

RegenBio Summit: Transforming Next-Gen Biotherapeutics 2024

Ponte Vedra Beach, Florida

December 9, 2024 to December 11, 2024

Eastern Standard Time

Day 1 Monday, December 9, 2024

8:00 AM **Breakfast, Registration & Exhibits**

9:00 AM **Welcome Announcements**
Julie G. Allickson, Ph.D. and Saranya P. Wyles, M.D., Ph.D.

9:10 AM **Welcome to Mayo Clinic Florida**
Kent R. Thielen, M.D.

9:15 AM **Keynote: Regulating Regenerative Therapies from IND to BLA**
Peter Marks, M.D., Ph.D.

10:00 AM **Fireside Chat: Mistakes and Lessons Learned from FDA CBER Director's Desk. An Insight into Accelerated Approval Pathways**
Moderator: Julie G. Allickson, Ph.D.
Panelist: Peter Marks, M.D., Ph.D.

10:30 AM **Laryngeal Transplantation and Regeneration: Breaking the Silence**
David G. Lott, M.D.

11:00 AM **Regenerative Transplantation: A New Paradigm**
Giuseppe Orlando, M.D., Ph.D.

11:30 AM **Lunch**

12:30 PM **7 advances in CAR-T cell therapy at Mayo Clinic | Inside Mayo Clinic Research**
Hong Qin, M.D., Ph.D.

1:00 PM **Fireside Chat: Impact of Generative Artificial Intelligence on Cell and Gene Therapy**
Moderator: Peter A. Noseworthy, M.D., M.B.A.
Panelist: Rickey E. Carter, Ph.D.

Regenerative Orthopedics and Musculoskeletal: From Care to Cure
Moderator: Daniel B. F. Saris, M.D., Ph.D.

1:30 PM **Hip RECLAIM: A Phase I Safety and Feasibility Trial of REcycled CartiLage Auto/Allo IMplantation for the Treatment and Repair of Focal Hip Cartilage Defects**
Daniel B. F. Saris, M.D., Ph.D.

1:45 PM **Regenerative Medicine Clinical Trials in Sports Medicine and Orthopedics**
Brennan J. Boettcher, D.O.

2:00 PM **Clinical Regenerative Medicine at Mayo Clinic**
Shane A. Shapiro M.D.

2:15 PM **Break and Exhibits**

Tissue Engineering Session
Moderators: David G. Lott, M.D.

2:45 PM **Plenary Highlight: Can We Grow a Personalized Heart**
Doris Taylor, Ph.D.

3:15 PM **3D Bioprinting Skin**
Saranya P. Wyles, M.D., Ph.D.

3:30 PM **Fireside Chat: Exosomes, Are We There Yet?**
Moderator: Abba Zubair M.D. Ph.D.
Panelist: Atta Behfar, M.D., Ph.D., and Jon Rowley Ph.D.

3:45 PM **Defining the Issues: Approaches to Centralized,
Decentralized, and Point of Care Manufacturing of
Regenerative Medicine Products**
Richard McFarland, Ph.D., M.D.

4:15 PM **Day 1 Closing Comments**

4:30 PM **Adjourn**

5:15 PM **Welcome Reception**
Women in Translational Science Award

Day 2 **Tuesday, December 10, 2024**

8:00 AM **Breakfast and Exhibits**

8:00 AM **Breakfast with Experts**

FDA Unveiled: Navigating U.S. Regulations and Regulators

Drew M. Witter, M.S., RAC

Harmonizing Excellence: A Global Odyssey to Meet GMP Requirements

Craig Hasilo

Precision Engineering of Regenerative Biotherapeutics: A Focus on Process Development and Biomanufacturing

Cynthia E. Wilkins, Ph.D.

Precision Engineering of Regenerative Biotherapeutics: A Focus on Process Development and Biomanufacturing

Snigdha Rai

Building a Culture of Quality: The Role of QA and QC

Tony W. DeFusco

Building a Culture of Quality: The Role of QA and QC

Cameron E. Jones

9:00 AM **From the Desk of Mayo Clinic Center for Regenerative Biotherapeutics Medical Director**

William Faubion, M.D.

9:30 AM **Panel: Ethics in Gene Therapy**

Moderators: Michelle McGowan, Ph.D.

Panelist: Megan Allyse, Ph.D., and Scott Wright M.D.

10:00 AM

Break & Exhibits

10:30 AM

Keynote: Trial of Beremagene Geperpavec (B-VEC) for Dystrophic Epidermolysis Bullosa – First FDA-Approved Topical Gene Therapy

M. Peter Marinkovich, M.D.

11:00 AM

Fireside Chat: Mistakes and Lessons Learned From Startups. An Insight into the Success Path of Krystal Biotech, Inc.

Moderator: Saranya P. Wyles, M.D., Ph.D.

Panelist: M. Peter Marinkovich, M.D.

11:20 AM

Addressing Challenges in Translation of Cell and Gene Therapy

Uma Lakshmipathy Ph.D.

11:40 AM

Mayo MedTech Accelerator: The Future of Cell and Gene Therapy

Marianne van der Steen, Ph.D.

12:00 PM

Lunch/Exhibits

Cell and Gene Therapy Plenary

Moderator: Sanjay Misra, M.D.

1:00 PM

Role of Stem Cells in Vascular Injury

Sanjay Misra, M.D.

1:15 PM

The Allure of Biomanufacturing Tregs for Inflammatory Bowel Disease

William Faubion, M.D.

1:30 PM

CELLTOP: An Update from Autologous Adipose Derived Mesenchymal Stem Cells in the Treatment of Paralysis Due to Traumatic Spinal Cord Injury

Mohamad Bydon, M.D.

1:45 PM **Tumor-Agnostic Cellular Therapies**
Michael Gustafson Ph.D.

2:00 PM **An Autologous Dendritic Cell Vaccine Targeting Folate Receptor Alpha in Patients with Ovarian Cancer**
Matthew S. Block, M.D., Ph.D.

2:15 PM **Panel Discussion**
Moderators: William Faubion, M.D. and Sanjay Misra, M.D.
Panelist: Session Presenters

2:30 PM **Fireside Chat: Next Gen Workforce Development**
Maggie A. DiGuardo, M.D., Saranya P. Wyles, M.D., Ph.D.,
Elena Maryamchik, M.D., M.B.A.

3:00 PM **Break and Exhibits**

Showcase: Mayo Clinic Florida
Moderators: Wenchun Qu, M.D., Ph.D.

3:30 PM **Beyond the Clinic: The Impact of Mayo Clinic Florida Center for Regenerative Biotherapeutics**
Alfredo Quinones-Hinojosa, M.D., Brian Hultgren

3:45 PM **Differentiation of Human Induced Pluripotent Stem Cells to Functional Brain Cells**
Tsuneya Ikezu, M.D., Ph.D.

4:00 PM **Mayo Clinic RNA Discovery and Therapeutics Program: Premise, Promise and Progress**
Nilufer Taner, M.D., Ph.D.

4:15 PM

Aptamers: Unlocking the Potential of Immunotherapy and Regenerative Medicine

Abba Zubair, M.D., Ph.D.

4:30 PM

Regenerative Clinical Trial Portfolio (Non-engineered Cell Therapy) Moderated Discussion

Moderators: Abba Zubair, M.D., Ph.D.

Panelist:

Wenchen Qu, M.D., Ph.D.- Regenerative Rehabilitation

LaTonya J Hickson, M.D. – Regenerative Nephrology

Sanjeet S. Grewal, M.D. – Regenerative Neurology

Katherine J. Bodiford, M.D. – Regenerative Dermatology

Sam Farres, M.D. – Regenerative Vascular Surgery

5:00 PM

Closing and Adjourn

5:05 PM

Poster Session & Networking

6:00 PM

Adjourn

Day 3 **Wednesday, December 11, 2024**

8:00 AM **Breakfast and Exhibits**

9:00 AM **Opening Remarks**
Saranya P. Wyles, M.D., Ph.D., and Praneetha R. Elugunti,
M.H.A., M.B.A.

9:05 AM **Keynote: Exploring the Promise of CRISPR Therapeutics In Vivo and Cell Therapy Applications**
Birgit Schultes, Ph.D.

9:35 AM **Fireside Chat: Mistakes and Lessons Learned from Startups. An insight into the success Path of Intellia Therapeutics, Inc.**
Moderator: Laura J. Lambert, Ph.D.
Panelist: Birgit Schultes Ph.D.

10:05 AM **Plenary Highlight: Transforming Lives with Personalized Cancer Therapies**
Introduction: Alina R. Oancea
Speaker: Bruce Levine, Ph.D.

10:35 AM **Mayo Clinic Highlight: New Directions in Engineered Cell Therapy**
Saad J. Kenderian, M.B., Ch.B.

11:05 AM **Fireside Chat: Immunotherapy**
Moderator: Saad J. Kenderian, M.B., Ch.B.
Panelist: Mohamed A. Kharfan Dabaja, M.D., M.B.A.

11:20 AM **Fireside Chat: Patient Access for Cell and Gene Therapies – Challenges and Future Solutions**
Moderator: Julie Allickson, PhD
Panelist: Saad J. Kenderian, M.B., Ch.B., Ph.D. and
Bruce L. Levine, Ph.D.

11:45 AM **Healthcare Innovation – Discussion**
Gloria R. Olivier, Ph.D., Rupa Pike, Ph.D., Sarah Creviston, Daniel Cushing Ph.D.

12:10 PM **Lunch & Exhibits**

iPSC Therapy Plenary
Moderator: Zachary Resch, Ph.D.

1:10 PM **A Vision to Grow Stem Cells into Retinal Pigment Epithelium (RPE) for Eyesight**
Kapil Bharti, Ph.D.

1:25 PM **The Path to a Cure for Type 1 Diabetes: What's Next in Cell Therapy and Beyond?**
Quinn P. Peterson, Ph.D.

1:40 PM **Closer to Precision Cardiology With iPSCs**
Max Gnecci M.D. Ph.D.

1:55 PM **iPSC-Derived Cardiac Lineages For Heart Repair in Congenital Heart Disease**
Timothy Nelson, M.D., Ph.D.

2:10 PM **Panel Discussion: iPSC**
Moderator: Dennis Wigle, M.D., Ph.D.
Panelist: Session Presenters

2:25 PM **Closing Remarks**
Julie G. Allickson, Ph.D., and Saranya P. Wyles, M.D., Ph.D.

2:30 PM **Adjourn**