THE ISCT-ASTCT CELL THERAPY TRAINING COURSE IS SPONSORED IN PART BY THE FOLLOWING ORGANIZATIONS:

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ISCT
Cell & Gene Therapy®

ASTCT
American Society for Transplantation and Cellular Therapy

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Penn Medicine

Full Scholarship Funding Provided By:

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PLANNING FACULTY

Co–Chairs

David DiGiusto, PhD
Chief Technical Officer
Semma Therapeutics
Cambridge, MA, USA

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Director, Stem Cell Transplantation and Cellular Therapy Program
Editor-in-Chief, British Journal of Haematology
George Washington University
Washington, DC, USA

Planning Committee

Catherine Bollard, MD, MBChB
Professor of Pediatrics
Director, Center for Cancer and Immunology Research (CCIR)
Director, Program for Cell Enhancement and Technologies for Immunotherapy (CETI)
Children’s National Health System
The George Washington University
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Executive Vice President of Research and Development
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Director, Cord Blood Transplant Program
Professor, University of Washington
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Department of Pathology and Laboratory Medicine Center for Cellular Immunotherapies Perelman School of Medicine and Abramson Cancer Center University of Pennsylvania Philadelphia, PA, USA

Krishna V. Komanduri, MD
Professor of Medicine, Microbiology & Immunology
Director, Adult Stem Cell Transplant Program
Associate Director for Clinical Innovation
Sylvester Comprehensive Cancer Center
University of Miami Miller School of Medicine
Miami, FL, USA
COURSE FACULTY

Session Speakers

Usman “Oz” Azam, MD
President & CEO
Tmunity Therapeutics
Philadelphia, PA, USA

Veronika Bachanova, MD, PhD
Associate Professor of Medicine
Division of Hematology, Oncology and Transplantation
University of Minnesota
Minneapolis, MN, USA

Joseph A. Fraietta, PhD
Assistant Professor of Microbiology
Director, Solid Tumor Immunotherapy Laboratory
Center for Advanced Cellular Therapies
University of Pennsylvania Perelman School of Medicine
Philadelphia, PA, USA

Philip J. Cross, MS
President and Principal Consultant
Philip J. Cross & Associates, Inc.
Harbeson, DE, USA

Anna Gilbert, ASQ CQA
Director, Industry Specialty Services
BDO USA
Seattle, WA, USA

Noelle Frey, MD, MSCE
Associate Professor of Medicine
Director of Clinical Cellular Therapy,
Cell and Transplant Therapy Program
University of Pennsylvania Perelman School of Medicine
Philadelphia, PA, USA

Elizabeth Hexner, MD, MSTR
Associate Professor of Medicine
Medical Director, Center for Cellular Immunotherapies
Division of Hematology/Oncology
Abramson Cancer Center
University of Pennsylvania
Philadelphia, PA, USA

Whitney Gladney, PhD
Associate Director, Translational Sciences Operations
Center for Cellular Immunotherapies
University of Pennsylvania Perelman School of Medicine
Philadelphia, PA, USA

Megan Kasimatis Singleton, JD, MBE, CIP
Assistant Dean, Human Research Protections
Director of the Human Research Protections Program
Office of Human Subjects Research
Johns Hopkins University School of Medicine
Baltimore, MD, USA

Wei-Ting Hwang, PhD
Professor of Biostatistics
Department of Biostatistics, Epidemiology and Informatics (DBEI)
University of Pennsylvania
Philadelphia, PA, USA

Peter Marks, MD, PhD
Director, Center for Biologies Evaluation and Research (CBER)
U.S. Food & Drug Administration
Silver Spring, MD, USA

Lester Lledo, MSN, CRNP
Director, CCI-Clinical Trials Unit
Penn Medicine, Center for Cellular Immunotherapies
University of Pennsylvania
Philadelphia, PA, USA

Jos Melenhorst, PhD
Adjunct Associate Professor
Pathology & Laboratory Medicine
University of Pennsylvania
Philadelphia, PA, USA

Shannon Maude, MD, PhD
Assistant Professor of Pediatrics, Division of Oncology
The Children’s Hospital of Philadelphia
Medical Director, Center for Cellular Immunotherapies
University of Pennsylvania Perelman School of Medicine
Philadelphia, PA, USA

Donald M. O’Rourke, MD
John Templeton, Jr. M.D. Professor in Neurosurgery
Director, Glioblastoma Translational Center of Excellence (TCE)
The Abramson Cancer Center and Perelman School of Medicine
The University of Pennsylvania, PA, USA

Doug Olson, PhD
Chief Executive Officer
BUHLMANN Diagnostics Corp.
Cancer survivor and patient number two in the initial CART 19 clinical trial
Boston, MA, USA

Johannes van der Loo, PhD
Director, Clinical Vector Core
Center for Cellular & Molecular Therapeutics
The Children’s Hospital of Philadelphia
Philadelphia, PA, USA

Elizabeth J. Shpall, MD
Howard and Lee Smith Chair in Cancer Research
Director, Cell Therapy Laboratories and Cord Blood Bank
Deputy Chair, Department of Stem Cell Transplantation and Cellular Therapy
The University of Texas MD Anderson Cancer Center
Houston, TX, USA
THE SCHOLARS

International Scholars

Gabor Foldes, MD, PhD
Imperial College London
London, United Kingdom
Mentor: Sian Harding
ISCT Scholar

Andrea Henden, MBBS (Hons), FRACP, FRCPA
QIMR Berghofer, Medical Research Institute
Brisbane, Australia
Mentor: Siok Tey
Emily Whitehead Foundation Scholar

Karin Wisskirchen, PhD
Helmholtz Zentrum München
Munich, Germany
Mentor: Martin Hildebrand
ISCT Scholar

Giulia Golinelli, PhD
University Hospital of Modena and Reggio Emilia
Modena, Italy
Mentor: Massimo Dominici
ISCT Scholar

Gaurav Sutrave, BSc(Med)/MBBS (Hons I), FRACP,
FRCPA
Westmead Hospital
Sydney, Australia
Mentor: David Gottlieb
Fresenius Kabi Scholar

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Westmead Hospital
Sydney, Australia
Mentor: David Gottlieb
Fresenius Kabi Scholar

North American Scholars

Saurabh Dahiya, MBBS
University of Maryland
Baltimore, MD, USA
Mentor: Aaron Rapoport
ASTCT Scholar

Shabnum Patel, PhD
Stanford University
Palo Alto, CA, USA
Mentors: Steven A. Feldman, Crystal L. Mackall,
David Milios
Kite, a Gilead Company Scholar

Frederico Simonetta, MD, PhD
Stanford University
Palo Alto, CA, USA
Mentor: Robert S. Negrin
ASTCT Scholar

Shoba A. Navai, MD
Baylor College of Medicine
Houston, TX, USA
Mentor: Meenakshi Hedge
Novartis Scholar

Irene Scarfo, PhD
Massachusetts General Hospital
Boston, MA, USA
Mentor: Marcela Maus
Legend Biotech Scholar

Host Institutional Scholars

Mauro Castellarin, PhD
University of Pennsylvania
Philadelphia, PA, USA
Mentor: Carl June
University of Pennsylvania Scholar

Philipp C Rommel, Dr. rer.nat.
University of Pennsylvania
Philadelphia, PA, USA
Mentor: Carl June
University of Pennsylvania Scholar

Saba Ghassemi, PhD
University of Pennsylvania
Philadelphia, PA, USA
Mentor: Michael C. Milone
University of Pennsylvania Scholar

Antonia Rotolo, MD, PhD
University of Pennsylvania
Philadelphia, PA, USA
Mentor: Marco Ruella, Carl June
University of Pennsylvania Scholar
## Program

### CO-CHAIRS

John Barrett, MD, George Washington University, Washington, DC  
David DiGiusto, PhD, Semma Therapeutics, Cambridge, MA

### MONDAY, OCTOBER 21, 2019

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<td>08:00 – 08:15</td>
<td>Introduction by Course Co-Chairs</td>
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<td>08:15 – 10:00</td>
<td>Scholar Presentations</td>
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<td>10:00 – 10:15</td>
<td>Coffee Break</td>
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<td>10:15 – 12:00</td>
<td>Scholar Presentations</td>
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<td>12:00 – 13:00</td>
<td>Lunch</td>
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<td>13:00 – 14:00</td>
<td>Scholar Presentations</td>
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<tr>
<td>14:00 – 15:00</td>
<td><strong>Study Design, Conduct and Analysis of Clinical Trials</strong></td>
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<td><strong>LEARNING OBJECTIVES:</strong></td>
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<td>1. Confirmatory &amp; Exploratory Analyses</td>
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<td>2. Choosing manufacture and product release criteria</td>
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<td>3. Biomarkers &amp; Surrogate Endpoints</td>
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<td>4. Stopping rules</td>
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<td>5. Safety considerations</td>
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<td>6. Including replacements</td>
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<td>7. Addressing Missing Data in Clinical Trials</td>
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<td>8. To review specific trial design issues from scholar presentations</td>
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<td><strong>SESSION LEAD:</strong></td>
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<td><strong>John Barrett, MD, George Washington University, Washington, DC</strong></td>
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<td><strong>SPEAKERS:</strong></td>
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<td>Noelle Frey, MD, MSCE, University of Pennsylvania, Philadelphia, PA</td>
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<td>Shannon Maude, MD, PhD, The Children’s Hospital of Philadelphia, PA</td>
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<td>15:00 – 16:00</td>
<td><strong>Statistics</strong></td>
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<td>Dr. Hwang will present on critical statistical approaches for efficacious analysis of early-phase studies and will engage with Scholars to review specific statistical issues associated with their respective projects.</td>
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<td><strong>LEARNING OBJECTIVES:</strong></td>
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<tr>
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<td>1. Modern designs for Phase I dose finding trial beyond 3+3 design</td>
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<td>2. Design considerations for expansion cohort and Phase II trial including early stopping rule</td>
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<td>3. Sample size considerations for safety and efficacy endpoints</td>
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<td>4. Common statistical Methods for correlative and translational endpoints</td>
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<td>5. Reporting guideline, results interpretation and presentations</td>
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<td><strong>John Barrett, MD, George Washington University, Washington, DC</strong></td>
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<td><strong>SPEAKER:</strong></td>
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<td>Statistical Considerations for Early-Phase Clinical Trials of Cell Therapies</td>
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<td>Wei-Ting Hwang, PhD, University of Pennsylvania, Philadelphia, PA</td>
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</tbody>
</table>
MONDAY, OCTOBER 21, 2019

16:00 – 17:00 Ethics Roundtable
This will be a highly interactive session, providing content and building discussion around key ethical considerations in the translation of cell & gene therapies.

LEARNING OBJECTIVES:
- To understand current ethical principles governing human clinical research
- To understand the process of ethical review and oversight governing human clinical research
- To understand unique ethical issues for human cell & gene therapy products
- To understand the challenges and risks posed by widespread use of unproven cellular therapies
- To review scholar projects for ethical issues and discuss management of ethical questions and risks

SESSION LEAD:
John Barrett, MD, George Washington University, Washington, DC

PANELISTS:
Megan Kasimatis Singleton, JD, MBE, CIP, Johns Hopkins University, Baltimore, MD
John Barrett, MD, George Washington University, Washington, DC

TUESDAY, OCTOBER 22, 2019

08:00 – 10:00 Quality Assurance Development Lectures
This section will introduce the regulations, guidance, and make recommendations for establishing Quality System elements consistent with the needs for early phase clinical studies.

LEARNING OBJECTIVES:
- Understanding what constitutes a Quality System
- Understanding the role Quality System plays in product development
- Understanding compliance requirements for cell therapy products
- Developing Quality Systems appropriate to the stage of the project

SESSION LEAD:
David DiGiusto, PhD, Semma Therapeutics, Cambridge, MA

SPEAKERS:
Quality – Benchtop to Bedside and Beyond
Philip J. Cross, MS, Philip J. Cross & Associates, Inc., Harbeson, DE

Quality Systems: Requirements for Virtual Companies
Anna Gilbert, ASQ CQA, BDO USA, Seattle, WA

10:00 – 10:15 Coffee Break
TUESDAY, OCTOBER 22, 2019

10:15 – 12:00  IND Development Lectures
This section will cover the format, topics, inclusions, and strategies for filing an Investigational New Drug (IND), with focus on the non-clinical and clinical data expectations required by the eCTD format. The session will also include discussion of translating the early phase R&D product into GMP including development of specifications pertinent to the Chemistry, Manufacturing and Controls (CMC) of the IND.

LEARNING OBJECTIVES:
- Learn the components of the IND submission, the clinical research protocol and the related and required monitoring plans
- Understand the components of the CMC and how to develop and present those components for inclusion in the IND
- Recognize the importance of gathering appropriate previous human and preclinical data to support the IND
- Recognize the importance of working closely with the GMP lab to design SOPs for product generation
- Learn the CTD/eCTD modular format and be able to identify nuances and differences of the format as compared to the FDA IND format

SESSION LEAD:
Bruce Levine, PhD, University of Pennsylvania, Philadelphia, PA

SPEAKERS:
Elizabeth Hexner, MD, MSTR, University of Pennsylvania, Philadelphia, PA
Whitney Gladney, PhD, University of Pennsylvania, Philadelphia, PA
Elizabeth J. Shpall, MD, MD Anderson Cancer Center, Houston, TX

12:00 – 13:00 Lunch

13:00 – 14:00  Regulatory Affairs Case Studies
This Regulatory Affairs Case Studies discussion will focus on real world experiences in developing and interpreting the regulatory requirements for cell and gene therapy. This session will be interactive, and the scholars will have extensive opportunity to ask questions of the faculty with respect to their specific project proposals.

LEARNING OBJECTIVES:
- Planning and preparing for Pre-IND/scientific guidance meetings
- Non-clinical efficacy, safety & toxicology – challenges for Advanced Therapies
- Product characterization & potency
- Comparability of non-clinical material with clinical material
- Comparability for process and product changes
- Tips for study report and submission compilation and writing

SESSION LEAD:
Catherine Bollard, MBChB, MD, Children’s National Health System, Washington, DC

SPEAKER:
Peter Marks, MD, PhD, U.S. Food & Drug Administration, Silver Spring, MD

14:00 – 15:00  Breakout Session: Career Development
This panel session will feature multiple faculty members and will highlight the many career path options in the cell and gene therapy space. The faculty will describe critical decisions that they have made throughout their career development, what transitions they have made within their careers, lessons learned and advice on how to positively influence your career path forward. This session will be interactive, and the scholars will have extensive opportunity to ask questions of the faculty with respect to career development.

LEARNING OBJECTIVES:
- Provide examples of career paths for Clinician Scientists
- Provide examples of career paths for Non-Clinician Scientists

SESSION LEAD:
Colleen Delaney, MD, MSc, Nohla Therapeutics, Seattle, WA

SPEAKERS:
Peter Marks, MD, PhD, U.S. Food & Drug Administration, Silver Spring, MD
Krisha Komanduri, MD, University of Miami, Miami, FL
Colleen Delaney, MD, MSc, Nohla Therapeutics, Seattle, WA
Catherine Bollard, MBChB, MD, Children’s National Health System, Washington, DC
**TUESDAY, OCTOBER 22, 2019**

**16:00 – 17:00**  
**Tour of U Penn Cell Processing Facility**  
The Clinical Cell and Vaccine Production Facility (CVPF) renders bench-to-bedside translational medicine a reality. Equipped with state of the art facilities, the CVPF manufactures cell and gene biotherapeutics and has been accredited by the Foundation for the Accreditation of Cellular Therapy (FACT) since 2008. As a shared resource within the Center for Cellular Immunotherapies (CCI) and the Division of Transfusion Medicine & Therapeutic Pathology within the Department of Pathology & Laboratory Medicine, the CVPF supports numerous investigational new drug (IND) protocols in adult and pediatric cancers, HIV, and stroke.

**LEARNING OBJECTIVES:**  
- Design and operation of a multi-product Clinical Cell and Vaccine Production Facility  
- Understand logistics and basic infrastructure of a cGMP facility, including personnel and supply access flow, suite preparation, environmental monitoring, air quality classification and handling, cleaning and sanitation.  
- Lessons learned from years of manufacturing

**17:00 – 18:00**  
**Networking Reception with Faculty**  
*Sponsored by Semma Therapeutics*

**WEDNESDAY, OCTOBER 23, 2019**

**08:00 – 09:45**  
**Manufacturing and Release Testing Cells Part I: Non-gene Modified**  
These lectures will cover basic principles and practices for the manufacturing and release testing of non-genetically modified cell and gene therapy products according to Good Manufacturing Practices. Examples from instructor experiences will be used to provide context for interpretation of federal regulations.

**LEARNING OBJECTIVES:**  
- Principles of GMP as applied to manufacturing operations of non-gene modified cells  
- Therapeutic applications of non-gene modified cells  
- Raw materials specifications and vendor selection  
- Working with primary cells from patients and patient specific product manufacturing  
- Open vs closed systems for manufacturing  
- Application of automation and product manufacturing  
- In process testing and setting metrics for success  
- Potency assays

**SESSION LEAD:**  
David DiGiusto, PhD, Semma Therapeutics, Cambridge, MA

**SPEAKERS:**  
Catherine Bollard, MBChB, MD, Children’s National Health System, Washington, DC  
John Barrett, MD, George Washington University, Washington, DC  
David DiGiusto, PhD, Semma Therapeutics, Cambridge, MA

**09:45 – 10:00**  
**Coffee Break**
### WEDNESDAY, OCTOBER 23, 2019

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<tr>
<th>Time</th>
<th>Session Title</th>
<th>Description</th>
<th>Learning Objectives</th>
<th>Session Lead</th>
<th>Speakers</th>
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</thead>
</table>
| 10:00 – 11:00 | **Manufacturing and Release Testing Cells Part II: Genetically Modified** | These lectures will cover basic principles and practices for the manufacturing and release testing of gene modified cell & gene therapy products according to Good Manufacturing Practices. Examples from instructor experiences will be used to provide context for interpretation of federal regulations. | - Principles of GMP as applied to manufacturing operations of genetically modified cells  
- Raw materials specifications and vendor selection  
- Working with primary cells from patients and patient specific product manufacturing  
- Open vs closed systems for manufacturing  
- Application of automation and product manufacturing  
- In process testing and setting metrics for success  
- Developing and qualifying appropriate release tests (compendial vs product specific) | Bruce Levine, PhD, University of Pennsylvania, Philadelphia, PA | Bruce Levine, PhD, University of Pennsylvania, Philadelphia, PA  
David DiGiusto, PhD, Semma Therapeutics, Cambridge, MA |
| 11:00 – 12:00 | **Manufacturing and Analytics of Viral Vectors** | This session will highlight current challenges associated with the manufacturing of viral vector products. Moreover, Dr. van der Loo will describe the regulations and standards that govern the production and release testing requirements for viral vectors intended for ex vivo or in vivo clinical use. | - To understand the basic differences between types of viral vectors, manufacturing methods, and applications  
- To understand issues associated with the manufacturing of viral vector products, including scale and lot-to-lot variability  
- To understand and appreciate facilities and equipment needed to support GMP manufacturing  
- To understand the basic regulatory requirements governing the manufacture and release of viral vector products  
- To understand the value of adopting a risk-based approach in GMP manufacturing | David DiGiusto, PhD, Semma Therapeutics, Cambridge, MA | Johannes van der Loo, PhD, The Children’s Hospital of Philadelphia, PA |
| 12:00 – 13:00 | Lunch |                                                                                              |                                                                                                                                                    |                                 |                                                                        |
| 13:00 – 14:00 | **Routes to Commercialization** | This session will focus on transition of cell & gene therapy projects from an academic setting into industry and address associated challenges and key considerations for commercialization. Personal experiences, strategies and lessons learned in making this often-difficult transition will be shared. | - To understand the product development life cycle and drivers of commercialization of cell and gene therapy products  
- To understand the drivers underlying the timing of transition from academic to commercial development, as a startup company or via licensing of technology to an existing company  
- Regulatory compliance for commercialization  
- To understand funding sources | Colleen Delaney, MD, MSc, Nohla Therapeutics, Seattle, WA | Colleen Delaney, MD, MSc, Nohla Therapeutics, Seattle, WA  
Usman “Oz” Azam, MD, Tmunity Therapeutics, Philadelphia, PA |
**WEDNESDAY, OCTOBER 23, 2019**

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
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<tbody>
<tr>
<td>14:00 – 16:00</td>
<td><strong>Pre-Clinical Development</strong>&lt;br&gt;In this session speakers will address the hurdles to translating a variety of cell therapy products with a focus on manufacturing considerations and particular issues related to the translation of T cells, NK cells, MSCs and islets.&lt;br&gt;&lt;br&gt;<strong>LEARNING OBJECTIVES:</strong>&lt;br&gt;1. To learn the challenges that have to be overcome to translate from the idea to the reality of cell product manufacture for the clinic&lt;br&gt;2. To learn the history behind the current manufacture of specific cell therapies&lt;br&gt;3. To understand the particular problems that had to be overcome for specific cell products (Stem cells, NK cells MSC and T cells)&lt;br&gt;&lt;br&gt;<strong>SESSION LEAD:</strong>&lt;br&gt;John Barrett, MD, George Washington University, Washington, DC&lt;br&gt;&lt;br&gt;<strong>SPEAKERS:</strong>&lt;br&gt;General Overview of CAR-T Cell Pre-Clinical Development&lt;br&gt;Gabriela Plesa, MD, PhD, University of Pennsylvania, Philadelphia, PA&lt;br&gt;Development of Natural Killer Cellular Products for Cancer Therapy&lt;br&gt;Veronika Bachanova, MD, PhD, University of Minnesota, Minneapolis, MN&lt;br&gt;Pre-Clinical Development of ES-Derived Pancreatic Islets&lt;br&gt;David DiGiusto, PhD, Semma Therapeutics, Cambridge, MA&lt;br&gt;Lessons Learned from Setting up an MSC Trial&lt;br&gt;John Barrett, MD, George Washington University, Washington, DC</td>
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<tr>
<td>16:00 – 17:00</td>
<td><strong>Clinical Trial Case Studies: Delivery of Cell Therapy Products (Infusion)</strong>&lt;br&gt;This session will focus on the nuances and issues related to delivering various cell and gene therapy products to patients including shipping, storage, infusion, managing product related adverse events and reporting.&lt;br&gt;&lt;br&gt;<strong>LEARNING OBJECTIVES:</strong>&lt;br&gt;1. To understand the issues related to shipping cell and gene therapy products – Manufacturing Issue&lt;br&gt;2. To understand the issues related to storage of cell and gene therapy products- Manufacturing Issue&lt;br&gt;3. To understand the issues related to infusing of cell and gene therapy products, monitoring and reporting product-related adverse events&lt;br&gt;4. To understand the issues related to managing product-related adverse events&lt;br&gt;&lt;br&gt;<strong>SESSION LEAD:</strong>&lt;br&gt;Krishna Komanduri, MD, University of Miami, Miami, FL&lt;br&gt;&lt;br&gt;<strong>SPEAKERS:</strong>&lt;br&gt;Cell Product Administration: Operational and Logistical Considerations&lt;br&gt;Lester Lledo, MSN, CRNP, University of Pennsylvania, Philadelphia, PA&lt;br&gt;CAR-T Cell Therapy for Glioblastoma: Current Observations and Future Directions&lt;br&gt;Donald O’Rourke, MD, University of Pennsylvania, Philadelphia, PA</td>
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<td>17:30 – 20:30</td>
<td><strong>Scholar + Faculty Dinner at Estia Restaurant</strong>&lt;br&gt;Guest Lecture by Doug Olson, PhD, BUHLMANN Diagnostics Corp., Boston, MA, USA&lt;br&gt;Doug is a cancer survivor and patient number two in the initial CART 19 clinical trial</td>
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### THURSDAY, OCTOBER 24, 2019

<table>
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<th>LEARNING OBJECTIVES</th>
<th>SESSION LEAD/SPAKERS</th>
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<tr>
<td>08:00 – 09:45</td>
<td>Correlative Studies Lectures</td>
<td>Lectures will encompass discussion on the importance of immune correlative studies for immune based therapies, along with associated challenges.</td>
<td>Krishna Komanduri, MD, University of Miami, Miami, FL</td>
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<td><strong>LEARNING OBJECTIVES:</strong></td>
<td>To understand the importance of correlative studies in accurately assessing aspects such as</td>
<td>Jos Melenhorst, PhD, University of Pennsylvania, Philadelphia, PA</td>
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<td>- Delivery and efficiency of gene transfer</td>
<td>- Target specificity</td>
<td>Krishna Komanduri, MD, University of Miami, Miami, FL</td>
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<td>- Immunogenicity and toxicity – genotoxicity: insertional mutagenesis</td>
<td>- Long term versus short term expression</td>
<td>Joseph Fraietta, PhD, University of Pennsylvania, Philadelphia, PA</td>
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<td><strong>SESSION LEAD:</strong></td>
<td><strong>SPEAKERS:</strong></td>
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<td>Krishna Komanduri, MD, University of Miami, Miami, FL</td>
<td>Jos Melenhorst, PhD, University of Pennsylvania, Philadelphia, PA</td>
<td>Krishna Komanduri, MD, University of Miami, Miami, FL</td>
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<td>Joseph Fraietta, PhD, University of Pennsylvania, Philadelphia, PA</td>
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<td>9:45 – 10:00</td>
<td>Coffee Break</td>
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<td>10:00 – 11:00</td>
<td>Evolving Financial Models for CAR-T and Gene Therapies</td>
<td>Understanding what goes into the actual cost of delivery of a clinical product, Reimbursement</td>
<td>Krishna Komanduri, MD, University of Miami, Miami, FL</td>
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<td>11:00 – 12:00</td>
<td>Team Science</td>
<td>To understand how to create a research team that addresses all of the requirements for creating, qualifying and delivering a cell therapy product to the clinic, How to motivate and keep the team together during the lengthy translational stages of product development, How to work successfully with scientific journals</td>
<td>John Barrett, MD, George Washington University, Washington, DC</td>
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<td><strong>SESSION LEAD:</strong></td>
<td><strong>SPEAKERS:</strong></td>
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<td><strong>SPEAKERS:</strong></td>
<td>Catherine Bollard, MBChB, MD, Children’s National Health System, Washington, DC</td>
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<td>12:00 – 13:00</td>
<td>Lunch</td>
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THURSDAY, OCTOBER 24, 2019

13:00 – 14:00
Funding Sources
Scholars will participate in a one hour workshop on the techniques and approaches involved in securing research funding.

LEARNING OBJECTIVES:
- Identifying funding sources
- How to write an application
- How to revise an application

SESSION LEAD/SPEAKER:
Catherine Bollard, MBChB, MD, Children’s National Health System, Washington, DC

14:00 – 15:30
Challenges for Gene Modified Cells and Regenerative Medicine Products
Aimed at providing insight into the unique considerations for specific therapeutic modalities, faculty will speak to the challenges faced when developing iPSC, T-cell and regenerative medicine products for clinical use.

LEARNING OBJECTIVES:
- To appreciate the subtleties of gene therapy and regenerative medicine products, their production, testing and use in the clinic
- To understand manufacturing issues related to gene therapy, iPSC and regenerative medicine products
- Consideration of immunogenicity, graft rejection and durability of engineered cell products
- Insight into new technologies that are quickly advancing the field to overcome current challenges

SESSION LEAD:
Bruce Levine, PhD, University of Pennsylvania, Philadelphia, PA

SPEAKERS:
David DiGiusto, PhD, Semma Therapeutics, Cambridge, MA
Bruce Levine, PhD, University of Pennsylvania, Philadelphia, PA
Colleen Delaney, MD, MSc, Nohla Therapeutics, Seattle, WA

15:30 – 17:00
Project Refinement and Presentation Preparation with Faculty Mentorship

FRIDAY, OCTOBER 25, 2019

08:00 – 09:45
Scholar Presentations

09:45 – 10:00
Coffee Break

10:00 – 11:30
Scholar Presentations

11:30 – 12:30
Lunch (Grab-and-Go)
About the Partners

International Society for Cell & Gene Therapy

The International Society for Cell & Gene Therapy (ISCT) is a global society of clinicians, regulators, technologists, and industry partners with a shared vision to translate cell and gene therapy into safe and effective therapies to improve patients’ lives worldwide. ISCT is the leading group focused on pre–clinical and translational aspects of developing cell and gene therapy products, thereby driving the translation of scientific research to innovative cell and gene therapies for patients. As such, ISCT helps academic, government and biotech/pharma sectors advance research into practice and product. ISCT Members are part of an influential global community of peers, experts, and organizations invested in cell and gene therapy, offering a unique collaboration between academia, regulatory bodies, and industry partners in cell and gene therapy translation.

American Society for Transplantation and Cellular Therapy

The American Society for Transplantation and Cellular Therapy (ASTCT), formerly known as the American Society for Blood and Marrow Transplantation, is a professional society of more than 2,200 healthcare professionals and scientists from over 45 countries who are dedicated to improving the application and success of blood and marrow transplantation and related cellular therapies. ASTCT strives to be the leading organization promoting research, education, and clinical practice to deliver the best, comprehensive patient care.

About the Institutional Host

University of Pennsylvania

Penn Medicine

Penn Medicine is one of the world’s leading academic medical centers, dedicated to the related missions of medical education, biomedical research, and excellence in patient care. Penn Medicine consists of the Raymond and Ruth Perelman School of Medicine at the University of Pennsylvania (founded in 1765 as the nation’s first medical school) and the University of Pennsylvania Health System, which together form a $7.8 billion enterprise.

The Perelman School of Medicine has been ranked among the top medical schools in the United States for more than 20 years, according to U.S. News & World Report’s survey of research-oriented medical schools. The School is consistently among the nation’s top recipients of funding from the National Institutes of Health, with $425 million awarded in the 2018 fiscal year.

The University of Pennsylvania Health System’s patient care facilities include: the Hospital of the University of Pennsylvania and Penn Presbyterian Medical Center—which are recognized as one of the nation’s top “Honor Roll” hospitals by U.S. News & World Report—Chester County Hospital; Lancaster General Health; Penn Medicine Princeton Health; and Pennsylvania Hospital, the nation’s first hospital, founded in 1751. Additional facilities and enterprises include Good Shepherd Penn Partners, Penn Home Care and Hospice Services, Lancaster Behavioral Health Hospital, and Princeton House Behavioral Health, among others.

Penn Medicine is powered by a talented and dedicated workforce of more than 40,000 people. The organization also has alliances with top community health systems across both Southeastern Pennsylvania and Southern New Jersey, creating more options for patients no matter where they live.

Penn Medicine is committed to improving lives and health through a variety of community-based programs and activities. In fiscal year 2018, Penn Medicine provided more than $525 million to benefit our community.

Center for Cellular Immunotherapies

The Mission of the Center for Cellular Immunotherapies is to build on Penn’s leadership in Translational Medicine and investigator initiated clinical trials established over the past two decade. CCI is focused on coordinated interdisciplinary approaches for the discovery and development of core platform technologies for personalized cell and gene based therapies in cancer, autoimmune disease, infectious disease, and organ and bone marrow transplantation. CCI interacts with a coalition of investigators in nearly all departments and centers in the Perelman School of Medicine; driving clinical translation of novel and investigational immune-based therapies. CCI will fuse clinical investigators and scientists within the Abramson Cancer Center, the Institute for Immunology, the Center for AIDS research, the Cardiovascular Institute and the Center for Orphan Disease Research and Therapy, and the program in novel biotherapeutics within the Institute of Translational Medicine and Therapeutics. The CCI mission is to accelerate and synergize efforts that quickly transition fundamental immunobiology research into the clinic; bringing value to the University of Pennsylvania and it’s faculty beyond existing department, center, and institutional structures.
About the Full Scholarship Sponsors

Emily Whitehead Foundation
The Emily Whitehead Foundation (EWF) is a nonprofit organization committed to activating the cure for childhood cancer. The foundation was created in honor of Emily Whitehead, the first pediatric patient in the world to receive CAR-T cell therapy. Now, the Emily Whitehead Foundation is dedicated to funding innovative pediatric CAR-T cell therapy research, raising awareness through education and sharing inspiring stories, and providing support and resources for families affected by childhood cancer.

For more information, visit EmilyWhiteheadFoundation.com.

Fresenius Kabi
Fresenius Kabi (www.chooselovo.com) is a global healthcare company that specializes in lifesaving medicines and technologies for infusion, transfusion and clinical nutrition. Brining more than 60 years’ experience in cell collection and separation, our team is focused on serving the needs for the cell and gene therapy industry with the Lovo Cell Processing System. Lovo is the only cell processing system that can wash, concentrate, and volume reduce cells using spinning membrane filtration technology.

Kite, a Gilead Company
Kite, a Gilead company, is dedicated to achieving one of the most ambitious goals of 21st century medicine: curing cancer. This mission is at the heart of everything we do, from early research to product development.

For the past three decades, members of our team have been at the forefront of cancer immunotherapy. Today, we are a leader in engineered T cell therapy, changing the paradigm of cancer treatment with what is potentially the biggest breakthrough since the introduction of combination chemotherapy more than 60 years ago.

With an unrelenting drive and a singular focus on cell therapy, our team is executing on our strategy to bring life-saving therapies to patients.

Legend Biotech
Legend Biotech is a global biopharmaceutical company with more than 500 employees across the United States, China, and Europe.

Dedicated to quality, driven by excellence and focused on the science, our research is centered on continuous innovation in the field of cell therapy to develop quality and impactful innovations for the treatment of hematologic malignancies, solid tumors, autoimmune and infectious diseases with an eye to emerging technologies.

Our goal is to reimagine healthcare and with efficiency, transparency, passion, fearlessness and accountability deliver transformative treatments that bring us closer to a cure and to improving the lives of patients worldwide.

Tmunity Therapeutics
Tmunity Therapeutics is a private clinical stage biotherapeutics company delivering the full potential of next-generation T cell immunotherapy to more patients with devastating diseases. Integrating broad collaborations with the University of Pennsylvania (UPenn) and the University of Minnesota (UMinn) with the groundbreaking scientific, clinical and manufacturing expertise and demonstrated track record of its founders, Tmunity represents a new center of gravity in translational T cell medicine. The company is developing a diversified portfolio of novel treatments that exhibit best-in-class control over T cell activation and direction in the body. These personalized next-generation immunotherapies for cancer, infectious disease and autoimmune disease are advancing rapidly toward the clinic. With headquarters in Philadelphia, Tmunity utilizes laboratories and production facilities at UPenn and its own dedicated cGMP manufacturing facility in East Norriton PA, to pursue process improvement and production scale-up in support of clinical development of T cell therapies.

Novartis
At Novartis, we are reimagining medicine in an effort to produce breakthroughs and address major unmet needs for patients with devastating diseases, including genetic disorders and certain deadly cancers. These efforts are done in collaboration with scientists, physicians, academia, industry partners, and patients.

In 2017, Novartis received approval of the first CAR-T cell therapy which is both a cell therapy and a gene therapy. Currently, there are three key focus areas for research and development of potentially transformative cell and gene therapies at Novartis: AAV-based therapies, CAR-T cell therapies and CRISPR-based technologies.
Achievement Award on him in 2019. International Society for Cellular Therapy which bestowed a Career achievement Award in 2018. He is a member of the American Blood and Marrow Transplantation Group and the American Society for Hematology. He has served as President both of the European Society for Cytotherapy and currently Editor-in-Chief of the British Journal of Hematology. He has published over 500 papers, and is the editor/author of 5 books related to stem cell transplantation and bone marrow research. He is past Editor of Immunology and Cellular Therapy and has developed methods for assessing ex-vivo stem cell manipulations using in vitro and in vivo models. David is a member of the NIH recombinant DNA advisory committee (RAC).

John Barrett, MD
John Barrett trained at St. Bartholomew’s Hospital, London and specialized in hematology studying in London and Paris. In 1978 he became Head of the Department of Hematology and in 1982 Professor of Hematology, at Charing Cross and Westminster Medical School. In 1988 he was appointed Professor of Hematological Medicine at the Hammersmith Hospital, London. In 1993, he moved to Bethesda MD, USA and established and directed the NHLBI bone marrow transplant unit at the National Institutes of Health. In 2018 he assumed directorship of the stem cell transplant and cellular therapy program at George Washington University Cancer Center, Washington DC.

His contributions to hematology and oncology center around his work in pioneering stem cell transplantation as a treatment for malignant and non-malignant disorders, and his studies on the role of the immune system in eradicating malignant disease. Major achievements of his group include the discovery of targetable leukemia antigens, a graft-versus-tumor effect in renal cell carcinoma and the use of immunosuppressive treatments for myelodysplastic syndrome. He has used cellular therapy approaches to prevent and treat graft-versus-host disease after allogeneic stem cell transplantation by selective allodepletion of the graft and mesenchymal stromal cell infusion. In a series of clinical trials he used CD34 cell selection of the graft and prophylactic administration of multivirus specific T cells to optimize transplant outcome.

In 2016 he received the American College of Physicians Award for “Science as it Relates to Medicine”. John Barrett has over 500 publications, and is the editor/author of 5 books related to stem cell transplantation and bone marrow research. He is past Editor of Cytotherapy and currently Editor-in-Chief of the British Journal of Haematology. He has served as President both of the European Bone Marrow Transplantation Group and the American Society for Blood and Marrow Transplantation, receiving an ASBMT Lifetime achievement Award in 2018. He is a member of the American Society of Hematology, the British Society for Haematology and the International Society for Cellular Therapy which bestowed a Career Achievement Award on him in 2019.

About the Planning Faculty

CO-CHAIRS:
David DiGiusto, PhD
David is the Chief Technical Officer for Semma Therapeutics. He has over 28 years of experience in the scientific, clinical and regulatory aspects of cells as therapeutic agents including the isolation, characterization and genetic modification of hematopoietic stem cells and T-cells for clinical applications. He has been instrumental in the creation of six GMP compliant biologics manufacturing facilities and associated quality systems, production and QC testing programs. Under his direction, plasmid DNA, CAR-T-cells, regulatory T-cells, engineered stem cell grafts and gene modified hematopoietic stem cell products have been manufactured and released for use in Phase I/II clinical trials. David is a major contributor to first in human studies for Cancer and HIV Gene Therapy and has developed methods for assessing ex-vivo stem cell manipulations using in vitro and in vivo models. David is a member of the NIH recombinant DNA advisory committee (RAC).

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COMMITTEE:
Catherine Bollard, MD, MBChB
Catherine Bollard received her medical degree at the University of Otago in Dunedin, New Zealand. She is Board certified both in Pediatrics and Hematology. She worked in New Zealand and London, England before moving to Houston in 2000 where she was Professor of Pediatrics, Medicine and Immunology at Baylor College of Medicine (BCM) and Director of the Texas Children’s Cancer and Hematology Center Pediatric Lymphoma Program. In August 2013, she moved to Washington DC to join Children’s National Health System and The George Washington University School of Medicine and Health Sciences. She is currently the Bosworth Chair for Cancer Biology, Director of the Center for Cancer and Immunology Research, and Director of the Program for Cell Enhancement and Technologies for Immunotherapy (CETI) at Children’s National Health System. She is a Professor of Pediatrics and of Microbiology, Immunology and Tropical Medicine at The George Washington University and Associate Center Director for Translational Research and Innovation at the GW Cancer Center. She is a member of the American Society for Clinical Investigation (ASCI) and is the immediate Past President of the International Society for Cellular Therapy (ISCT) and has chaired the Non Hodgkin Lymphoma (NHL) Committee of the Children’s Oncology Group since 2012. She was on the Board of Directors of the Foundation in for the Accreditation of Cellular Therapy (FACT) from 2010-2018 and was a member of the Cellular, Tissues and Gene Therapies Advisory Committee of the Food and Drug Administration (FDA) from 2015-2019. In 2019 she became a member of the Frederick National Laboratory Advisory Committee (FNLAC) for the NIH and an ad hoc member of the Pediatric Oncologic Drugs Advisory Committee (ODAC) for the FDA. She has been an Associate Editor for the journal Blood since 2014 and recently completed her six year term on NCI’s Clinical Oncology (CONC) Study Section. Her bench and translational research focuses on improving outcomes for patients after hematopoietic stem cell transplantation and on the development of novel cell therapies for viral diseases and hematologic malignancies.

Colleen Delaney, MD, MSc
Colleen Delaney, MD, MSc is Scientific Founder and Chief Scientific Officer, Executive Vice President of Research and Development of Nohla Therapeutics, Inc., a cellular therapy company focused on development of universal, off-the-shelf cell therapies for patients with hematologic malignancies and other critical diseases. She is an Affiliate and former Member of the Fred Hutchinson Cancer Research Center Clinical Research Division, where she also held the Madeline Dabney Adams Endowed Chair in AML Research and was the principal investigator of an active, NIH-funded laboratory. In 2006, she established and became the Director of the Cord Blood Transplant Program at the Fred Hutch/Seattle Cancer Care Alliance, which has grown to be one of the leading programs in the country and the coordinating center of several multicenter clinical trials.

Dr. Delaney’s laboratory focused on the role of the Notch signaling pathway in the regulation and directed differentiation of hematopoietic stem and progenitor cells for clinical applications. This work led to several clinical trials investigating the potential of cryopreserved, non-HLA matched “off the shelf” ex vivo...
expanded cord blood progenitor cells to provide rapid but transient hematopoietic reconstitution in the settings of cord blood transplant and dose-intensive chemotherapy, ultimately leading to the spin-out of Nohla Therapeutics in December 2015. Nohla continues to explore the full potential of this novel and clinically feasible cell therapy platform for the ex vivo generation not only of hematopoietic progenitors but also of other clinically relevant hematopoietic cells, such as NK cells for off the shelf adoptive immunotherapy. Dr. Delaney received her MSc from Oxford University and her MD from Harvard Medical School and is the recipient of numerous awards, including the prestigious Damon Runyon Foundation Clinical Investigator Award, the Dr. Ali Al-Johani Award in recognition of exemplary clinical medical care and compassion to patients and families, the Seattle Business Journal’s Leaders in Health Care Award for Outstanding Medical Research and the Seattle American Women in Science’s Award for the Scientific Advancement and Leadership in STEM.

Bruce Levine, PhD

Dr. Bruce Levine, Barbara and Edward Netter Professor in Cancer Gene Therapy, is the Founding Director of the Clinical Cell and Vaccine Production Facility (CVPF) in the Department of Pathology and Laboratory Medicine and the Abramson Cancer Center, Perelman School of Medicine, University of Pennsylvania. He received a B.A. (Biology) from Penn and a Ph.D. in Immunology and Infectious Diseases from Johns Hopkins. First-in-human adoptive immunotherapy trials include the first use of a lentiviral vector, the first infusions of gene edited cells, and the first use of lentivirally-modified cells to treat cancer. Dr. Levine has overseen the production, testing and release of 3,000 cellular products administered to >1,200 patients in clinical trials since 1996. He is co-inventor of the first FDA approved gene therapy (Kymriah), chimeric antigen receptor T cells for leukemia and lymphoma, licensed to Novartis. Dr. Levine is co-inventor on 26 issued US patents and co-author of >170 manuscripts and book chapters with a Google Scholar citation h-index of 81. He is a Co-Founder of Tmunity Therapeutics, a spinout of the University of Pennsylvania. Dr. Levine is President Elect of the International Society for Cell and Gene Therapy and serves on the Board of Directors of the Alliance for Regenerative Medicine. He has been interviewed by the NY Times, Wall Street Journal, Washington Post, NPR, Time Magazine, National Geographic, Bloomberg, Forbes, BBC, and other international media outlets.

Krishna Komanduri, MD

Dr. Komanduri holds the Kalish Family Chair in Stem Cell Transplantation and is Professor of Medicine, Microbiology & Immunology and is the Director of the Adult Stem Cell Transplant Program and Associate Director for Clinical Innovation at the Sylvester Comprehensive Cancer Center at the University of Miami Miller School of Medicine. He is the inaugural Chair of the Division of Transplantation and Cellular Therapy. Dr. Komanduri received his undergraduate education at MIT (1987), his MD at the University of Minnesota Medical School (1991) and trained at UCLA (in Internal Medicine) and UCSF (in Hematology/Oncology). Prior to moving to Miami in 2008, he was a faculty member at UCSF and at the University of Texas M.D. Anderson Cancer Center. His laboratory research is focused on studies of cancer immunology and has been widely published and supported by the NIH and cancer-related foundations.

Dr. Komanduri served as the 2017-18 President of the American Society for Transplantation and Cellular Therapy (formerly the ASBMT). He serves as Co-Chair of the CIBMTR Working Committee on Infections and Immune Reconstitution and as a member of the Board of Directors of the National Marrow Donor Program (BeTheMatch) and as a member of the MIT NEWDIGS “think and do" tank on financing of novel curative therapies in oncology. He has also served as Chair of the Immunology and Host Defense Scientific Committee for the American Society of Hematology and as a clinical expert for the Institute for Clinical and Economic Review Policy Roundtable on CAR-T Therapies. Dr. Komanduri co-chaired the 2018 ASBMT consensus conference on grading and reporting of toxicities for CAR-T cell therapies and continues to serve as co-chair of the ASBMT Committee on Cellular Therapies. He has been the recipient of awards including election to the American Society for Clinical Investigation (in 2009).
About the International Scholars

Gabor Foldes, MD, PhD
Dr Gabor Foldes has considerable experience in balancing clinical work with academia throughout his career. He was working as a clinician (internal medicine and cardiology) in Budapest and London between 2002 and 2013. On the academic side, he received his PhD in cardiovascular biology in 2002. In 2006 he completed a fellowship on cardiac progenitor cells at Harvard University. Between 2007-2018, he worked as a research associate; since 2018 he has been a research fellow at Imperial College London, focusing on the generation and characterisation of cardiovascular derivatives of human pluripotent stem cells. He also holds an Associate Professor post at the Heart and Vascular Centre, Semmelweis University, Hungary. In addition to his clinical background he has extensive, translatable experience of stem cell biology, cardiovascular biology and pathology. His current research interests are in new cellular and therapeutic mechanisms in vascular disease and developing novel therapeutic and diagnostic strategies for patients with peripheral artery disease. His major research contributions to date have been to develop new vascular cell types from hPSCs and to perform successful transplantation experiments with hESC- and hiPSC-derived vascular grafts.

Giulia Golinelli, PhD
Giulia Golinelli holds an MA, summa cum laude et encomium, in Medical Biotechnology and a PhD in Molecular and Regenerative Medicine (2018), with the distinction cum laude, from the University of Modena. She has been recently awarded of a two-year fellowship in cancer research by The Fondazione Italiana per la Ricerca sul Cancro – AIRC (2019). She is committed to pursue her research career in understanding the mechanisms involved in cancer development and its treatment using cell-based approaches. She began undergraduate research at Laboratory of Cellular Therapies, headed by Prof. Massimo Dominici (University of Modena), working on pancreatic cancer to provide new insights on tumor-stroma interaction, exploring new therapeutics based on modified mesenchymal stromal cells (MSC) expressing anticancer molecules, such as TNF-related apoptosis inducing ligand (TRAIL). Next, thanks to a University fellowship, she took part in the development of an immunotherapy approach based on genetic reprogramming of cytotoxic immune-effector cells with a chimeric antigen receptor (CAR) to treat neuroblastoma and others disialoganglioside GD2-positive malignancies. The effector cells she is dealing with, gene-modified MSC on one hand and CAR-CTL on the other, are methods both aimed to manipulate the tumor microenvironment to destroy cancer from within. Recently, she experienced a challenging PhD path, during which her research experiences combined for the empowering of MSC effector cells to specifically target GD2-positive tumors, especially glioblastoma and Ewing's sarcoma. As metastases are a great challenge in Ewing's sarcoma patients, her current research focuses on the pre-clinical validation of this new strategy aiming to drive MSC localization and persistence to Ewing's sarcoma metastatic sites. Giulia is now mentoring an undergraduate student for its MS research project. She has been member of SITC and ISCT. Beside scientific topics her interests include modern dance, music and pottery. One of her favorite hobbies is walking. She mostly enjoys excursions in the Dolomites with her german shorthaired pointer called Heidi.

Andrea Henden, MBBS (Hons), FRACP, FRCPath
Dr Henden is an early career clinician-scientist currently working as a Staff Specialist in Haematology and Bone Marrow Transplantation at the Royal Brisbane and Women’s Hospital in Brisbane, Australia and also at QIMR Berghofer Medical Research Institute. Her PhD explored novel immunobiology of allogeneic bone marrow transplantation, including the effects of Interferon on acute graft-versus-host disease, and on enhancing graft-versus-leukaemia effects in a clinical setting. Her research focuses on translation of pre-clinical observations to clinical trials and clinical practice. Dr Henden’s current research examines the clinical utility of exogenous cytokine manipulation of T cell responses to improve treatment outcomes in bone marrow transplantation and adoptive T cell therapy.

Gaurav Sutrave, BSc(Med)/MBBS (Hons I) FRACP, FRCPath
Gaurav Sutrave is a clinician scientist currently undertaking a PhD in the field of cellular therapy through the University of Sydney, being supervised by Prof David Gottlieb and Dr Kenneth Micklethwaite. His work focuses on utilising gene modified T cells for the treatment of fungal infections and assessing alternative methods of gene modification to generate chimeric antigen receptor T cells. He has held a dual fellowship with the Royal Australasian College of Physicians and the Royal College of Pathologists of Australasia since 2017, and as the current Bone Marrow Transplant and Cell Therapies Fellow at Westmead Hospital in Sydney, he assists in several cell therapies clinical trials.

Karin Wisskirchen, PhD
Already during my studies of biology at the Philipps University in Marburg (2002-2007) I focused on learning about infectious diseases and majored in virology. I developed a particular interest in hepatitis B virus (HBV) infection and decided to write my diploma thesis on disinfection of HBV at the Justus Liebig University in Gießen.

Troubled by the question, why - despite a successful vaccine and other medications - there are still millions of people chronically infected with HBV, I started my PhD thesis on adoptive T cell therapy of chronic hepatitis B at the Institute of Virology at the Helmholtz Center in Munich (2007-2011). During this time, I generated and characterized HBV-specific receptors to enable T cells to recognize HBV-infected liver cells. I continued this project as a postdoc with a focus on clinical development and preclinical testing, including work as a guest scientist at the NIH. After spending time at home for maternity leaves I now go ahead with the project as the head of a small research team. Since chronic HBV infection has a high prevalence in Asia we also collaborate with a Singapore-based start-up company and hope that our research findings will be translated into a clinical success.

Robert Myles Wright, MBBS
Myles Wright completed his medical training at the University of Queensland after a Bachelor in Science at Monash University. He has since completed physician training at St Vincent’s Hospital in Melbourne and is due to complete his fellowship in haematology in 2020. He was the inaugural cell therapy fellow at the Peter MacCallum Cancer Centre / Victorian Comprehensive Cancer Centre and has worked on first in human trials associated with novel cell therapies. His particular areas of interest include improving outcomes in patients with myeloma and in novel methods of in vivo monitoring of cellular therapy activity.
About the North American Scholars

**Saurabh Dahiya, MBBS**
Saurabh Dahiya, MD, FACP is an Assistant Professor of Medicine in the Division of Hematology and Oncology at the University of Maryland School of Medicine. His clinical focus includes taking care of patients undergoing Allogeneic or Autologous Hematopoietic Stem Cell Transplantation and CAR-T cell therapy for hematologic conditions. His research interests include conducting clinical trials and translational research in the fields of Adoptive T-cell therapy such as CAR-T cell therapy, and stem cell transplantation.

**Shoba A. Navai, MD**
Shoba Navai, MD is an Assistant Professor in the Department of Pediatrics, Section of Hematology-Oncology, at Baylor College of Medicine in Houston, Texas. Dr. Navai is a graduate of the Medical College of Georgia and completed her pediatric residency training and pediatric hematology-oncology fellowship training at Baylor College of Medicine (BCM) and Texas Children’s Hospital. Since 2015, she has been working in the research laboratory of Drs. Nabil Ahmed and Meenakshi Hegde in the Center for Cell and Gene Therapy at BCM. Dr. Navai’s research interest focuses on translating CAR T cell products for the treatment of pediatric patients with solid tumors and brain tumors.

**Shabnum Patel, PhD**
Shabnum Patel is currently a Process Development & Manufacturing Scientist in the Center for Cancer Cell Therapy at Stanford University. She works on the scale up, process optimization, and testing of new technologies to generate CAR T cell therapies for leukemia, lymphoma, and brain tumors. She is involved in efforts to tech transfer new processes to both academic GMPs and CMOs, and manages logistics to support cell therapy trials for both the adult and pediatric cell therapy clinical teams. Previous to Stanford, Shabnum worked at both The George Washington University and Children’s National Health System in Washington, D.C., where she received her PhD in Immunology & Microbiology. Her research focused on the development of HIV immunotherapies for use post-HSCT as a cure strategy for HIV. This involved efforts to develop both autologous and allogeneic HIV T cell therapies, gene-editing approaches to cell therapies, and the translation of this work into several Phase I clinical trials.

**Irene Scarfo, PhD**
Dr. Scarfo received her PhD in Biomedical Sciences and Oncology at University of Torino in her native Italy. She worked with Prof. Roberto Piva on the molecular characterization of T-cell lymphomas identifying a new subclass of anaplastic large cell lymphomas. After completing her PhD, she accepted a position as Postdoctoral Research Fellow in Dr. Marcela Maus laboratory at Massachusetts General Hospital, moving towards a more translational research. During the last 3 years, she focused on designing new immunotherapies for the treatment of cancer. In particular, she developed a new chimeric antigen receptor (CAR) T-cell therapy targeting CD37, an antigen expressed by B- and T-cell lymphomas.

**Frederico Simonetta, MD, PhD**
Federico Simonetta, MD, PhD, is a postdoctoral research fellow in Robert S. Negrin Lab in the Division of Blood and Marrow Transplantation at Stanford University. He received his MD from the University of Genoa, Italy, his MSc in Immunology from University of Paris VII/Institut Pasteur, France, and his PhD in Immunology from University of Paris Sud, France. He trained in internal medicine and hematology at the Geneva University Hospitals, Switzerland, before joining the Negrin Lab for his postdoctoral training. He received in 2018 a New Investigator Award from the American Society of Blood and Marrow Transplantation and in 2019 a Best Abstract Award at the first TCT meeting.
About the Host Institutional Scholars

Mauro Castellarin, PhD
Mauro Castellarin received his PhD in the Department of Molecular Biology and Biochemistry from Simon Fraser University at the Genome Sciences Centre in Vancouver, Canada. His PhD research involved a metagenomic analysis of infectious agents associated with colon cancer, characterizing the mutational landscape in ovarian cancer tumors and immunoprofiling B cells and T cells associated with ovarian cancer. Mauro received a MSc in the Department of Surgical Research at McGill University in Montreal, Canada where he studied the genetics of newly formed insulin-producing cells in the pancreas. Mauro is currently a postdoctoral researcher in Dr. Carl June’s laboratory at the University of Pennsylvania. His research interests include creating a mouse model to study on-target, off-tumor CAR T cell toxicity and developing a new universal CAR that would allow T cells to retarget relapsed and refractory disease.

Saba Ghassemi, PhD
Dr. Ghassemi’s research focuses on developing chimeric antigen receptor (CAR) T cells with improved potency for adoptive immunotherapies. Dr. Ghassemi obtained her PhD at Columbia University in Mechanical Engineering. She gained extensive expertise in micro- and nanofabrication as well as significant experience in cell biology. Her work uncovered the existence of a nano-scale sarcomere-like unit for sensing the mechanical features of the extracellular environment. In 2011, she started her post-doctoral fellowship at the University of Pennsylvania in the Center for Cellular immunotherapies (CCI) under the mentorship of Drs. Michael Milone and Carl June. Her work showed how an abbreviated culture paradigm gives rise to less differentiated progeny with improved potency in xenograft models of ALL. Meanwhile, by actively working on the clinical translation of her novel cellular product, she gained a great deal of knowledge of the European technicalities and regulatory aspects of GMP-grade cell manufacturing.

In 2018, Dr. Ghassemi’s research was featured in Cancer Cell. Her work has led to the development of a new platform (PCT application no GB1803376.1), combining the unique antitumor features of a rare T cell subset, i.e. invariant Natural Killer T (iNKT) lymphocytes, and CAR technology, with the ultimate goal of developing a more effective CAR-iNKT immunotherapy with off-the-shelf usage potential. Dr. Ghassemi’s research has been recognized with a number of awards, including the St. Baldrick’s Scholar Career Development Award (2018) and the National Blood Foundation’s Early-Career Scientific Research Grants (2019). She has also been selected as an ISCT/ASBMT CTTC scholar in 2019.

Antonia Rotolo, MD, PhD
Dr. Rotolo graduated in her native Italy, where she earned her medical degree from the University of Turin in 2008. In the same year, she was also awarded a prize from the Medical Academy of Turin for her research studies aiming to identify molecular mechanisms of chemoresistance and targetable pathways in refractory/relapsed acute myeloid leukemias.

In 2014, Dr. Rotolo received her MD in internal medicine and hematology. She spent her last 2 years of clinical training at the Centre for Haematologym, Hammersmith Hospital, Imperial College London, UK, where she was offered the opportunity to conduct research aimed to develop novel therapeutic strategies for relapsed/refractory lymphoma patients. A number of exciting reports were suggesting that CART cells could potentially cure B lymphoma patients where all available treatments had previously failed. Hence, she enrolled in a PhD program at Imperial College London and committed to research in the field of CAR immunotherapy.

Within 4 years, she established and validated a novel cellular platform (PCT application no GB1803376.1), combining the unique antitumor features of a rare T cell subset, i.e. invariant Natural Killer T (iNKT) lymphocytes, and CAR technology, with the ultimate goal of developing a more effective CAR-iNKT immunotherapy with off-the-shelf usage potential (Rotolo et al., Cancer Cell, 2018). Meanwhile, by actively working on the clinical translation of her novel cellular product, she gained a great deal of knowledge of the European technicalities and regulatory aspects of GMP-grade cell manufacturing.

In 2018, Dr. Rotolo joined the team of CAR T-cell pioneer Carl June at the University of Pennsylvania, where she is currently a postdoc. Here, she was awarded a Lymphoma Research Foundation postdoctoral fellowship and was accepted to competitive ISCT programs that will be instrumental to provide her with additional skills and expertise which will facilitate her transition to independence.

PhD on using synthetic biology to develop enhanced chimeric antigen receptor T cell (CART) products for the efficacious treatment of solid malignancies.

Dr. Rotolo is committed to pursuing an academic career in the field of cancer immunotherapy and establish herself as an independent investigator, with active involvement in translational projects and clinical trials. Her ultimate goal is to generate and implement innovative cell-based therapeutics to positively impact on cancer patients lives.

Philipp C Rommel, Dr. rer. nat.
Philipp Rommel obtained his master’s (Diplom) and Ph.D. degree (Dr. rer. nat.) from the University of Stuttgart in Germany. He studied Technical Biology specializing in immunology, cell biology, biological process engineering and industrial genetics. His research studies comprise the fields of biochemistry, immunology and immunotherapy. For his bachelor’s thesis (Studienarbeit, 2009-2010), he joined the laboratory of Professor Marion Schmidt at the Albert Einstein College of Medicine in New York. During his master’s thesis (Diplomarbeit, 2011-2012) and Ph.D. thesis (Doktorarbeit, 2012-2017), he worked in the laboratory of Professor Michel C. Nussenzweig at the Rockefeller University in New York. In 2017 he joined the laboratory of Professor Carl H. June at the Perelman School of Medicine of the University of Pennsylvania as postdoctoral researcher. His current research studies are focused on using synthetic biology to develop enhanced chimeric antigen receptor T cell (CART) products for the efficacious treatment of solid malignancies.