ridge Restaurant
7:30 – 10:00 PM	**Poster Session II: Scaling New Heights in Bioprocessing** (Odd Numbered Posters) CHAIRS: Michelle Butler, Sanchayita Ghose, Bramie Lenhoff Grand Ballroom C

Conference Program Overview

Time	THURSDAY, OCT 11
The Future of Bioprocess Development and Management	
6:30 - 8:30 AM	**Breakfast Buffet** Grove Park Inn Pavilion
8:30 - 9:50 AM	**Oral Session 10: Evolving Membrane and Chromatographic Separations** CHAIRS: Andrew Zydney, Massimo Morbidelli Grand Ballroom A & B
9:50 AM	**Coffee and Social Break** Vanderbilt Terrace
10:20 - 11:40 AM	**Oral Session 11: Case Studies on the Future of Bioprocessing** CHAIRS: Raquel Orozco, Jeff Salm Grand Ballroom A & B
11:40 AM - 12:30 PM	**30 Spokes to Define One Hub: Management of Residual Uncertainty in Biopharmaceutical Development and Manufacturing** Jeffrey Baker US FDA Grand Ballroom A & B
12:30 PM	**Mexican Lunch Buffet** Blue Ridge Restaurant **Recovery Board Meeting** Eisenhower Conference Room
2:00 – 3:45 PM	**Oral Session 12: Case Studies on the Control of Future Bioprocesses** CHAIRS: Gisela Ferreira, Bernt Nilsson Grand Ballroom A & B
4:30 PM	**Buses Depart for Closing Event and Banquet**
5:00 – 10:00 PM	**RXVIII Conference Special Event and Banquet** Biltmore Estate • Biltmore House Tours • Gala Reception • Awards Banquet • Roaring 20's Swing Dance

Time	FRIDAY, OCT 12
6:30 - 9:00 AM	**Breakfast Buffet** Grove Park Inn Pavilion
8:30 - 10:00 AM	**RXVIII Conference Close**

Daily Technical Program Schedule

Sunday 7 Oct

"BioProcessing 3.0 - A Vision for Future Biologics Manufacturing"

3:00 PM **Welcome to RXVIII (Conference Co-Chairs)**

Oral Session 1: Revolutionary Products and Process Development

CHAIRS: Nina Bauer and Brian Kelley

Time	
3:10 PM	**Introductory Remarks**
3:20 PM	**Gene Therapy Approaches for Treating Hemophilia A: Strategy for Process Development, Manufacturing and Control** Robert Baffi, BioMarin Pharmaceuticals, Inc.
3:45 PM	**When Accelerated CMC Timelines Meet Innovative Therapies: Have We Really Learned the Right Lessons, Fast Enough** Gene Schaefer, Janssen Research and Development
4:10 PM	**Individualized mRNA-Based Cancer Immunotherapy** Andreas Kuhn, BioNTech RNA Pharmaceuticals, Inc.
4:35 PM	**Recovery of Antibodies Produced in a Cell-Free Expression System Derived from *E. coli*** Alex Steiner, Sutro Biopharma
4:50 PM	**Announcement - The Recovery of Biological Products Satellite Meetings Series** Brian Kelley, Recovery Conference Board

Keynote Address

Host: Jon Coffman

5:00 PM **Adapting Gene Editing Technologies for Therapeutic Applications**
Ed Rebar, Sangamo Therapeutic, Inc.

5:45 PM **Announcements from RXVIII Organizing Committee**

Plenary Lecture

Host: Jon Coffman

8:00 PM **Gene Therapy - A Long Time Coming**
Olivier Danos, RegenXBio, Inc.

Monday 8 Oct

"Downstream Processing of an Expanding Biologics Portfolio"

Oral Session 2: Bioprocessing of New mAb Modalities

CHAIRS: Nihal Tugcu and Marcel Ottens

Time	
8:30 AM	**Introductory Remarks**
8:35 AM	**Fitting Bispecific Antibodies into an Established Downstream Process: A Case Study** Timothy Iskra, Pfizer, Inc.
9:00 AM	**Double-Digit Titers and Highly Cost Effective Processing of Nanobodies® via *P. pastoris*** Willem Van de Velde, Ablynx NV
9:25 AM	**Hetero-mAb QbD: Know Your Product and Product-Related Impurities to Make Better Decisions at Variant Selection, Clone Selection, and Affinity Capture Resin Selection** Brian Bowes, Eli Lilly and Co.
9:50 AM	**Integrated Continuous Biomanufacturing (ICB) and Complex mAb Modalities: Quality with Productivity** Aleksandar Cvetkovic, Sanofi, Inc.

Oral Session 3: Emerging Biological Classes and the Multi-Scale Problem

CHAIRS: Anne Kantardjieff and Kent Göklen

Time	
10:40 AM	**Introductory Remarks**
10:45 AM	**Case Studies from Accelerated, Parallel Tech Transfers of a Recombinant Protein Vaccine to Internal and External Manufacturing Sites** Kristin Valente, Merck & Co.
11:10 AM	**Establishment of a Modular Process for the Production of Clinical-Grade Exosomes as Next Generation Biotherapeutics** Aaron Noyes, Codiak Bio, Inc.
11:35 AM	**Next Generation CAR-T Production** Matthew Westoby, Juno Therapeutics, Inc.

Keynote Address

Host: Jon Coffman

Noon	**The Case for Continuous Manufacturing to Improve Global Access to Therapeutics** Lisa Connell-Crowley, Just Biotherapeutics, Inc.

Tuesday 9 Oct

"Downstream Processing in the Age of Big Data and Automation"

Oral Session 4: Quantifying Manufacturability

CHAIRS: Ranga Godavarti and Juergen Hubbuch

Time	
8:30 AM	**Introductory Remarks**
8:35 AM	**Chemical and Physical Rules for Assessing Antibody Developability** Peter Tessier, University of Michigan
9:00 AM	**Manufacturability Considerations for Antibody-Drug Conjugates: Emerging Issues and Strategies** Michaela Wendeler, MedImmune, Inc.
9:25 AM	**Mechanistic Insight into Two-Peak Elution Behaviors of Therapeutic IgG4 in Cation Exchange Chromatography** Xuankuo Xu, Bristol-Myers Squibb

Oral Session 5: Big Data and the Fully Realized QbD Process

CHAIRS: Jayme Franklin and Karol Lacki

Time	
10:10 AM	**Introductory Remarks**
10:15 AM	**Computational and Big Data Driven Biomanufacturing** Steven Cramer, Rensselaer Polytechnic Institute
10:40 AM	**Machine Learning Techniques in the Context of Quality by Design** Annika Kleinjans, Roche, Inc.
11:05 AM	**Estimation of Platform Process Variation from Large Portfolio Datasets** Roger Hart, Amgen, Inc.

Oral Session 6: Integrated and Continuous Bioprocessing

CHAIRS: Suzanne Farid and Konstantin Konstantinov

Time	
1:00 PM	**Introductory Remarks**
1:05 PM	**Analysis of Economic Drivers for Integrated Continuous Bioprocessing of mAbs, Including Risk Assessment** Guenter Jagschies, GE Healthcare, Inc.
1:30 PM	**Building the Case for Implementation of an Integrated Manufacturing Process** Jeffrey Salm, Pfizer, Inc.
1:55 PM	**Whole Process Residence Time Distribution Modeling: The Key to Batching in a Continuous World** Mark Brower, Merck & Co.
2:20 PM	**Advanced in Attribute Measurement for Next-Generation Continuous Biomanufacturing** Gang Xue, Amgen, Inc.

Oral Session 7: The Need for Speed - Automated Bioprocess Design and Validation

CHAIRS: Jennifer Pollard and Ben Tran

Time	
3:15 PM	**Introductory Remarks**
3:20 PM	**In Silico Process Development for Orthogonally Selective Integrated Separations** Nicholas Vecchiarello, Rensselaer Polytechnic Institute
3:45 PM	**Process Clearance Predictions Using Mathematical Modeling** Jessica Yang, Genentech, Inc.
4:10 PM	**A QbD-Guided Model-Based Approach to Chromatography Process Development and Characterization** Tobias Hahn, GoSilico, Inc.

Evening Session: The Highland Games and Dinner

***In silico* prediction of molecular properties and processing options from only gene sequence data**

Co-Chairs: Bruno Marques and Richard Wilson

Time	
5:15 PM	**Buses depart for the Highland Brewery**
6:00 PM	**Welcome Reception and Beer Tasting** Emcee: Jon Coffman
6:45 PM	**Introducing THE HIGHLAND GAMES** Richard Willson
7:00 PM	**Recognition of Data Generators for Competition Scoring** Raquel Orozco, Minni Aswath and Eike ZImmermann (Boehringer-Ingelheim), John Schiel (NIST), Jan Griesbach, Stefan Hepbildikler and Thanmaya Peram (Roche/Genentech)
7:10 - 8:30 PM	**Prediction Team Presentations** Merck & Co. (Speaker: John Welsh) GlaxoSmithKline (Speaker: Siddharth Parimal) Roche/Genentech (Speaker: Saeed Izada) MedImmune (Speaker: Gisela Ferreira)
8:30 PM	**Tapas Dinner, Beer Tasting and Tours of the Brewery**
9:15 PM	**Scoring Summary - Where Are We Accurate and Not So Accurate?** Bruno Marques
9:30 PM	**Highland Games Award Ceremony** Bruno Marques, Richard Willson and Jon Coffman
9:45 PM	**First Bus Departs back to Grove Park Inn** (every 15 minutes thereafter)

Wednesday 10 Oct

"Next Generation Tools and Technologies for Process Design, Control and Optimization"

Oral Session 8: Disruptive Downstream Processing Tools and Technologies

CHAIRS: Nooshafarin Sanaie and Jorg Thommes

Time	
8:30 AM	**Introductory Remarks**
8:35 AM	**Applying Transformative Technologies to Biomanufacturing: Combining Process, Plant and Operations to Realize High-Capacity Drug Substance Production** Steven Doares, Biogen, Inc.
9:00 AM	**In silico Bioprocess Modeling: How to Ensure Implementation Success** Ferdinand Stuekler, Roche, Inc.
9:25 AM	**An End-to-End Processing Approach that Includes Spectral Deconvolution** Peter Tiainen, Novo Nordisk, Inc.

Keynote Address

Host: David Roush

10:15 AM	**Human Plasma Fractionation: A 75-Year Journey of Innovation, Ingenuity and a Degree of Serendipity** Nigel Titchener-Hooker, University College London

Oral Session 9: Lightning Round - Next Generation Downstream Bioprocess Development

CHAIRS: Hanne Bak and Glen Bolton

Time	
11:00 AM	**Introductory Remarks**
11:10 AM	**The Emerging Rules of Engagement for Viral Vector Recovery** Dan Bracewell, University College London
11:20 AM	**Pairing Affinity Monolithic Chromatography with Flow-Through RP-HPLC for the Rapid Purification of mRNA Therapeutics** Mary Stahley, Alexion Pharmaceuticals, Inc.
11:30 AM	**CHRETO Technology™ - a Novel, Cost-Effective and Flexible Single-Use Platform Technology for Affinity Purification** Jan Kyhse-Andersen, CHRETO, Inc.

Time	
11:40 AM	**Use of 3D Printing for the Development of Next Generation Single-Use Continuous Purification Platforms - Opportunities in Continuous Countercurrent Tangential Chromatography** Andrew Zydney, Penn State University
11:50 AM	**Comparison of Batch, Modified Batch, Simulated Moving Bed, and Continuous Countercurrent Tangential Chromatography for Flow-Through Anion Exchange Purification of a Monoclonal Antibody** Travis Tran, Regeneron Pharmaceuticals, Inc.
Noon	**How a 3D Printed Bed Support Lattice Can Extend Performance, Productivitiy and Reliability of Chromatography Processes** Guido Stroehlein, JSR Life Sciences
12:10 PM	**Evaluating the Effect of Flow Mechanics on Critical Process Parameters in a Continuous Viral Inactivation Reactor** Matthew Brown, Boehringer Ingelheim, Inc.
12:20 PM	**Super-Batch Chromatography: Enhancing Downstream Productivity Using an Existing Facility and Equipment through Rapid Cycling of Short Columns** Dharmesh Kanani, Teva Pharmaceuticals, Inc.
12:30 PM	**Efficient and Cost-Effective Manufacturing of Antibody Biotherapeutics Employing Novel Integrated Chromatography Technology for Continuous Processing** Mariangela Spitali, UCB Parma, Inc.
12:40 PM	**Innovations in Engineering for Next-Generation Manufacturing Systems** Rob Fahrner, Pfizer, Inc.

Thursday 11 Oct

"The Future of Bioprocess Development and Management"

Oral Session 10: Evolving Membrane and Chromatographic Separations

CHAIRS: Andrew Zydney and Massimo Morbidelli

Time	
8:30 AM	**Introductory Remarks**
8:35 AM	**Regularities and Anomalies in Modeling Protein Elution in Ion-Exchange Chromatography** Abraham Lenhoff, University of Delaware
9:00 AM	**Mechanistic Modeling of the Loss of Protein Sieving Due to Internal and External Fouling of Microfilters** Glen Bolton, Amgen, Inc.
9:25 AM	**Competitive Adsorption of Antibodies in Protein A Chromatography: Implications for Variant Resolution** Todd Przybycien, Rensselaer Polytechnic Institute

Oral Session 11: Future Bioprocessing Case Studies - Next Generation Processing Facilities

CHAIRS: Raquel Orozco and Jeff Salm

Time	
10:20 AM	**Introductory Remarks**
10:25 AM	**Is Technology the Limitation or Are We? - Simple Evolution of Current Technologies Can Enable Revolutionary Change in Biopharmaceutical Drug Development** Development Joseph Shultz, Novartis Pharma AG
10:50 AM	**Process Economics and Optimization for Ultra-Low Cost Vaccine Manufacture** Tania Pereira Chilima, University College London
11:15 AM	**Downstream Challenges in Clinical-Scale Production of rAAV Vectors for Gene Therapy** David Gray, Sangamo Therapeutics, Inc.

Keynote Address

Host: David Roush

11:40 AM **Thirty Spokes to Define One Hub: Management of Residual Uncertainty in Biopharmaceutical Development and Manufacturing**
Jeffrey Baker, US Food and Drug Administration

Oral Session 12: Case Studies on the Control of Future Bioprocesses

CHAIRS: Gisela Ferreira and Bernt Nilsson

Time	
2:00 PM	**Introductory Remarks**
2:05 PM	**Process Analytical Technology (PAT) for Biologics: A "Disruptive" Toolbox for Process Development and Characterization** Douglas Richardson, Merck & Co.
2:30 AM	**Leveraging Light: Spectroscopy as a PAT Tool for Biologics** Matthias Rudt, Karlsruhe Institute of Technology
2:55 AM	**Process Analytical Technologies for Connected Downstream Processing** Huanchun Cui, Novartis, Inc.
3:20 PM	**Process Science to Regulatory Science: Control Strategy 2018** David Robbins, MedImmune, Inc.

Keynote Speakers

Biographies and Abstracts

Ed Rebar

Sangamo Therapeutics, Inc.
Sunday 7 October, 5:00 PM
Grand Ballroom A & B

Olivier Danos

RegenXBio, Inc.
Sunday 7 October, 8:00 PM
Grand Ballroom A & B

Lisa Connell-Crowley

Just Biotherapeutics, Inc.
Monday 8 October, Noon
Grand Ballroom A & B

Nigel Titchener-Hooker

University College London
Wednesday 10 October, 10:15 AM
Grand Ballroom A & B

Jeffrey Baker

US Food and Drug Administration
Thursday 11 October, 11:45 AM
Grand Ballroom A & B

Ed Rebar

Biography

Edward Rebar, Ph.D. is Chief Technology Officer at Sangamo Therapeutics, which focuses on developing zinc finger proteins for therapeutic editing and regulation of targeted genes. In prior roles at Sangamo Ed has served as Vice President and Senior Director of Technology, capacities in which he has directed the design and characterization of zinc finger proteins for therapeutic applications and also helped to establish Sangamo's zinc finger protein design platform. Ed has coauthored 60 publications relating to the development of customized DNA binding proteins and nucleases for genome editing, as well as numerous patents. Prior to joining Sangamo he was a post-doctoral fellow at the University of California, Berkeley. Ed earned his B.S. in Biochemistry from Rutgers University and his Ph.D. in Biophysics and Structural Biology from MIT.

Adapting Gene Editing Technologies for Therapeutic Applications

Edward Rebar
Sangamo Therapeutics

Proteins that can be designed to target user-chosen sites within our genome provide powerful tools for engineering cells with new and therapeutically useful properties. When target recognition is accompanied by site-specific cleavage, these reagents can mediate highly efficient and precise gene disruption, gene editing or gene addition, offering the prospect of single-step genome repair for diverse pathological conditions. Such capabilities may also be applied to engineer producer cells for enhancing the yield and quality of biological therapeutics. These proteins may also be designed to mediate other molecular outcomes besides cleavage, for example the introduction of base edits and epigenetic modifications, as well as the activation or repression of proximal genes. The rich and expanding diversity of effector functions, as well as the prospect for multiplex interventions, points to the potential of these technologies to engineer cells with behaviors that are increasingly sophisticated and finely tuned for therapeutic benefit. The development of this field has been enabled by the establishment of key platforms for engineering proteins that can recognize and cleave new, user-chosen DNA sequences, in particular zinc finger nucleases (ZFNs), transcription activator-like effector nucleases (TALENs) and CRISPR/Cas systems. This talk will provide a brief overview of the design principles and capabilities for each, along with strategies for applying these platforms for the development and production of therapeutics. Key innovations that have enhanced activity, specificity and versatility will also be discussed. The talk will conclude with a consideration of current and future medical applications, with an emphasis on Sangamo's programs for addressing key indications including cancer and tauopathies via both ex vivo and in vivo engineering of targeted cells with new, therapeutically beneficial properties.

Olivier Danos

Biography

Dr. Olivier Danos is Senior Vice President and Chief Scientific Officer at RegenXBio. He is a world-renowned pioneer in the field of gene therapy, and has dedicated his career to advancing the use of this technology to develop life-saving therapies for patients. Olivier joined RegenXBio in 2017 from Biogen where he was a Senior VP in charge of Cell and Gene Therapy. Over the past twenty years, he has played leadership roles in cell and gene therapy as Director of the Gene Therapy Consortium of the University College of London, at the Necker Hospital – Enfants Malades in Paris, as Chief Scientific Officer of Genethon and as senior director of research at Somatix Therapy Corporation. He has held senior research positions in France at the Centre National de la Recherche Scientifique and at the Institut Pasteur. Olivier is the former President and a founding member of the European Society of Gene and Cell Therapy. Olivier received a Master's in Genetics and Molecular Biology at University of Paris Orsay, and his Ph.D. at the Pasteur Institute and University of Paris Dideros.

Gene Therapy – A Long Time Coming

Olivier Danos
RegenXBio, Inc.

Gene therapy is a technology by which nucleic acids are introduced into cells to compensate for a deficit or to modify their properties with the goal of treating diseases. The notion of therapeutic reprogramming of cells represents a radical change from traditional approaches where a therapeutic response is triggered by a small molecule or a protein biologic. Although these principles were laid out in the 1960's their practical implementation had to wait for technological breakthroughs and progresses that happened over the past three decades. Powerful gene transfer and gene modifying tools have been designed based on our refined understanding of biomolecular and cellular processes. Vectors can be engineered from viruses or assembled form synthetic parts, allowing to express virtually any protein in any cell type. They can also be designed to transfer antisense RNAs, mRNAs, siRNAs or components of a gene editing complex. With these tools, we can perform genetic reprogramming with high levels of precision in cultured cells, including stem cells. Here, I will discuss how technologies using lentiviral and Adeno Associated Viral (AAV) vectors, are being harnessed for the development of new treatments for genetic as well as acquired diseases. The field is maturing rapidly, with multiple clinical proofs of concept obtained over the past five years, and in a few cases, products reaching market authorization. Yet, our ability to translate the full potential of genetic reprogramming into treatments remains limited. A full understanding of the pharmacology of gene transfer vectors is still lacking. The concept of one-off curative treatments through genetic reprogramming challenges the classical notions of efficacious dose, pharmacokinetics and pharmacodynamics. Specific toxicology issues need to be addressed, including genotoxicity and immune responses against the vector and/or the transgene product. Manufacturing of gene transfer vectors at a commercial scale is often suboptimal. With the emergence of the first marketed gene therapy products, gene therapy is now recognized as a bona fide therapeutic modality, worthy of interest for most players in the biopharmaceutical industry. Consequently, the current limitations of gene therapy are increasingly being addressed by drug development and bio-manufacturing industry experts rather than academic scientists. Yet, advances in our scientific knowledge will be needed before we can achieve a precise control of therapeutic gene transfer in time and space and apply gene therapy treatments without triggering toxic immune responses.

Lisa Connell-Crowley

Biography

Lisa Connell-Crowley is Director of Downstream Process Design at Just Biotherapeutics, leading an innovative group scientists and engineers to drive down the cost of biotherapeutics through streamlined, cost-effective process and plant design. She has 15 years of downstream process development experience, including 12 years at Amgen as a downstream group leader, with expertise in downstream process development and scale-up, batch and multi-column chromatography, high throughput resin screening, and viral clearance strategies. Additionally, she has been a CMC process team leader for several early and late stage monoclonal antibody, Fc fusion and biosimilar products**.** Dr. Connell-Crowley received her Ph.D. in Biochemistry from the Baylor College of Medicine.

The Case for Continuous Manufacturing to Improve Global Access to Biotherapeutics

Lisa Connell-Crowley
Just Biotherapeutics

The biopharmaceutical industry currently faces multiple challenges, including the pressure to reduce health care costs, the rise in personalized medicine with smaller patient populations, and the needs of developing countries. In response, many companies are transitioning from batch to continuous manufacturing to reduce costs and increase flexibility through the development of simplified, highly productive processes that can be run within a small footprint facility. This presentation will provide an overview of integrated, continuous manufacturing in the industry and discuss the drivers and challenges of developing a continuous manufacturing platform for implementation in a distributed, podular manufacturing facility at Just Biotherapeutics. Additionally, key technological, quality and regulatory challenges will be highlighted as areas be addressed in order to facilitate industry acceptance and adoption of continuous manufacturing.

Nigel Titchener-Hooker

Biography

Nigel Titchener-Hooker (CEng, FIChemE, FREng) is Dean of the Faculty of Engineering Sciences and a Professor in the Department of Biochemical Engineering at University College London where he directs the Hub for Future Targeted Therapies. The Hub's goal is to create the technological and supply chain solutions for the next generations of biological medicines where precision of diagnosis will see both stratified and fully personalized drug products. The Hub involves collaboration with an international consortium of over 35 companies and valued at over £50 Million. As the first director of Engineering Doctorate Centre for Bioprocess Leadership, Professor Titchener-Hooker managed a portfolio of over 60 doctorate programs with companies spanning the whole breadth of the biotech industry from small to macromolecular processing and to the nascent regenerative medicines area. Nigel has held consultancies with a broad range of international companies and serves on the editorial board of key peer-reviewed journals. He was elected a Fellow of the Royal Academy of Engineering in 2008 in recognition of his pioneering work on biopharmaceuticals manufacturing. In 2013, he led Biochemical Engineering to the award of a Queens Anniversary Award for its contributions to UK healthcare. He is a Fellow of the Institution of Chemical Engineers.

Human Plasma Fractionation: A 75-year Journey of Innovation, Ingenuity, and a Degree of Serendipity

Nigel Titchener-Hooker, Paul R. Foster and Michael Hoare
University College London

Three quarters of a century ago, EJ Cohn and JT Edsall produced their monograph on proteins, amino acids, and peptides. This deep level of knowledge lay behind the Cohn-Cold Ethanol Fractionation scheme devised for the fractionation of bulk plasma proteins into therapeutically significant products. Cohn and Edsall at Harvard had been exploring protein structure and function for years but this was the defining moment! The fractionation scheme that provided a scaleable method for the delivery of life-saving treatments. In the context of the Second World War, this was a pivotal invention but juts the start of a remarkable journey in bioprocessing. This talk will follow that legacy forward over the 75-year period tracing how these insights and the ingenuity of others has helped us to create bioprocesses and methods that have shaped our industry. In particular, the paper will follow the flow of the ideas surrounding the Cohn and Edsall work as these were picked up in the post-war years at University College London and the Scottish National Blood Transfusion Service, which over the decades has built a profound spectrum of process knowledge that enables us to integrate protein precipitation as a key unit operation for modern isolation and purification sequences. The thesis developed in the talk will be that some of the most enduring advances stem from a deep and fundamental understanding of our biological systems, translated by elegant engineering into robust and resistant practice. True too is the fact that from such flashes of inspiration come many other, unforeseen developments and that these events are crucial to our capacity to deliver truly advanced bioprocess solutions for the recovery of biological products.

Jeffrey Baker

Biography

Jeffrey Baker, PhD, received a bachelor's degree in biochemistry and molecular biology at Northwestern University, his doctorate in biochemistry from the University of North Texas, and completed post-doctoral studies at the University of California, Berkeley. Dr. Baker joined the biosynthetic process development group at Eli Lilly & Co in 1988. While at Lilly, Dr. Baker led the development of several biologics from bench top purification to global launch, supported post-launch manufacturing process development activities, and established and led Lilly's Global Validation Practices Team. After completing his certification as a Lean Six Sigma Black Belt, Dr. Baker led several corporate initiatives related to assessment and enhancement of control, capability, and functional governance processes in pharmaceutical manufacturing. Dr. Baker left Lilly to be Sr. Director of Manufacturing Science and Technology at MedImmune, a subsidiary of AstraZeneca. In 2011, Dr. Baker was appointed Deputy Director of the Office of Biotechnology Products in the Center for Drug Evaluation and Research at the FDA. Dr. Baker helped establish the Quality Management System used today in OBP and has been recognized with several CDER citations for leadership and program development. He was a member of the team which conceptualized and stood up the Office of Pharmaceutical Quality at FDA and has participated in the development of both regulatory guidances and operational policies for CDER and reporting offices.

Thirty Spokes to Define One Hub: Management of Residual Uncertainty in Biopharmaceutical Development and Manufacturing

Jeffrey Baker
US Food and Drug Administration

As biopharmaceuticals have evolved from biological extracts to complex mixtures to specified and highly characterized proteins with the potential to be biosimilars, analytical and process control strategies have evolved from rote art, where process defines the product, to complex systems of purification and process control framed by increasingly complex analytics. Current trends in rapid process development, flexible manufacturing, and process intensification focus on high impact measurements to assure product quality, consistency of supply, and mitigation of unanticipated outcomes. Nevertheless, the parameters and attributes measured are in largest part predictors a far greater number of unmeasured interactions and, indeed, patient experiences. This talk will examine issues associated with quality assurance and regulatory oversight of residual uncertainty in biopharmaceutical process development and lifecycle management. This talk will offer the challenge that even as analytics and knowledge management systems become more sophisticated there remain unmet needs in mapping and managing "what we don't know". In the emerging environment, our ability to articulate and manage uncertainty and probabilized outcomes will impact biopharmaceutical development and manufacturing and robust assurance of clinically relevant patient outcomes.