

# Workshop on: Immunotherapies and Cellular Therapies for Systemic Sclerosis and Chronic GVHD

April 30 – May 1, 2024

Building 35, Rooms 620/630

National Institutes of Health, Bethesda, Maryland

## Agenda Items

### April 30, 2024

**8:00 AM – 8:10 AM**

#### **Welcome**

**Steven Pavletic M.D., PhD**, Senior Clinician; Head, GVHD and Late Effects Section, Immune Deficiency and Cellular Therapy Program; Clinical Director, Myeloid Malignancies Program, National Cancer Institute, Center for Cancer Research

**John Varga M.D.**, Frederick G. L. Huetwell Professor of Rheumatology; Chief, Division of Rheumatology; Professor of Internal Medicine. University of Michigan

**Bruce Blazar M.D.**, Regents Professor, Children's Cancer Research Fund Land Grant Chair in Pediatric Oncology, University of Minnesota

**8:10 AM - 8:20 AM**

#### **Introductory Remarks**

**James Gulley M.D., PhD**, Clinical Director; Deputy Director; Director, Medical Oncology Service; Senior Investigator, Co-Director, Center for Immuno-Oncology, National Cancer Institute, Center for Cancer Research

**8:20 AM - 8:30 AM**

#### **Opening Remarks**

**Lindsey A. Criswell, M.D., M.P.H., D.Sc.**, Director of the National Institute of Arthritis and Musculoskeletal and Skin Diseases.

**Part I Moderator**

**Bruce Blazar M.D.**, Regents Professor, Children's Cancer Research Fund Land Grant Chair in Pediatric Oncology, University of Minnesota  
(20 minutes presentation, 5 min Q&A)

#### **Session 1: Basic Mechanisms**

**8:30 AM – 8:55 AM**

#### **“Fibrosis: Major Advances and Ongoing Challenges”**

**Thomas A. Wynn, Ph.D.**, Vice President, and Discovery Head Inflammation & Immunology Pfizer Research & Development

- 8:55 AM – 9:20 AM**      **“TGF-Beta Production by Trem2+ Lipid-Associated Macrophages”**  
**Jesse Williams, Ph.D.**, Assistant Professor, Department of Integrative Biology and Physiology, University of Minnesota
- 9:20 AM – 9:45 AM**      **“Resident Memory T Cells and Fibrosis”**  
**Rachael Clark, MD, PhD**, Martin C. Mihm, Jr., MD and Shing-Yiu Yip  
Professor of Dermatology, Harvard Medical School
- 9:45 AM - 10:10 AM**      **“The Role of Microvascular Dysfunction and Microvasculopathy”**  
**Nicholas A Flavahan PhD**, Edward D. Miller Endowed Professor,  
Department of Anesthesiology and Critical Care Medicine  
John’s Hopkins University
- 10:10 AM – 10:25 AM**      **BREAK**
- Session 2: SSc and cGVHD Biology**
- 10:25 AM – 10:50 AM**      **“SSc Pathogenesis Overview”**  
**John Varga M.D.**, Chief, Division of Rheumatology, Professor of Internal Medicine, University of Michigan
- 10:50 AM – 11:15 AM**      **“Current cGVHD Overview”**  
**Nataliya Prokopenko Buxbaum, MD.**, Assistant Professor of Oncology,  
Division of Pediatric Oncology, Roswell Park Comprehensive Cancer Center, Department of Pediatrics, University at Buffalo Jacobs School of Medicine and Biomedical Sciences, Oishei Children’s Hospital
- 11:15 AM – 11:40 AM**      **“SSc Pathogenesis: Myeloid Cells Transcriptome”**  
**Michael L. Whitfield, Ph.D.**, Chair and Professor of Biomedical Data Science, Professor of Molecular and Systems Biology, Director, COBRE Center for Quantitative Biology, Co-Director, Burroughs Wellcome Big Data in the Life Sciences Training Program, Geisel School of Medicine at Dartmouth
- 11:40 AM – 12:15 PM**      **Panel 1: Current State of Affairs**
- Moderator: Edward Cowen, M.D., M.H.Sc.**, National Institute of Arthritis and Musculoskeletal and Skin Diseases
- Panelists:**
- Miguel-Angel Perales**, Memorial Sloan Kettering Cancer Center
  - Robert Colbert**, National Institute of Arthritis and Musculoskeletal and Skin Diseases
  - Donna Przepiorka**, Food and Drug Administration
  - Mary Wheatley**, National Scleroderma Foundation
  - Vicki Shanmugam**, Office of Research on Women’s Health, NIH, OD

**12:15 PM – 1:15 PM**      **LUNCH**

**12:25 PM – 1:10 PM**      **Lunch Panel: “Novel approaches to funding and infrastructure”**

**Moderator(s): John Varga, University of Michigan**  
**Keith Sullivan, Duke University**

**Panelists:**  
**Lindsey Criswell, National Institute of Arthritis and Musculoskeletal and Skin Diseases**  
**Steven Pavletic, National Cancer Institute**  
**Luke Evnin, Scleroderma Research Foundation**  
**Sophie Paczesny, University of Southern California**  
**Lis Welniak, National Heart, Lung, and Blood Institute**  
**Aimee Ahmed, Foundation for the National Institutes of Health**

**12:15 PM – 1:15 PM**      **LUNCH**

**Part II Moderator**      **John Varga M.D., Division of Rheumatology; Professor of Internal Medicine, University of Michigan**  
**(20 minutes presentation, 5 min Q&A)**

**Session 3: Preclinical Studies**

**1:15 PM – 1:40 PM**      **“Preclinical Models of Chronic GVHD”**  
**Bruce Blazar, MD., Director, Clinical and Translational Science Institute (CTSI), University of Minnesota**

**1:40 PM – 2:05 PM**      **“Transcriptomics in cGVHD non-human primates, cause-effect (Abatacept for Acute GVHD)”**  
**Leslie S. Kean, MD, PhD, Director, Stem Cell Transplant Center, Dana-Farber/Boston Children’s Cancer and Blood Disorders Center, Robert A. Stranahan Professor of Pediatrics, Harvard Medical School**

**2:05 PM – 2:30 PM**      **“Operational Tolerance, Omics, and Clinical Outcomes”**  
**Gerard Socie, MD., Ph.D., Prof of Hematology, University Paris Cité / Hematology Transplantation, AP-HP Hospital Saint Louis, Paris**

**2:30 PM – 2:55 PM**      **“Scleroderma: NOTCHing it up”**  
**Pravitt Gourh MD., Head, Scleroderma Genomics and Health Disparities Unit, Assistant Clinical Investigator, National Institute of Arthritis and Musculoskeletal and Skin Diseases, Adjunct Investigator, NIMHD**

**2:55 PM – 3:10 PM**      **BREAK**

**Session 4: Anti-fibrotics - Cell Therapies**

**3:10 PM – 3:35 PM**      **“Fibrosis Therapies”**  
**Joe Arron, MD, Ph.D., Chief Scientific Officer, Sonoma Biotherapeutics**

- 3:35 PM – 4:00 PM**      **“CART-T: Targeting Fibroblasts”**  
**Jonathan Epstein, MD.**, Interim Dean and Executive Vice President of the  
University of Pennsylvania Health System
- 4:00 PM – 4:25 PM**      **"Profiling and Immunotherapeutic Targeting of Fibrogenic Cells"**  
**Christian Stockman, MD.**, Assistant Professor  
Institute of Anatomy Winterthurerstrasse, Switzerland
- 4:25 PM – 5:00 PM**      **Panel 2: Road Towards Novel Therapeutic Strategies**
- Moderator: Pravitt Gourh**, National Institute of Arthritis and  
Musculoskeletal and Skin Diseases
- Panelists:**  
    **Nair Raj**, Food and Drug Administration  
    **Rachel Rosenstein**, Hackensack Meridian School of Medicine  
    **Resat Cinar**, National Institute on Alcohol Abuse and Alcoholism  
    **Leanne Peiser**, Bristol Myers Squibb  
    **Joe Arron**, Sonoma Biotherapeutics
- 5:00 PM – 5:10 PM**      **Day 1: Concluding Remarks**
- John Varga M.D.**, Division of Rheumatology; Professor of Internal  
Medicine, University of Michigan

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### May 1, 2024

8:00 AM - 8:15 AM

Opening Remarks

**John Varga M.D.**, Frederick G. L. Huetwell Professor of Rheumatology; Chief, Division of Rheumatology; Professor of Internal Medicine, University of Michigan.

Part III Moderator

**Steven Pavletic M.D., PhD**, Senior Clinician; Head, GVHD and Late Effects Section, Immune Deficiency and Cellular Therapy Program; Clinical Director, Myeloid Malignancies Program, National Cancer Institute, Center for Cancer Research

#### Session 5: Senolytics

8:15 AM - 8:40 AM

**"Senolytics: The Path to Translation"**

**Jim Kirkland, MD, PhD**, Consultant, Division of General Internal Medicine, Department of Internal Medicine, Consultant, Department of Physiology & Biomedical Engineering, Consultant, Section of Geriatric Medicine and Gerontology, Department of Internal Medicine, Mayo Clinic

#### Session 6: Immune Regulation and B Cell Studies

8:40 AM – 9:05 AM

**"B Cells and cGVHD"**

**Stefanie Sarantopoulos, MD., PhD**, Professor of Medicine, Professor of Integrative Immunobiology, Member of the Duke Cancer Institute, Hematologic Malignancies and Cellular Therapy, Duke University

9:05 AM – 9:30 AM

**"B cells in SSC: Resetting immune system/ tolerance (SSc) HSCT trials form the mechanistic perspective (tolerance)"**

**Bill Robinson, MD, PhD**, Professor of Medicine, Stanford University

9:30 AM – 9:45 AM

**BREAK**

## **Session 7: Overview & Clinical Studies**

- 9:45 AM – 10:10 AM**      **“Precision Medicine in Systemic Sclerosis.”**  
**Elizabeth Volkmann, MD., MS.,** Associate Professor, Director, UCLA Scleroderma Program, Co-Director, CTD-ILD Program, Division of Rheumatology, Department of Medicine
- 10:10 AM – 10:35 AM**      **“Systemic Sclerosis - Current Therapeutic Targets [including HSCT] and Lessons Learned from Recent Trials”**  
**Dinesh Khanna, MD., MSc.,** Professor of Medicine, Frederick G. L. Huetwell Professor of Rheumatology, Director, University of Michigan Scleroderma Program, Division of Rheumatology/Dept. of Internal Medicine
- 10:35 AM – 11:00 AM**      **“Overview of cGVHD Therapies and Pipeline”**  
**Stephanie Lee, MD., MPH.,** Professor, Fred Hutchinson Cancer Center
- 11:00 AM – 11:35 AM**      **Panel 3: “Road to Tomorrow”**
- Moderator: Steven Pavletic, National Cancer Institute, Center for Cancer Research**
- Panelists:**  
**Aimee Payne, Columbia University**  
**Eric Tkaczyk, Vanderbilt University Medical Center**  
**Max Konig, Johns Hopkins University**  
**Vedran Radojicic, Syndax Pharmaceuticals, Inc.**  
**Bruce Blazar, University of Minnesota**
- 11:35 AM – 12:00 PM**      **Day 2: Concluding Remarks**
- John Varga M.D.,** Frederick G. L. Huetwell Professor of Rheumatology; Chief, Division of Rheumatology; Professor of Internal Medicine, University of Michigan.
- Bruce Blazar M.D.,** Regents Professor, Children's Cancer Research Fund Land Grant Chair in Pediatric Oncology, University of Minnesota
- Steven Pavletic M.D., PhD, Senior Clinician;** Head, GVHD and Late Effects Section, Immune Deficiency and Cellular Therapy Program; Clinical Director, Myeloid Malignancies Program, National Cancer Institute, Center for Cancer Research