

PROGRAM

SUNDAY SEPTEMBER 8		
8:00am – 5:30pm	REGISTRATION HOURS	COLUMBUS FOYER
11:00am – 12:30 pm	WORKSHOP 1 T C POINT-OF-CARE CELL PROCESSING Chair: Janice Davis-Sproul, MAS, MT(ASCP)SBB, Johns Hopkins Medicine Speakers: Lee Buckler, BEd, LLB, Cell Therapy Group Yong Fan (FDA), MD, Office of Cellular, Tissue, and Gene Therapies, CBER/FDA Brian Barnes, PhD, Arteriocyte Medical Systems	COLUMBUS A
	WORKSHOP 2 C T S UPGRADING TO CLOSED, LARGE SCALE PRODUCTIONS-REQUIREMENTS FOR EQUIPMENT AND TRAINING Chair: Shirley Bartido, PhD, MBA, Memorial Sloan Kettering Cancer Center Speakers: Steven Roberts, PhD, Lonza Clive Glover, PhD, GE Healthcare Shirley Bartido, PhD, MBA, Memorial Sloan Kettering Cancer Center	COLUMBUS B
	QUALITY TOOLBOX SESSION 1 Q T RISK ASSESSMENTS AND MITIGATION Chair: Olive Sturtevant, MHP, MT(ASCP)SBB, SLS, CQA(ASQ), Dana-Farber Cancer Institute Speakers: Karen Snow, (ASCP)BB, CQA(ASQ), Bone Marrow Transplant Program, Massachusetts General Hospital Angela Ondo, MT(ASCP), Johns Hopkins Hospital	COLUMBUS C
12:30 pm – 1:30 pm	LUNCH	COLUMBUS FOYER
1:30 pm – 3:00 pm	WORKSHOP 3 C Q RAW MATERIALS, ANCILLARY PRODUCTS, QUALIFICATION Chair: Claudia Zylberberg, PhD, Akron Biotech Speakers: Fouad Atouf, PhD, U.S. Pharmacopeia Lynn Csontos, STEMCELL Technologies Inc Jennifer Solomon, PhD, STEMCELL Technologies Inc Kim Nguyen, PhD, Terumo BCT	COLUMBUS A
	WORKSHOP 4 T C WORKING WITH BIOTECH COMPANIES – MANAGEMENT'S PERSPECTIVE Co-Chairs: Janice Davis-Sproul, MAS, MT(ASCP)SBB, Johns Hopkins Medicine Lynn O'Donnell, PhD, The Ohio State University, Comprehensive Cancer Center Speakers: Olive Sturtevant, MHP, MT(ASCP)SBB, SLS, CQA(ASQ), Dana-Farber Cancer Institute Paul Griggs, MSc, Fate Therapeutics, Inc. Panelists: Lynn O'Donnell, PhD, The Ohio State University, Comprehensive Cancer Center Janice Davis-Sproul, MAS, MT(ASCP)SBB, Johns Hopkins Medicine Doug Padley, MT(ASCP), Mayo Clinic	COLUMBUS B

T Technical/Ops, Q Quality/Ops, S Scientific (Clinicians, Principal Investigators), C Commercialization



SUNDAY SEPTEMBER 8 *continued*

1:30 pm – 3:00 pm	QUALITY TOOLBOX SESSION 2 Q T MANAGING DEVIATIONS Aisha Khan, <i>PhD</i> , University of Miami School of Medicine William Janssen, <i>PhD</i> , H. Lee Moffitt Cancer Center	COLUMBUS C
3:00pm – 3:15pm	COFFEE BREAK WITH EXHIBITS	GRAND BALLROOM ABCD
3:15 pm – 4:45 pm	WORKSHOP 5 S MSC BIOLOGY Chair: Allan Dietz, <i>PhD</i> , Mayo Clinic Speakers: Allan Dietz, <i>PhD</i> , Mayo Clinic Satoru Otsuru, <i>MD, PhD</i> , The Children's Hospital of Philadelphia Robert Deans, <i>PhD</i> , Athersys, Inc.	COLUMBUS A
	WORKSHOP 6 S OPTIMIZING BENCH TO BEDSIDE TRANSLATION OF RESEARCH – AN UPDATE FROM PACT Chair Deborah Wood, <i>MT ASCP</i> , The EMMES Corporation Speakers: David H. McKenna Jr., <i>MD</i> , University of Minnesota Medical Center Frits van Rhee, <i>MD, PhD, MRCP (UK)</i> , Myeloma Institute for Research and Therapy, University of Arkansas for Medical Sciences	COLUMBUS B
	QUALITY TOOLBOX SESSION 3 Q T TECHNICAL WRITING LED BY THE CO-EDITORS OF THE TELEGRAFF Deborah Griffin, <i>MSc</i> , University of Pittsburgh Lynn O'Donnell, <i>PhD</i> , The Ohio State University, Comprehensive Cancer Center	COLUMBUS C
5:00pm – 5:30pm	WELCOME ADDRESS	COLUMBUS AB
5:30pm – 8:00pm	NETWORKING RECEPTION	GRAND BALLROOM ABCD (EXHIBIT HALL)

MONDAY SEPTEMBER 9

7:00am – 4:00pm	REGISTRATION HOURS	COLUMBUS FOYER
7:30am – 8:30am	TECHNICAL SESSION 1 Q T THE WHO, WHAT, WHERE, WHEN, WHY AND HOW OF FDA DEVIATION REPORTING Chair: Karen Snow, (<i>ASCP</i>) <i>BB, CQA(ASQ)</i> , Bone Marrow Transplant Program, Massachusetts General Hospital Speakers: Fran Rabe, <i>MS, CQM(ASQ)</i> , University of Minnesota Molecular and Cellular Therapeutics Sharon O'Callaghan, <i>MT(ASCP)</i> , FDA/Center for Biologics Evaluation and Research, Office of Compliance and Biologics Quality	COLUMBUS A
	TECHNICAL SESSION 2 C Q TRANSPORTATION LOGISTICS FOR CELL THERAPY PRODUCTS Chair: Janice Davis-Sproul, <i>MAS, MT(ASCP)SBB</i> , Johns Hopkins Medicine Speakers: Doug Padley, <i>MT(ASCP)</i> , Mayo Clinic Daniel O'Donnell, Fisher BioServices	COLUMBUS B

T Technical/Ops, **Q** Quality/Ops, **S** Scientific (Clinicians, Principal Investigators), **C** Commercialization

MONDAY SEPTEMBER 9 *continued*

7:30am – 8:30am	<p>TECHNICAL SESSION 3 T Q</p> <p>MOVIN' ON UP: DESIGNING, COMMISSIONING & QUALIFYING YOUR PIECE OF THE PIE</p> <p>Chair: Lynn O'Donnell, <i>PhD</i>, The Ohio State University, Comprehensive Cancer Center</p> <p>Speakers: Lynn O'Donnell, <i>PhD</i>, The Ohio State University, Comprehensive Cancer Center Kurt Last, <i>Specialty Operations Solutions, Inc</i></p>	COLUMBUS C
8:45am – 10:15am	<p>PLENARY SESSION 1 S</p> <p>CORD BLOOD EXPANSION</p> <p>Chair: Colleen Delaney, <i>MD, MSc</i>, Fred Hutchinson Cancer Research Center</p> <p>Speakers: Colleen Delaney, <i>MD, MSc</i>, Fred Hutchinson Cancer Research Center William Reed, <i>MD</i>, Cellerant Mitch Horwitz, <i>MD</i>, Duke University School of Medicine</p>	COLUMBUS ABC
10:15am – 10:30am	COFFEE BREAK WITH EXHIBITS	GRAND BALLROOM ABCD
10:30am – 12:00pm	<p>WORKSHOP 7 C Q</p> <p>CELL THERAPY MEDICAL TOURISM AND UNPROVEN STEM CELL THERAPIES</p> <p>Chair: Linda Miller, <i>MPA</i>, Foundation for the Accreditation of Cellular Therapy</p> <p>Speakers/Panelists: Leigh Turner, <i>PhD</i>, University of Minnesota Center for Bioethics & School of Public Health Kurt Gunter, <i>MD, FASCP</i>, Cell Medica Inc. Lee Buckler, <i>BEd, LLB</i>, Cell Therapy Group Michael Lill, <i>MD</i>, Cedars-Sinai Medical Center</p>	COLUMBUS A
	<p>WORKSHOP 8 T</p> <p>CRYOPRESERVATION BIOLOGY</p> <p>Chair: Lizette Caballero, <i>BSc, MT(ASCP)</i>, UCSF Adult Blood and Marrow Transplant Lab</p> <p>Speakers: Allison Hubel, <i>PhD</i>, University of Minnesota Utkan Demirci, <i>PhD</i>, Harvard Medical School Brigham & Women's Hospital Michael H. Creer, <i>MD</i>, Pennsylvania State University Hershey Medical Center</p>	COLUMBUS B
10:30am – 12:00pm	<p>WORKSHOP 9 C</p> <p>MANUFACTURING COMPARABILITY</p> <p>Chair: David Williams, <i>FREng, DEng, PhD</i>, EPSRC Centre for Innovative Manufacturing in Regenerative Medicine, Loughborough University</p> <p>Speakers: Nick Medcalf, <i>MSc</i>, Loughborough University Robert Deans, <i>PhD</i>, Athersys, Inc. David Williams, <i>FREng, DEng, PhD</i>, EPSRC Centre for Innovative Manufacturing in Regenerative Medicine, Loughborough University</p>	COLUMBUS C
12:00pm – 1:30pm	LUNCH	GRAND BALLROOM ABCD
12:15pm – 1:15pm	<p>CORPORATE TUTORIAL HOSTED BY TERUMO BCT</p> <p>CELL THERAPY MANUFACTURING: PRE-CLINICAL, CLINICAL, AND COMMERCIAL SCALE</p> <p>Speaker: Jim Beltzer, <i>PhD</i>, Terumo BCT</p>	COLUMBUS A



MONDAY SEPTEMBER 9 *continued*

12:15pm – 1:15pm	PATIENT ADVISORY STATEMENT Q & A Speakers: <i>ISCT North America Legal & Regulatory Affairs Committee Co-Chairs:</i> William Janssen, PhD, H. Lee Moffitt Cancer Center Karen Nichols, Esq. RAC, OvaScience, Inc.	COLUMBUS B
1:30pm – 3:00pm	PLENARY SESSION 2 S C MSC TOPIC – TRANSLATIONAL DEVELOPMENT OF UNIVERSAL DONOR MSC-LIKE CELLULAR PHARMACEUTICALS Chair: Jacques Galipeau, MD, FRCP(C), Emory University Speakers: Jacques Galipeau, MD, FRCP(C), Emory University Ohad Karnieli, PhD, Pluristem Therapeutics Paul Simmons, PhD, Mesoblast Ltd	COLUMBUS ABC
3:00pm – 3:15pm	COFFEE BREAK WITH EXHIBITS	GRAND BALLROOM ABCD
3:15pm – 4:45pm	WORKSHOP 10 S T CORD BLOOD: DISTINGUISHING THE GOOD, THE BAD, AND THE UNACCEPTABLE Chair: Karen Snow, (ASCP)BB, CQA(ASQ), Bone Marrow Transplant Program, Massachusetts General Hospital Speakers: Fran Rabe, MS, CQM(ASQ), University of Minnesota Molecular and Cellular Therapeutics Karen Ballen, MD, Massachusetts General Hospital	COLUMBUS A
	WORKSHOP 11 C T REGULATORY CHALLENGES Chair: Lynn O'Donnell, PhD, The Ohio State University, Comprehensive Cancer Center Speakers: Lizette Caballero, BSc, MT(ASCP), UCSF Adult Blood and Marrow Transplant Lab Sharon Miller, MT(ASCP), CHS(ABHI), ClinImmune Labs	COLUMBUS B
3:15pm – 4:45pm	ORAL ABSTRACTS SESSION 1 Chair: Paul Eldridge, PhD, St. Jude Children's Research Hospital SOLUTION-PHASE CROSS-TALK AND REGULATORY INTERACTIONS BETWEEN MULTIPO-TENT ADULT PROGENITOR CELLS AND PERIPHERAL BLOOD MONONUCLEAR CELLS Speaker: Richard Maziarz, MD, Oregon Health Science University ADMINISTRATION OF TUMOR-SPECIFIC CYTOTOXIC T LYMPHOCYTES ENGINEERED TO RESIST TGF-β TO SUBJECTS WITH EBV-ASSOCIATED LYMPHOMAS Speaker: Catherine Bollard, MD, FRCP(C), Children's National Medical Center ACTIN CYTOSKELETAL DISORIENTATION FOLLOWING CRYOPRESERVATION LIMITS EN-GRAFTMENT POTENTIAL OF HUMAN MSCS Speaker: Raghavan Chinnadurai, PhD, Emory University THE EMERGENCE OF UNIVERSAL IMMUNE RECEPTORS FOR HIGHLY PERSONALIZED T CELL THERAPY Speaker: Daniel Powell Jr., PhD, University of Pennsylvania CHALLENGES IN THE TRANSLATION AND COMMERCIALIZATION OF CELL THERAPIES Speaker: Aaron Levine, M.Phil., PhD, Georgia Institute of Technology	COLUMBUS C
4:45pm – 5:45pm	POSTER SESSION RECEPTION	GRAND BALLROOM FOYER

T Technical/Ops, **Q** Quality/Ops, **S** Scientific (Clinicians, Principal Investigators), **C** Commercialization

TUESDAY SEPTEMBER 10 *continued*

7:00am – 4:00pm	REGISTRATION HOURS	COLUMBUS FOYER
7:30am – 8:30am	TECHNICAL SESSION 4 S NOVEL PUBLISHED GUIDELINES FROM ISCT MSC COMMITTEE Chair: Donald Phinney, <i>PhD</i> , Scripps Florida Speakers: Jacques Galipeau, <i>MD, FRCP(C)</i> , Emory University Donald Phinney, <i>PhD</i> , Scripps Florida	COLUMBUS A
	TECHNICAL SESSION 5 C REIMBURSEMENT 101 Chair: Richard Maziarz, <i>MD</i> , Oregon Health and Science University Speakers: Joel Brill, <i>MD, AGAF, CHCQM</i> , FAIR Health Inc. Robert Wanerman, <i>Epstein Becker & Green</i>	COLUMBUS B
	TECHNICAL SESSION 6 T Q FACT ACCREDITATION OF MINIMAL AND MORE THAN MINIMAL MANIPULATION OF RESEARCH PRODUCTS Chair: Linda Miller, <i>MPA</i> , Foundation for the Accreditation of Cellular Therapy Speaker: Deborah Griffin, <i>MSc</i> , University of Pittsburgh	COLUMBUS C
8:45am – 10:15am	PLENARY SESSION 3 S REDIRECTING IMMUNITY THROUGH CHIMERIC ANTIGEN RECEPTOR T CELLS Chair: Bruce Levine, <i>PhD</i> , University of Pennsylvania Speakers: Mark Dudley, <i>PhD</i> , Surgery Branch, National Cancer Institute, NIH Isabelle Rivière, <i>PhD</i> , Cell Therapy and Cell Engineering Facility, Memorial Sloan Kettering Cancer Center Bruce Levine, <i>PhD</i> , University of Pennsylvania	COLUMBUS ABC
10:15am – 10:30am	COFFEE BREAK WITH EXHIBITS	GRAND BALLROOM ABCD
10:30am – 12:00pm	WORKSHOP 12 T Q PRODUCT LABELING - UPDATES, PERSPECTIVES AND ISBT 128 Chair: Paul Eldridge, <i>PhD</i> , St Jude Children's Research Hospital Speakers: Leigh Sims Poston, <i>BS, MT(ASCP)</i> , University of Virginia Medical Center Lizette Caballero, <i>BSc, MT(ASCP)</i> , UCSF Adult Blood and Marrow Transplant Lab William Janssen, <i>PhD</i> , H. Lee Moffitt Cancer Center	COLUMBUS A
	WORKSHOP 13 S PRECLINICAL ASSESSMENT OF INVESTIGATIONAL CELLULAR AND GENE THERAPY PROJECTS Chair: Doug Padley, <i>MT(ASCP)</i> , Mayo Clinic Speakers: Doug Padley, <i>MT(ASCP)</i> , Mayo Clinic Christopher Breuer, <i>MD</i> , Nationwide Children's Hospital	COLUMBUS B
	WORKSHOP 14 C S COMMERCIALIZATION CASE STUDY: CELL THERAPY FROM CONCEPT TO MARKET Chair: Robert Deans, <i>PhD</i> , Athersys, Inc. Speakers: Robert Preti, <i>PhD</i> , Progenitor Cell Therapy Dolores Baksh, <i>PhD</i> , Organogenesis	COLUMBUS C

T Technical/Ops, Q Quality/Ops, S Scientific (Clinicians, Principal Investigators), C Commercialization



TUESDAY SEPTEMBER 10 *continued*

12:00pm – 1:30pm	LUNCH	GRAND BALLROOM ABCD
12:15pm – 1:15pm	<p>REGULATORY UPDATES FROM THE JAPANESE PMDA (PHARMACEUTICALS AND MEDICAL DEVICES AGENCY) COLUMBUS A</p> <p>Chair: Akihiro Shimosaka, <i>PhD</i>, Research Foundation for Community Medicine</p> <p>Speakers: Akihiro Shimosaka, <i>PhD</i>, Research Foundation for Community Medicine, Daiju Okuda, <i>PhD</i>, Japanese PMDA</p>	
1:30pm – 3:00pm	<p>PLENARY SESSION 4 S C</p> <p>TISSUE ENGINEERING & ORGAN RECELLULARIZATION COLUMBUS ABC</p> <p>Chair: Allan Dietz, <i>PhD</i>, Mayo Clinic</p> <p>Speakers: Allan Dietz, <i>PhD</i>, Mayo Clinic Jeffrey Ross, <i>PhD</i>, Miromatrix Medical Inc. Christopher Breuer, <i>MD</i>, Nationwide Children's Hospital</p>	
3:00pm – 3:15pm	COFFEE BREAK WITH EXHIBITS	GRAND BALLROOM ABCD
3:15pm – 4:45pm	<p>WORKSHOP 15 C T</p> <p>HARMONIZING MANUFACTURING ACROSS MULTIPLE SITES AND REGIONS COLUMBUS A</p> <p>Chair: Karen Edward, <i>BSc, MT(ASCP)</i>, Mesoblast Inc.</p> <p>Speakers: Byron McAllister, <i>BT</i> McAllister Associates Inc. Karen Edward, <i>BSc, MT(ASCP)</i>, Mesoblast Inc EJ Read, <i>MD</i>, Fate Therapeutics, Inc.</p>	
	<p>WORKSHOP 16 C S</p> <p>THE PERSPECTIVES OF ACADEMIA AND INDUSTRY IN CELLULAR THERAPY RESEARCH COLUMBUS B</p> <p>Chair: Linda Miller, <i>MPA</i>, Foundation for the Accreditation of Cellular Therapy</p> <p>Speaker: Ian McNiece, <i>PhD</i>, MD Anderson Cancer Center</p>	
	<p>ORAL ABSTRACTS SESSION 2 COLUMBUS C</p> <p>Chair: Lizette Caballero, <i>BSc, MT(ASCP)</i>, UCSF Adult Blood and Marrow Transplant Lab</p> <p>UNDERSTANDING CELL THERAPY COST OF GOODS FOR COMPETITIVE ADVANTAGE Speaker: Mark McCall, <i>Loughborough University</i></p> <p>T-CELL ENGINEERING FOR ADOPTIVE IMMUNOTHERAPY USING TAL-EFFECTOR NUCLEASES (TALENTM) Speaker: Sophie Derniame, <i>PhD</i>, Collectis Therapeutics</p> <p>ENHANCED HOMING OF INTRAVENOUS BONE MARROW STROMAL CELLS (BMSC) TO THE KIDNEY BY PULSED FOCUSED ULTRASOUND (PFUS) IMPROVES OUTCOMES IN A MURINE MODEL OF ACUTE TUBULAR NECROSIS Speaker: Joseph Frank, <i>MD</i>, National Institute of Health Clinical Center</p> <p>A METHOD TO COMPARE IPSC COLONIES FROM DAY TO DAY AND ACROSS LABORATORIES Speaker: Gregory Cooksey, <i>PhD</i>, National Institute of Standards and Technology</p> <p>MAPPING THE FATE OF CELL THERAPY PRODUCTS IN PRECLINICAL SAFETY STUDIES BY ¹⁹F NMR Speaker: Eric Ahrens, <i>PhD</i>, Carnegie Melon University</p>	

WEDNESDAY SEPTEMBER 11

8:00am – 5:00pm	FACT WORKSHOP	COLUMBUS ABC
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T Technical/Ops, Q Quality/Ops, S Scientific (Clinicians, Principal Investigators), C Commercialization

SESSION DESCRIPTIONS

PLENARY SESSION 1

CORD BLOOD EXPANSION

Chair: Colleen Delaney, MD, MSc

Fred Hutchinson Cancer Research Center, Seattle, WA

This plenary session will focus on recently developed technologies for the ex vivo expansion of hematopoietic stem and progenitor cells for clinical application. Current clinical trials utilizing these expanded cell therapy products will be discussed, including both the challenges and opportunities of expanded cell-based therapies.

Colleen Delaney, MD, MSc

*Associate Member, Clinical Research Division
Director, Cord Blood Transplant and Research Program
Fred Hutchinson Cancer Research Center*

“THE PROMISE OF EX VIVO EXPANDED CORD BLOOD PROGENITOR CELLS FOR CLINICAL APPLICATION AS A MYELOID BRIDGE”

1. Review current clinical problems in cord blood transplant
2. Current strategies to overcome low cell dose associated with cord blood grafts
3. Describe ex vivo expansion strategies for cord blood stem/progenitor cells using Notch ligand

William Reed, MD

Cellerant, VP Clinical Development

“EARLY CLINICAL APPLICATION OF HUMAN MYELOID PROGENITOR CELLS (MPC) IN THE SETTINGS OF CORD BLOOD TRANSPLANTATION AND CHEMOTHERAPY”

1. Review the rationale for use of human MPC as an “off the shelf” product in neutropenia
2. Describe the preclinical data supporting the clinical development of human MPC
3. Review the early clinical data from phase 1 studies of Cellerant human MPC

Mitchell Horwitz, MD

*Associate Professor
Duke University School of Medicine*

“UMBILICAL CORD BLOOD STEM CELLS EXPANDED IN THE PRESENCE OF NICOTINAMIDE (NICORD) PROVIDE LONG TERM MULTI-LINEAGE ENGRAFTMENT”

1. Review the impact of low stem cell dose on the outcome of adult umbilical cord blood transplantation

2. Describe the advantages of expansion of both short-term and long-term repopulating cells from umbilical cord blood
3. Review the results of a pilot trial of NiCord®-expanded umbilical cord blood transplantation

PLENARY SESSION 2

MSC TOPIC – TRANSLATIONAL DEVELOPMENT OF UNIVERSAL DONOR MSC-LIKE CELLULAR PHARMACEUTICALS

Chair: Jacques Galipeau, MD FRCP(C)

Emory University

The use of “universal donor” MSCs in clinic is entering a mature phase III clinical trial phase required for final regulatory approval for marketing. The therapeutic promise of alternatively sourced industrial MSCs will be presented as well as conceptual analysis of cellular variables which may influence outcome of randomized trials

Jacques Galipeau, MD FRCP(C)

Emory University

“THE MESENCHYMAL STROMAL CELLS DILEMMA-A FAILURE ANALYSIS OF NEGATIVE PHASE III TRIAL OF INDUSTRIAL MESENCHYMAL STROMAL CELLS IN STEROID-RESISTANT GRAFT-VERSUS-HOST DISEASE”

1. Variance in potency of random donor allogeneic MSCs for immune modulation
2. Immunogenicity of unmatched random donor MSCs
3. Effect of senescence and cryopreservation on suppressor potency

Ohad Karnieli, PhD

Pluristem Therapeutics

“THE ROAD NOT TAKEN.... MOVING CELL THERAPY FROM THE BENCH TOP TO AN INDUSTRY”

1. Challenges in translating cell therapy from the bench to the clinics
2. Controlling the process as the key for success
3. PLX cells, an MSC-like universal cellular therapy

Paul Simmons, PhD

Mesoblast Ltd



PLENARY SESSION 3

REDIRECTING IMMUNITY THROUGH CHIMERIC ANTIGEN RECEPTOR T CELLS

Chair: Bruce Levine, PhD

Perelman School of Medicine, Department of Pathology and Laboratory Medicine, Abramson Cancer Center, University of Pennsylvania

Adoptive T-cell therapy in advanced metastatic melanoma or B-cell leukemias is garnering increasingly encouraging clinical data. Chimeric antigen receptors (CARs) are fusion proteins between single-chain variable fragments (scFv) from monoclonal antibodies recognizing tumor-associated antigens and intracellular signaling domains such as the CD3 ζ -chain cytoplasmic tail. Transduction of peripheral blood T lymphocytes with these targeting moieties confers specific cytotoxic immunoreactivity against cells expressing the target epitope. These agents have shown high activity in preclinical models of immunotherapy, and several sites have observed significant clinical responses as well.

Mark E. Dudley, PhD

Surgery Branch, National Cancer Institute, NIH

“OPTIMIZING T CELL PRODUCTION FOR EARLY PHASE CLINICAL TRIALS”

1. Process design considerations
2. Implementing change

Isabelle Rivière, PhD

Director, Cell Therapy and Cell Engineering Facility, Memorial Sloan Kettering Cancer Center, NY

“CD19-TARGETED T CELL THERAPIES FOR ADULT AND PEDIATRIC B CELL MALIGNANCIES”

1. Update on patient enrollment and clinical outcomes in the CAR T cell program at MSKCC
2. Comparative outcomes in ALL, CLL and B-NHL
3. Discussion of CAR T cell properties vs outcomes and safety

Bruce Levine, PhD

Perelman School of Medicine Department of Pathology and Laboratory Medicine, Abramson Cancer Center at the University of Pennsylvania

“CLINICAL APPLICATION OF CHIMERIC ANTIGEN RECEPTOR T CELLS DIRECTED AGAINST CD19 FOR B CELL MALIGNANCIES IN ADULTS AND CHILDREN”

1. Manufacturing Update
2. Clinical Update
3. Modified T cell Persistence Update

PLENARY SESSION 4

TISSUE ENGINEERING & ORGAN RECELLULARIZATION

Chair: Allan B. Dietz, PhD

Scientific Director, Human Cell Therapy Lab, Mayo Clinic, Rochester, MN

Tissue engineering and organ recellularization will take cell therapy to three dimensional reconstitution of organized tissues and functional organs. This session will discuss the growing field of tissue decellularization and recellularization in the terms of considerations of the process, tissues, pre-clinical data and trial design needed to move this exciting technology into the clinic.

Allan B. Dietz, PhD

Mayo Clinic

“TRANSLATIONAL CONSIDERATIONS FOR RECELLULARIZED TISSUES”

1. Understanding the unique difficulties in clinical scale recellularization
2. Combinations of matrix, cells and engineering to move towards organ recellularization and clinical use.

Jeff Ross, PhD

Miromatrix, Eden Prairie, Minnesota

“ENGINEERING A TRANSPLANTABLE, REVASCULARIZED LIVER GRAFT FOR THE TREATMENT OF LIVER DISEASE”

1. Describe a practical approach for data in support of clinical trials
2. Discuss considerations for experiments with re-cellularization of organs in large animal models

Christopher Breuer, MD

Director, Tissue Engineering Program, Nationwide Children's Hospital, Columbus, OH

“FROM THE BENCH TO THE BEDSIDE AND BACK AGAIN: THE DEVELOPMENT AND TRANSLATION OF THE TISSUE ENGINEERED VASCULAR GRAFT”

1. Define the role of basic science in translational research
2. Contrast rational clinical trial design and empiricism
3. Highlight the importance of understanding the mechanism of action of a product

WORKSHOP 1

POINT-OF-CARE CELL PROCESSING

Chair: Janice Davis-Sproul, MAS, MT(ASCP) SBB
Johns Hopkins Medicine

Around the world, Point-of-Care cell processing devices are in use in operating rooms and clinics for an assortment of indications. The aim of this session is to discuss how Point-of-Care cell processing devices are being used in North America. This session will provide an overview of these devices, discuss regulatory oversight and provide insights from a device manufacturer's perspective.

Lee Buckler, LLB
Founder & Managing Director, Cell Therapy Group

“AN OVERVIEW OF POINT-OF-CARE CELL PROCESSING TECHNOLOGIES IN U.S. CLINICAL USE”

1. To identify the primary categories and types of point-of-care cell processing technologies currently in clinical use in the United States
2. To provide examples of different technology platforms
3. To introduce top-level regulatory and commercial implications associated with the different types of technology

Yong Fan, MD
*Division of Cellular and Gene Therapies
Office of Cellular, Tissues, and Gene Therapies, CBER/FDA*

“AN OVERVIEW OF MEDICAL DEVICE REGULATIONS IN THE U.S.”

1. To briefly review the history of medical device regulation in the U.S.
2. To discuss medical device classifications and regulatory pathways
3. To provide examples of medical devices reviewed in CBER/OCTGT

Brian Barnes, PhD
*Vice President, Clinical and Regulatory Affairs
Arteriocyte*

“POINT-OF-CARE CELL PROCESSING DEVICES – A MANUFACTURER'S PERSPECTIVE”

1. To identify the challenges associated with clinical trials which use point-of-care cell processing devices
2. To discuss approaches to balancing regulatory compliance and commercial interests

WORKSHOP 2

UPGRADING TO CLOSED, LARGE SCALE PRODUCTIONS-REQUIREMENTS FOR EQUIPMENT AND TRAINING

Chair: Shirley M. Bartido, PhD, MBA
Memorial Sloan Kettering Cancer Center (MSKCC)

Cell therapy manufacturing needs are maturing to a level where production lot sizes need to expand well beyond 10 billion cells in order to support programs targeting trillions of cells per year. In this workshop, robust, scalable manufacturing workflows and the time, cost and risk of implementing viable cGMP manufacturing processes at this scale will be discussed. These factors are critical to the continuing successful clinical evaluation and ultimate commercialization of cell based therapeutics. Discussion of the development of specific scalable productions of CAR modified T cells and Retroviral Vector stocks will be presented.

Steven Roberts, PhD
LONZA Walkersville

“MULTIPLE OPTIONS FOR LARGE-SCALE PRODUCTION OF CELL THERAPEUTICS: EVALUATION AND IMPLEMENTATION”

1. Discuss process evaluation and proof of concept strategies specific to:
 - a. robotic handling of 40-layer cell factories
 - b. hyper-layer vessel technology
 - c. microcarriers in stirred bioreactors
5. Discuss the downstream cell concentration and washing options of continuous counter flow centrifugation and hollow fiber tangential flow filtration
6. Discuss the closed process integration with semi-automated aseptic vialing

Clive Glover, PhD
GE Healthcare

“THE XURI™ CELL EXPANSION SYSTEM FOR SCALE-UP OF CELLULAR IMMUNOTHERAPIES”

1. Xuri™ Cell Expansion System 5W and 25W are functionally closed, automated, single use systems
2. Xuri™ System 25W allows for complete control over the cell culture environment with the addition of data logging and remote monitoring and control. Furthermore, multiple systems can be controlled and monitored off a single computer giving a simplified path to scale out
3. Data showing that densities of 2.0×10^7 cells/mL in a 1 L volume can be routinely achieved will be presented



Shirley M. Bartido, PhD, MBA

Memorial Sloan Kettering Cancer Center (MSKCC)

**“CELL THERAPY PRODUCTS AND BIOREACTORS-
DEVELOPING A CLOSED SYSTEM”**

1. Identify the need for semi-closed/closed systems
2. Identify the proper supplies/equipment to achieve a closed system
3. Provide examples of semi-closed/closed systems processes that were developed

WORKSHOP 3

**RAW MATERIALS, ANCILLARY PRODUCTS,
QUALIFICATION**

Chair: Claudia Zylberberg, PhD,

CEO Akron Biotech

As cell therapies (CT) progress into the clinical space and eventual approach final approval, the quality and consistency of raw materials will come under increasing scrutiny. This session will bring raw material providers, a standard setting organization, and a company developing a product where the quality of the raw materials and its impact can be challenged. As the industry grows and cell therapies go through the regulatory evaluation so does the oversight of its individual components and its significance.

Fouad Atouf, PhD

Director, Biologics and Biotechnology, U.S. Pharmacopeia

**“STANDARDS FOR RAW MATERIALS USED IN THE
MANUFACTURING OF CELLULAR THERAPIES”**

1. Regulatory framework for cellular therapies and the raw/ ancillary materials
2. Quality of raw materials and impact on finished product
3. Compendia standards: challenges and opportunities

Lynn Csontos

Associate Director, Quality Assurance and Regulatory Affairs, STEMCELL Technologies Inc.

Jennifer Solomon, PhD

Manager, Key Relationships, STEMCELL Technologies

**“A RISK BASED APPROACH TO SOURCING,
CHARACTERIZING, AND APPROVING THE USE OF
ANCILLARY MATERIALS FOR CELL THERAPY”**

1. Outline risk based approach to choosing Ancillary Materials
2. Responsibilities in the process of qualifying Ancillary Materials
3. Review Case Studies

Kim Nguyen, PhD

Senior Scientist, Terumo BCT

**“RAW MATERIAL QUALIFICATION IN LARGE-SCALE
CELL THERAPY MANUFACTURING: THE ADVENT OF
AUTOMATION AND CLOSED SYSTEMS”**

1. Raw material sourcing, verification and validation, and supply chain logistics for reproducible large-scale automated clinical cellular therapy manufacturing.
2. Major issues and trends in manufacturing: Xeno- vs Serum-free and R&D vs cGMP/clinical grade reagents.
3. The role of strategic partnerships and R&D collaborations within industry and between industry and academia to advance ancillary products innovation.

WORKSHOP 4

**WORKING WITH BIOTECH COMPANIES –
MANAGEMENT’S PERSPECTIVE**

Co-Chairs: Lynn O’Donnell, PhD

The Ohio State University, Comprehensive Cancer Center

Janice Davis-Sproul, MAS, MT(ASCP) SBB

Johns Hopkins Medicine

Working with a biotech company to study the use of a novel cell therapy product or device can be exciting and challenging. How are costs recovered? What labels should be used? Which Standards apply? What should be included in a Quality Agreement? This session will focus on biotech/hospital collaborations from different perspectives - Laboratory Director, Quality Assurance, Laboratory Staff, and the Company (Technical and Regulatory). Scenarios will be discussed by a panel with a focus on encouraging attendee participation.

Olive Sturtevant, MHP, MT(ASCP)SBB, SLS, CQA(ASQ)

Director of Quality Assurance, Cell Therapies, Dana-Farber Cancer Institute

**“THE CHALLENGES AND PERKS IN WORKING WITH
BIOTECH COMPANIES”**

1. Review what regulations or standards need to be followed: US vs. EU vs. accrediting standards
2. Describe how to handle differences between the needs of the biotech company and your routine operations
3. Review what you need to know to recoup costs at various points during the terms of the agreement: development, validations, manufacturing during trial, post trial

Paul Griggs, MSc

Field Technical Specialist, Fate Therapeutics, Inc.

“THE CHALLENGES AND PERKS IN WORKING WITH CELL THERAPY FACILITIES: MANUFACTURING OF PROHEMA-CB”

1. Describe the strategy for initial training and qualification of Cell Processing Facilities
2. Discuss operations and clinical production support

Panelists:

Lynn O'Donnell, PhD, *The Ohio State University, Comprehensive Cancer Center*

Janice Davis-Sproul, MAS, MT(ASCP) SBB, *Johns Hopkins Medicine*

Doug Padley, MT(ASCP), *Mayo Clinic*

WORKSHOP 5

MSC BIOLOGY

Chair: Allan B. Dietz, PhD

Scientific Director, Human Cell Therapy Lab, Mayo Clinic, Rochester, MN

Mesenchymal stromal cells (CMSC) are being investigated for their therapeutic usefulness in academic and commercial settings for a wide variety of pathologies including stroke, wound healing, graft versus host disease and many others. Yet many key aspects of the mechanisms of action of observed responses are unknown.

Allan B. Dietz, PhD

Mayo Clinic

“CHARACTERIZING THE HEALING AND DIFFERENTIATION PROGRAMS OF MSC”

1. Understand a novel method to characterize MSC using qPCR
2. Understand differentiation concepts as they relate to MSC function

Satoru Otsuru, MD, PhD

The Children's Hospital of Philadelphia

“MSC MICROVESICLES AS MEDIATORS OF TISSUE REPAIR”

1. To understand the presence and function of microvesicles in MSC
2. To realize the potential of a future cell-free MSC therapy

Robert Deans, PhD

Exec VP Regenerative Medicine, Athersys, Inc

“CLINICAL DESIGN IN THE ADHERENT ADULT STEM CELL SPACE: BUILDING ON MECHANISM”

1. The spectrum of MSC functions and their clinical implications
2. Clinical trial design based on mechanisms of MSC function

WORKSHOP 6

OPTIMIZING BENCH TO BEDSIDE TRANSLATION OF RESEARCH – AN UPDATE FROM PACT

Chair: Deborah Wood, MT ASCP

The EMMES Corporation

This workshop will discuss the challenges in bench to bedside development for early phase cellular therapy products and the preclinical IND-enabling studies needed to meet regulatory expectations. The pathways for bringing promising therapies into clinical trials and the challenges and successes in launching early Phase 1 clinical trials will be illustrated in two case studies that have reached the clinical trial stages.

David H. McKenna, Jr., MD

Molecular & Cellular Therapeutics Facility University of Minnesota

“MSCS FOR ACUTE LUNG INJURY”

1. Review proof of principle studies in small animal models of ALI.
2. Summarize the general regulatory pathway in obtaining an IND for a cell therapy trial.
3. Discuss the regulatory journey for this particular IND, including the need for large animal studies.
4. Provide overview of a newly initiated multi-institutional clinical trial.

Frits van Rhee, MD, PhD, MRCP(UK)

Myeloma Institute for Research and Therapy, University of Arkansas for Medical Sciences

“NATURAL KILLER CELL THERAPY FOR HEMATOPOIETIC MALIGNANCIES”

1. Discuss rationale for NK cell therapy
2. Preclinical models of NK cell therapy
3. Optimization of NK cell therapy
4. Challenges in manufacturing clinical grade NK cells



WORKSHOP 7

CELL THERAPY MEDICAL TOURISM AND UNPROVEN STEM CELL THERAPIES

Chair: Linda Miller, MPA

Foundation for the Accreditation of Cellular Therapy

This panel presentation aims to identify the major concerns with cell therapy medical tourism and issues related to clinics offering experimental stem cell therapies. Efforts to educate and protect patients, develop risk-based assessment of treatments and providers, and the need for developing global standards for stem cell clinics offering unproven therapies will be discussed. Audience participants will be encouraged to provide input for developing a cohesive approach to fighting non-compliant stem cell clinics.

Leigh Turner, PhD

University of Minnesota Center for Bioethics & School of Public Health

“ETHICAL AND LEGAL ISSUES RELATED TO DOMESTIC CLINICS MARKETING ACCESS TO UNLICENSED AND UNPROVEN STEM CELL INTERVENTIONS.”

1. Both international clinics and domestic businesses market access to unlicensed and unproven stem cell-based interventions. While a substantial body of scholarship addresses “stem cell tourism,” this presentation will review ethical and legal issues associated with U.S.-based companies marketing domestic access to unlicensed and apparently noncompliant stem cell procedures.
2. The presentation will describe how various domestic clinics marketing stem cell interventions for multiple sclerosis, Parkinson’s disease, and other illnesses depict themselves as engaged in ethical and legal activity even though they appear to violate federal regulations and contemporary standards in research ethics.
3. The presentation will use particular examples of non-compliant practices, emphasize discussion of practical ethical issues, and ensure that ethical analysis is grounded in careful empirical study of clinics marketing noncompliant stem cell interventions.

Kurt Gunter, MD, FASCP

Cell Medica, Inc.

“CELL THERAPY PROFESSIONAL ORGANIZATIONS’ EFFORTS TO EDUCATE AND PROTECT PATIENTS”

1. Present the multi-stakeholder position statement on Medical Tourism and Unproven Stem Cell Therapies.
2. Discuss a proposed action plan to promote awareness and provide education to patients, advocacy groups, providers, and the public.

3. Discuss the need for global regulatory requirements, adverse event reporting, standardized informed consent, etc.

Lee Buckler, BEd. LLB

Cell Therapy Group

“HELPING PATIENTS AND THEIR FAMILIES ASSESS NON-COMPLIANT CELL THERAPY TREATMENTS: A RISK-BASED AND CONTEXT-DRIVEN APPROACH”

1. Identify rationale for assisting patients and their families assess non-compliant cell therapy treatments .
2. Identify key measures for assessing non-compliant cell therapy treatments.
3. Outline and discuss a case study demonstrating one way of assisting patients and their families assess these treatments in a patient-specific context.

Michael Lill, MD

Cedars-Sinai Medical Center

“DEVELOPMENT OF STANDARDS FOR REGENERATIVE MEDICINE BASED ON 15 YEARS OF EVOLUTION OF FACT STANDARDS FOR HEMATOPOIETIC STEM CELL THERAPY”

1. Discuss the drivers from the cell therapy field to initially develop FACT’s voluntary standards for hematopoietic stem cell therapy.
2. Describe the benefits of standardization for stakeholders as they relate to transplant centers, patients, advocacy groups, payers, regulatory bodies, cooperative groups, funding agencies, biopharma, and the international market.
3. Describe FACT’s current accreditation for hematopoietic-derived cellular therapy product processing with more than minimal manipulation.
4. Discuss the potential for utilizing FACT core standards to expand into non-hematopoietic stem cells for other clinical specialties.

WORKSHOP 8

CRYOPRESERVATION BIOLOGY

Chair: Lizette Caballero, BSc, MT(ASCP)

UCSF Adult Blood and Marrow Transplant Lab

Cell Cryopreservation maintains cellular life at sub-zero temperature by slowing down cellular biochemical reactions. It has been widely used in modern regenerative, transfusion, and reproductive medicine. Not surprisingly, what happens to a cell prior to freezing influences its ability to survive

the stresses of freezing and thawing. In this session, we will review the biology of current cryopreservation techniques based on slow and rapid freezing (conventional vitrification) approaches, discuss the manner by which prefreeze processing influences post thaw recovery and describe common pitfalls in determining post thaw viability and function and effective strategies to eliminate them. In addition, although highly reliable, conventional controlled-rate cryopreservation systems are subject to malfunction leading to conditions that may adversely impact product quality. Accordingly, we will also provide practical guidelines for interpretation of cryopreservation cooling curves to identify conditions where product quality may be compromised.

Allison Hubel, PhD

Biopreservation Core Resource, University of Minnesota

“BOOKENDS TO THE FREEZING PROCESS: PREFREEZE AND POST THAW ASSESSMENT”

1. Discuss the manner by which prefreeze processing influences post thaw recovery
2. Describe methods of assessing the state of your cell prior to cryopreservation
3. Describe common pitfalls in determining post thaw viability and effective strategies to determine post thaw recovery

Utkan Demirci, PhD

Harvard Medical School Brigham & Women's Hospital

“TRENDS IN CELL CRYOPRESERVATION TECHNOLOGIES”

1. Describe how cryo-injury usually affects the cells due to osmotic shock or toxicity of cryoprotectants (e.g., glycerol and DMSO).
2. Identify recent approaches based on using minimum volume vitrification and bio-inspired materials (e.g., ectoin and trehalose)
3. Share advances in cell cryopreservation technology and the limitations of conventional technologies and highlight future directions in cell cryopreservation.

Michael H. Creer, MD

Pennsylvania State University Hershey Medical Center

“PRACTICAL GUIDELINES FOR INTERPRETATION OF CRYOPRESERVATION CURVES TO IDENTIFY CRYOPRESERVATION INJURY”

1. Identify the essential elements of a typical CRF system and identify those components subject to operational failure.

2. Briefly describe laboratory approaches used to assess cryopreservation injury and their advantages and limitations
3. Illustrate the features of normal and abnormal cryopreservation curves and provide summary guidelines for cryopreservation curve interpretation (order to reflect the order of presentations)

WORKSHOP 9

MANUFACTURING COMPARABILITY

Chair: David Williams, FEng, DEng, PhD

Director, EPSRC Centre for Innovative Manufacturing in Regenerative Medicine, of Loughborough University

Shared understanding of how to show comparability in manufacturing is seen by many as having the potential to unlock the industry because of its effects on product availability to patients, the business model and its viability, robust and cost effective manufacturing and supply, and regulatory compliance. This session, including discussion and introduced by short presentations, will allow sharing of current perspectives on addressing the problem, highlight where industry has most concerns, make progress in defining how these concerns can best be addressed and the key stakeholders that need to be engaged in the process.

Nick Medcalf, MSc

Professor of Regenerative Medicine Manufacture, Loughborough University (formerly Bioprocessing Manager, Biologics & Spine, of Smith & Nephew)

“AN INDUSTRIAL PERSPECTIVE ON PROCESS COMPARABILITY FOR NEW THERAPEUTICS”

1. Industrial reality of current approaches to comparability during development
2. Impact of comparability protocols on flexibility of business model
3. Areas of focus for future work

Robert Deans, PhD

Executive Vice President, Regenerative Medicine, Athersys Inc.

“A TOOLBOX AND BLUEPRINT FOR COMPARABILITY TESTING IN PROCESS DEVELOPMENT”

1. Determining critical product attributes and composite testing requirements
2. Tactical value of broad ‘omics based comparability approaches
3. Practical experience in moving into xeno-free, automated manufacturing



David Williams, FEng, DEng, PhD

Director, EPSRC Centre for Innovative Manufacturing in Regenerative Medicine, Loughborough University

“PERMITTING ROLL-OUT OF CLINICALLY LED AUTOLOGOUS CELL THERAPIES”

1. Alternative manufacturing models
2. Regulatory and scientific challenges for comparability (an EU based perspective)
3. Issues and enablers: dealing with input variation, simultaneous sites or incremental addition of sites, establishing limits for safe expansion, manufacturing technology and standards enablers

WORKSHOP 10

CORD BLOOD: DISTINGUISHING THE GOOD, THE BAD, AND THE UNACCEPTABLE

Chair: Karen Snow, (ASCP)BB, CQA(ASQ)

Quality Assurance Officer Bone Marrow Transplant Program, Massachusetts General Hospital

Determining the best cord blood unit is a complex process that involves evaluation of the individual cord blood units available for the patient, the reputation/past experience with the cord blood bank, and methods used to process the unit. This session will explore attributes important from the physician’s perspective in trying to determine the very best cord blood unit for the patient and from the quality assurance perspective how to qualify an unrelated cord blood bank. Complex issues to be evaluated include HLA matching, HLA antibodies, additional cord attributes, as well as the multiple aspects of donor screening and testing, processing and accreditation examined during the qualification of a cord blood bank.

Fran Rabe, MS, CQM(ASQ)

Quality Manager, Director of Quality Assurance, University of Minnesota Molecular and Cellular Therapeutics

“ESTABLISHMENT OF AN UNRELATED CORD BLOOD BANK QUALIFICATION PROGRAM, A TRANSPLANT CENTER PERSPECTIVE; WHY BOTHER?”

1. Understand the importance of a strong qualification program
2. Differentiate between quality versus regulatory qualification criteria
3. Examine retrospective and prospective qualification approaches
4. Advantages and limitations to the establishment of a qualification system

Karen Ballen, MD Professor of Medicine

Harvard Medical School, Director, Leukemia Program, Massachusetts General Hospital

“SELECTION OF THE OPTIMAL CORD BLOOD UNIT”

1. Understand the importance of cell dose and HLA match in umbilical cord blood transplantation
2. Appreciate the importance of additional factors including HLA antibodies in cord blood selection
3. Recognize cord blood units that may be unacceptable for transplantation

WORKSHOP 11

REGULATORY CHALLENGES

Chair: Lynn O’Donnell, PhD, The Ohio State University

The U.S. federal regulations covering human cells, tissues and cell and tissue-based products (HCT/Ps, 21 CFR Part 1271) have been around since the proposed rules were published between 1998 and 2001, and cell therapy facilities have been living and breathing these regulations since the first effective date of January 21, 2004. Additional regulations for current Good Manufacturing Practices (cGMPs) for drugs and biologics, as well as regulations under the Clinical Laboratory Improvement Amendments (CLIA) apply to many cell therapy facilities depending on the type of products and testing they perform. Despite media coverage and lawsuits to the contrary, the vast majority of cell therapy facilities aspire and devote significant resources to achieving full compliance with these federal regulations. This session will examine how well the field is doing in this endeavor, as well as why there are still regulations that facilities find problematic in either interpretation or implementation. Three presentations by experts’ experiences with FDA inspections of their facilities will be followed by an interactive panel discussion, which will include results of a recent poll of cell therapy facilities.

Lynn O’Donnell PhD, The Ohio State University

Lizette Caballero, MT(ASCP)CM, University of California, San Francisco

Sharon Miller, MT(ASCP), CHS(ABHI), ClinImmune Labs

1. Learn about three facilities’ experiences with FDA inspections for HCT/P donor eligibility, GTPs and pre- and post-licensure BLAs
2. Evaluate potential industry misconceptions, limitations and areas for improvement
3. Hear lessons learned during the BLA submission and pre-approval and annual FDA inspections
4. Review results of peer group polling and discussion boards of cell therapy facility experiences with FDA inspections, unclear or difficult regulations and other regulatory challenges

5. Examine scenarios that demonstrate some regulations that continue to be challenging for facilities

WORKSHOP 12

PRODUCT LABELING – UPDATES, PERSPECTIVES AND ISBT 128

Chair: Paul W. Eldridge, PhD
St. Jude Children's Research Hospital

ISBT 128 is a system for identification and labeling of HCT/ Ps using an internationally standardized system that is commonly required by accrediting organizations (e. g, FACT). Updates to the standard will be discussed along with real-world perspectives of how to implement the standard in a cell therapy facility and manage changes in the standard. Use of ISBT 128 for licensed products will be reviewed.

Leigh Sims Poston, BS, MT(ASCP)
University of Virginia Medical Center

“ISBT 128 TERMINOLOGY CHANGES: WHERE IS MY MODIFIER?”

1. Review the changes in terminology and discuss the impact of the changes to the existing product codes
2. Help the attendees understand product codes and how to apply them to Cell Therapy products
3. Discuss how the terminology / code changes may impact those who have validated and implemented ISBT 128 for Cell Therapy Products

Lizette Caballero, BSc, MT(ASCP)
UUCSF Adult Blood and Marrow Transplant Lab

“ISBT 128 LABELING OF CELLULAR THERAPY PRODUCTS: WE DID IT! YOU CAN DO IT TOO.”

1. Discuss Validation Plan details
2. Share examples of tools and labels used during the process of validation
3. Discuss roadblocks encountered during the journey
4. Explain how are we planning to implement new changes

William E. Janssen, PhD
H. Lee Moffitt Cancer Center, University of South Florida

“ISBT 128 LABELING VERSUS NDC LABELING OF CELLULAR THERAPEUTICS – CONSIDERATIONS ON THE ROAD TO LICENSED PRODUCTS”

1. Examination of current FDA requirements for naming (proprietary versus generic names) and labeling of licensed drug products

2. Show the example of one currently commercialized licensed cell therapy product, and how it is labeled
3. Draw the comparison between “traditional pharmaceuticals” and cell therapy products that are addressed by ISBT 128 labeling
4. Identify ways that workshop participants may (and should) enter the conversation regarding licensed cell therapy product labeling

WORKSHOP 13

PRECLINICAL ASSESSMENT OF INVESTIGATIONAL CELLULAR AND GENE THERAPY PROJECTS

Chair: Doug Padley, MT (ASCP)
Research and Development Coordinator, Human Cellular Therapy Laboratory, Mayo Clinic, Rochester, MN

One of the challenges of bench to bedside translation of investigational cellular therapies is the design and execution of preclinical studies. In this session two speakers will review their experiences and lessons learned about preclinical activities that support cell therapy product development. The speakers will also review a recent draft guidance from the US FDA on this topic.

Doug Padley, MT (ASCP)
Research and Development Coordinator, Human Cellular Therapy Laboratory, Mayo Clinic, Rochester, MN

“PRECLINICAL INVESTIGATIONS FOR CELLULAR THERAPEUTICS: CHALLENGES AND LESSONS LEARNED”

1. Discuss the types of pre-clinical investigations performed to support a manufacturing platform for Mesenchymal stromal cells for a variety of indications
2. Review the highlights of a recent US FDA draft guidance document. Preclinical Assessment of Investigational Cellular and Gene Therapy Products

Christopher Breuer, MD
Deputy Vice Chair of Research and Director, Tissue Engineering Program, Ohio State University, Nationwide Children's Hospital

“THE CLINICAL RELEVANCE OF PRECLINICAL INVESTIGATIONS FOR BIOMATERIALS AND TISSUE ENGINEERED PRODUCTS”

1. Describe the challenges in choosing appropriate pre-clinical animal models to support clinical trials of tissue engineered products
2. Review the utility and advantages of using murine models to elucidate the primary mode of action of a product.



WORKSHOP 14

COMMERCIALIZATION CASE STUDY: CELL THERAPY FROM CONCEPT TO MARKET

Chair: Bob Deans, PhD

Exec VP Regenerative Medicine Athersys, Inc, ReGenesys, BVBA

Commercializing CT requires more than meets the eye. The manufacturing settings and models have a great impact on the cost of the therapy and, ultimately, in its market inception. This session will bring together a global CMO, a device company, and a CT company with product in the market to address these issues and other challenges they face in every step along the way. There are key aspects like scale-up, logistics, and design of devices that can assist in the streamline and cost reduction of the manufacturing of a CT product. These speakers will bring expertise and case studies for discussion.

Robert Preti, PhD, Progenitor Cell Therapy

Dolores Baksh

Head of Research and Development, Organogenesis

WORKSHOP 15

HARMONIZING MANUFACTURING ACROSS MULTIPLE SITES AND REGIONS

Chair: Karen Edward, BSc, MT(ASCP)

VP QA Operations, Mesoblast Inc

Biotechnology companies rely on contract manufacturing organizations, either commercial or hospital/academic-based laboratory facilities to manufacture cell therapy products for clinical trials and commercial use. This workshop will include different perspectives from 3 speakers on how this is managed successfully, as well as the challenges in harmonizing systems and standardizing products across facilities and regions.

Byron McAllister,

BT McAllister Associates, Inc.

“CHANGE CONTROL, AN ESSENTIAL ELEMENT FOR SUCCESSFUL TRANSITION TO COMMERCIAL SUCCESS”

1. Prospective written and approved change management plan;
2. Project management deliverables; and
3. Periodic effectiveness checks.

Karen Edward, BSc, MT(ASCP)

VP QA Operations, Mesoblast, Inc.

“SUCCESSSES AND LESSONS LEARNED IN MANAGING CMOS GLOBALLY”

1. Startup of a New Facility
2. Technology Transfer and Comparability
3. Harmonizing Quality Systems

EJ Read, MD

Head of Product Development, Fate Therapeutics, Inc.

“ON-SITE MANUFACTURING OF A PATIENT-SPECIFIC ALLOGENEIC CELL THERAPY PRODUCT”

1. Design and Standardization of a Manufacturing Process
2. Challenges of On-Site Manufacturing and Testing

WORKSHOP 16

THE PERSPECTIVES OF ACADEMIA AND INDUSTRY IN CELLULAR THERAPY RESEARCH

Chair: Linda Miller, MPA

Foundation for the Accreditation of Cellular Therapy

This session, sponsored by the FACT Regenerative Medicine Task Force and ISCT Commercialization Committee, will differentiate studies performed in academia without industry sponsorship and industry-sponsored trials. The speaker will describe the viewpoints of academia and industry in relation to cellular therapy research. The audience will be invited to share challenges they have faced and offer suggestions for enhancing the industry-academic partnership to design clinical trials that will ultimately result in regulatory approval and market acceptance.

Ian McNiece, PhD

MD Anderson Cancer Center

“DIFFERENCES BETWEEN ACADEMIC AND INDUSTRY CELLULAR THERAPY RESEARCH”

1. Products developed by industry via the standard drug development model
2. Products developed in academia as standard of care
3. Viewpoints of industry and academia in relation to clinical trial partnerships

TECHNICAL SESSION 1

THE WHO, WHAT, WHERE, WHEN, WHY AND HOW OF FDA DEVIATION REPORTING

Chair: Karen Snow, (ASCP)BB, CQA(ASQ)

Quality Assurance Officer Bone Marrow Transplant Program, Massachusetts General Hospital

Determining what is reportable to the FDA as an HCT/P deviation is often a difficult and confusing task. Working together two experts in the field will explore HCT/P deviations reported to FDA: the who, what, where, when why and how of reporting. Audience participation and questions are encouraged during the session.

Fran Rabe, MS, CQM(ASQ)

Quality Manager, Director of Quality Assurance, University of Minnesota Molecular and Cellular Therapeutics

Sharon O'Callaghan, MT (ASCP)

Consumer Safety Officer, Program Surveillance Branch, FDA/Center for Biologics Evaluation and Research, Office of Compliance and Biologics Quality

Learning Objectives:

1. Discuss the who, what, where, when, why, & how of deviation reporting to FDA using an audience participation format
2. Identify events reportable to the FDA as HCT/P Deviations
3. Explore FDA reasoning for what is reportable and what is not

TECHNICAL SESSION 2

TRANSPORTATION LOGISTICS FOR CELL THERAPY PRODUCTS

Chair: Janice Davis-Sproul, MAS, MT(ASCP) SBB

Johns Hopkins Medicine

There can be challenges related to the shipment of cell therapy products, especially across international borders. This session will identify issues with temperature-sensitive samples and products and discuss practices which increase efficiency. The speakers will discuss technical (validation of shipping containers) and quality/regulatory issues.

Doug Padley, MT(ASCP)

Assistant Professor, Laboratory Medicine and Pathology, Development Coordinator, Human Cellular Therapy Laboratory, Mayo Clinic

“TEMPERATURE MONITORING AND TRACEABILITY OF CELLULAR THERAPY PRODUCTS DURING SHIPMENT: EXPERIENCES FROM A CELLULAR THERAPY LABORATORY”

Objectives:

1. Provide one laboratory's experience with use and validation of shipping containers for various transport conditions
2. Describe transportation logistics successes and challenges observed from the laboratory perspective

Daniel O'Donnell,

Director Cell Therapy Logistics, Fisher BioServices

“COLD-CHAIN AND LOGISTICAL CHALLENGES OF CELL THERAPEUTICS IN CLINICAL TRIALS”

Objectives:

1. Understanding the challenges presented in moving high value material at ultra cold temperatures
2. Developing effective logistic strategies to overcome those challenges
3. Creating a Chain-of-Custody that documents; temperature, location and security of the material at all times and at all points along the logistics chain

TECHNICAL SESSION 3

MOVIN' ON UP: DESIGNING, COMMISSIONING & QUALIFYING YOUR PIECE OF THE PIE

Chair: Lynn O'Donnell, PhD

The Ohio State University, Comprehensive Cancer Center

It seems like everyone is building, expanding, renovating or just longing for larger and better facilities for cell therapy manufacturing. The burgeoning regenerative medicine applications of cellular therapies, need for cord blood processing facility improvement for licensure, and continuing robust hematopoietic progenitor cell transplant programs have led to a shortage of adequate space and an abundance of projects requiring project managers, engineers, and consultants with the necessary expertise. This session will explore trends in cell therapy facility projects and what it takes to get it done right.



Lynn O'Donnell, PhD
The Ohio State University

“CELL THERAPY FACILITY EXPANSION AND RENOVATION FROM THE END USER PERSPECTIVE”

1. Review results of peer group polling of cell therapy facility expansion and renovation projects
2. Watch facility video tours and learn where other such resources will be available through ISCT
3. Hear about one center's experiences with a renovation project and a new facility build

Kurt Last, CEO
Specialty Operations Solutions, Inc.

“COMMISSIONING & QUALIFICATION CHALLENGES ON REGULATED MANUFACTURING FACILITIES PROJECTS”

1. Gain insight into the processes of facility construction, commissioning, classification and qualification
2. Learn about potential pitfalls and areas for concern and possible ways to mitigate them

TECHNICAL SESSION 4

NOVEL PUBLISHED GUIDELINES FROM ISCT MSC COMMITTEE

Chair: Donald G. Phinney, PhD

The ISCT MSC committee has recently published a series of reviews and guidance papers addressing issues germane to MSC biology and defining functional endpoints relevant to their use as cell therapy products. The speakers will give an overview of these position papers and thereafter animate a discussion germane to defining a modernized functional metric of MSC for clinical use.

Donald G. Phinney, PhD

“BIOLOGICAL CHARACTERIZATION OF MULTI-POTENT MESENCHYMAL STROMAL CELLS – GUIDELINES FOR CLINICAL TRIALS.

1. Discuss criteria to consider when manufacturing MSCs for clinical therapies
2. Describe relationship between progenitor and non-progenitor functions of MSCs.
3. Illustrate how culture conditions influence MSC potency

Jacques Galipeau, MD FRCP(C)

“IMMUNOLOGICAL CHARACTERIZATION OF MULTI-POTENT MESENCHYMAL STROMAL CELLS-THE INTERNATIONAL SOCIETY FOR CELLULAR THERAPY (ISCT) WORKING PROPOSAL.”

1. Discuss the use of MSCs as immune suppressive pharmaceutical agents.
2. Describe mechanisms that confer immuno-modulatory activity onto MSCs.
3. Discuss a proposal to develop surrogate functional metrics of immune suppressive potency based on immune plasticity assays.

TECHNICAL SESSION 5

REIMBURSEMENT 101

Richard T. Maziarz, MD

Leader, Reimbursement Subcommittee, ISCT; Adult Blood & Stem Cell Transplantation Director, Oregon Health & Science University, Portland, OR

Overall Objectives:

- Understand financial reimbursement landscape within the USA
- Appreciate the complexity of CMS based funding of cellular therapies
- Gain perspectives of the critical role that coding plays on health care reimbursement

Richard T. Maziarz, MD

Leader, Reimbursement Subcommittee, ISCT; Adult Blood & Stem Cell Transplantation Director, Oregon Health & Science University, Portland, OR

“PERSPECTIVES ON REIMBURSEMENT – A BIRD'S EYE VIEW”

1. Review changing population demographics and evolving care delivery systems and impact on health care costs
2. Review differences between governmental and private payer reimbursement for cell therapy

Joel V. Brill, MD, AGAF, CHCQM
Predictive Health LLC, Paradise Valley, AZ

“CODING, COVERAGE AND REIMBURSEMENT CHALLENGES: IMPACT ON STEM CELL THERAPIES”

1. Understand impact of coding on coverage and reimbursement for emerging therapies
2. Understand pathways towards obtaining CPT/HCPCS codes
3. Understand how data collection around outcomes can drive adoption of emerging therapies

Robert Wanerman,

Partner, Epstein, Becker and Green Law, Inc., Washington, DC

“IF YOU BUILD IT, WILL THEY BUY IT?”

1. To understand administration and regulatory processes to achieve coverage, coding and reimbursement
2. To assess strategies to achieve optimal reimbursement for cellular therapies

TECHNICAL SESSION 6

FACT ACCREDITATION OF MINIMAL AND MORE THAN MINIMAL MANIPULATION OF RESEARCH PRODUCTS

Chair: Linda Miller, MPA

Foundation for the Accreditation of Cellular Therapy

This session will discuss why a facility may wish to pursue accreditation for research products, including more than minimal manipulation activities. The speaker will discuss this new accreditation opportunity from the perspective of facilities who have already achieved FACT accreditation of their processing activities for research products.

Deborah Griffin, MSc

University of Pittsburgh Cancer Institute Immunologic Monitoring and Cellular Therapy Products Laboratory

“RAISING THE STANDARD WITH FACT ACCREDITATION: FACILITIES’ EXPERIENCES MEETING MINIMUM STANDARDS FOR CELLULAR THERAPY RESEARCH”

1. Explain why FACT is offering its services to research facilities
2. Discuss why accreditation helps facilities
3. Describe how a facility may obtain funding for accreditation

QUALITY TOOLBOX SESSION 1

RISK ASSESSMENTS AND MITIGATION

Chair: Olive J Sturtevant, MHP, MT(ASCP)SBB, SLS, CQA(ASQ)

Dana-Farber Cancer Institute, Director of Cellular Therapies Quality Assurance Department

This session will look at risk assessment strategies and how to lower the potential for actual exposure to perceived risks in three areas common to most cellular therapy labs; materials and equipment, technical process and risk assessment of software. The speakers will present tools for assessment, mitigation and performing on-going effectiveness checks.

Olive J Sturtevant, MHP, MT(ASCP)SBB, SLS, CQA(ASQ)

Dana-Farber Cancer Institute, Director of Cellular Therapies Quality Assurance Department

“OVERVIEW OF RISK MANAGEMENT ASSESSMENTS AND STRATEGIES USED IN CELLULAR THERAPY APPLICATIONS TO GATHER DATA AND MITIGATE RISKS”

1. Review various risk assessment methods commonly used cell therapy (CT) laboratories
2. Discuss some of the challenges found in CT and misconceptions regarding our ability to impact overall risk reduction strategies
3. Methods used to measure and reduce risk from common CT processes

Karen Snow, (ASCP)BB, CQA(ASQ)

Massachusetts General Hospital, Quality Assurance Officer, Bone Marrow Transplant Program

“ELECTRONIC SYSTEMS: SOLVING THE PROBLEM BEFORE IT HAPPENS”

1. Review standards and requirements for electronic systems
2. Describe ways to mitigate risk before problems occur
3. Present real life examples of risk, consequences and mitigation strategy

Angela Ondo, MT (ASCP)

Johns Hopkins Hospital BMT/CTL QA Manager

“RISK ASSESSMENT OF NEW AND REVISED PROCESSES IN THE CELL THERAPY LAB”

1. Identify and manage risks associated with a new or revised process
2. Tips and strategies for an effective risk management in the lab
3. Use a risk assessment tool in the evaluation of a cell therapy procedure

QUALITY TOOLBOX SESSION 2

MANAGING DEVIATIONS

Aisha Khan, PhD

University of Miami School of Medicine

William Janssen, PhD

H. Lee Moffitt Cancer Center

This session will focus on management of deviations and non-conformances that may occur in the cell therapies production facility. Specific areas to be covered include:

- What is a deviation? What is a non-conformance?



- How can you know when a deviation has occurred?
- How should a deviation be documented?
- What types of follow-up should occur after a deviation has been detected?
- Are there different types / levels of deviation?
- Do all deviations have to be reported to outside authorities?
- Are there tried and true ways to reduce the risk of future deviations?

There will be “case studies” that the presenters will use to illustrate. Participants are invited to bring their own examples for analysis and discussion.

QUALITY TOOLBOX SESSION 3

TECHNICAL WRITING LED BY THE CO-EDITORS OF THE TELEGRAFF

Deborah L. Griffin *MSc*
University of Pittsburgh Cancer Institute

Lynn O'Donnell *PhD*
Ohio State University, James Cancer Hospital

Clear, concise language is essential to communicate information. Whether that message is in the form of a scientific paper or a laboratory report, the right words will disseminate information effectively. This practical session aims to assist a writer in identifying possible areas of confusing text and provide resources and assistance with word choice, tone and format.

1. Identify areas of laboratory writing that could be stronger (SOPs, Quality Plans, Forms, Reports, Deviation Management Investigations)
2. Provide examples of confusing or poorly written language
3. Demonstrate use of effective language.

PATIENT ADVISORY STATEMENT Q&A SESSION

MONDAY, SEPTEMBER 9TH
12:15 PM – 1:15 PM, COLUMBUS B

William Janssen, *PhD*
H. Lee Moffitt Cancer Center
Karen Nichols, *Esq, RAC*
OvaScience

All attendees welcome. This session will provide an overview of how and why the statement was developed, what the intended purpose is, and how the Society and other cell therapy stakeholders can get involved.

A copy of the Patient Advisory Statement is available in your delegate bag. Please visit the Registration Desk if you need an additional copy.

REGULATORY UPDATES FROM THE JAPANESE PMDA (PHARMACEUTICALS AND MEDICAL DEVICES AGENCY)

TUESDAY, SEPTEMBER 10TH
12:15 PM – 1:15 PM, COLUMBUS A

Chair: Akihiro Shimosaka, *PhD*
Research Foundation for Community Medicine
Akihiro Shimosaka, *PhD*
Research Foundation for Community Medicine

CELLULAR THERAPY SITUATION IN EASTERN ASIA
(25 MIN. + 5 MIN. DISCUSSION)

Daiju Okuda, *PhD*
Japanese PMDA

**JAPANESE ADVANCED THERAPY PASS WAY AND NEW
REGULATION FOR CELLULAR THERAPY**
(25 MIN. + 5 MIN. DISCUSSION)