

**RegenBio Summit: Transforming Next-Gen
Biotherapeutics 2024
Ponte Vedra Beach, Florida
December 9-11, 2024
Eastern Standard Time**

Monday, December 9, 2024

- 8:00 a.m. **Registration, Breakfast and Exhibits**
- 9:00 a.m. **Welcome Announcements**
Julie G. Allickson, Ph.D. and Saranya P. Wyles, M.D., Ph.D.
- 9:10 a.m. **Welcome to Mayo Clinic Florida**
Kent R. Thielen, M.D.
- 9:15 a.m. **Keynote: Regulating Regenerative Therapies from IND to BLA**
Peter Marks, M.D., Ph.D.
- 10:00 a.m. **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 a.m. **Laryngeal Transplantation and Regeneration: Breaking the Silence**
David G. Lott, M.D.
- 11:00 a.m. **Regenerative Transplantation: A New Paradigm**
Giuseppe Orlando, M.D., Ph.D.
- 11:30 a.m. **Lunch and Exhibits**
- 12:30 p.m. **7 Advances in CAR-T Cell Therapy at Mayo Clinic**
Hong Qin, M.D., Ph.D.
- 1:00 p.m. **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 p.m. **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 p.m. **Regenerative Medicine Clinical Trials in Sports Medicine and Orthopedics**
Brennan J. Boettcher, D.O.

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

2:15 p.m. **Break and Exhibits**

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

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

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

3:30 p.m. **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 p.m. **Defining the Issues: Approaches to Centralized, Decentralized, and Point of Care Manufacturing of Regenerative Medicine Products**
Richard McFarland, Ph.D., M.D.

4:00 p.m. **Closing Comments and Women in Translational Science Award**

4:15 p.m. **Adjourn**

5:15 p.m. **Welcome Reception**

Tuesday December 10, 2024

- 8:00 a.m. **Breakfast and Exhibits**
- 8:00 a.m. **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 a.m. **From the Desk of Mayo Clinic Center for Regenerative Biotherapeutics
Medical Director**
William Faubion, M.D.
- 9:15 a.m. **Panel: Ethics in Gene Therapy**
Moderators: Michelle McGowan, Ph.D.
Panelist: Megan Allyse, Ph.D., and Scott Wright M.D.
- 9:45 a.m. **Break and Exhibits**
- 10:15 a.m. **Keynote: Trial of Beremagene Geperpavec (B-VEC) for Dystrophic
Epidermolysis Bullosa – First FDA-Approved Topical Gene Therapy**
M. Peter Marinkovich, M.D.
- 10:45 a.m. **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.
- 11:05 a.m. **Addressing Challenges in Translation of Cell and Gene Therapy**
Uma Lakshmi Ph.D.

- 11:15 a.m. **Platelet-Derived Exosomes as a Regenerative Medicine
Therapeutic: A Look Back on Translation from Bench Bedside**
Chris Paradise, Ph.D.
- 11:45 a.m. **Lunch and Exhibits**
- Cell and Gene Therapy Plenary Moderator: Sanjay Misra, M.D.**
- 12:45 p.m. **Role of Stem Cells in Vascular Injury**
Sanjay Misra, M.D.
- 1:00 p.m. **The Allure of Biomanufacturing Tregs for Inflammatory
Bowel Disease**
William Faubion, M.D.
- 1:30 p.m. **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 p.m. **Tumor-Agnostic Cellular Therapies**
Michael Gustafson Ph.D.
- 2:00 p.m. **An Autologous Dendritic Cell Vaccine Targeting Folate Receptor Alpha
in Patients with Ovarian Cancer**
Matthew S. Block, M.D., Ph.D.
- 2:15 p.m. **Panel Discussion**
Moderators: William Faubion, M.D. and Sanjay Misra, M.D.
Panelist: Session Presenters
- 2:30 p.m. **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 p.m. **Mayo MedTech Accelerator: Current State and Incorporation of CGT**
Steven J. Lester, M.D.
- 3:10 p.m. **Break and Exhibits**
- Showcase: Mayo Clinic Florida
Moderator: Wenchun Qu, M.D., Ph.D.**
- 3:30 p.m. **Beyond the Clinic: The Impact of Mayo Clinic Florida Center for
Regenerative Biotherapeutics**
Alfredo Quinones-Hinojosa, M.D., and Brian Hultgren
- 3:45 p.m. **Differentiation of Human Induced Pluripotent Stem Cells to Functional
Brain Cells**
Tsuneya Ikezu, M.D., Ph.D.

- 4:00 p.m. **Mayo Clinic RNA Discovery and Therapeutics Program: Premise, Promise and Progress**
Nilufer Taner, M.D., Ph.D.
- 4:15 p.m. **Aptamers: Unlocking the Potential of Immunotherapy and Regenerative Medicine**
Abba Zubair, M.D., Ph.D.
- 4:30 p.m. **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 p.m. **Closing and Adjourn**
- 5:05 p.m. **Poster Session and Networking**
- 6:00 p.m. **Adjourn**

Wednesday, December 11, 2024

- 8:00 a.m. **Breakfast and Exhibits**
- 9:00 a.m. **Opening Remarks**
Saranya P. Wyles, M.D., Ph.D., and Praneetha R. Elugunti, M.H.A., M.B.A.
- 9:05 a.m. **Keynote: Exploring the Promise of CRISPR Therapeutics In Vivo and Cell Therapy Applications**
Birgit Schultes, Ph.D.
- 9:35 a.m. **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 a.m. **Plenary Highlight: Transforming Lives with Personalized Cancer Therapies**
Introduction: Alina R. Oancea
Speaker: Bruce Levine, Ph.D.
- 10:35 a.m. **Mayo Clinic Highlight: New Directions in Engineered Cell Therapy**
Saad J. Kenderian, M.B., Ch.B.
- 11:05 a.m. **Fireside Chat: Immunotherapy**
Moderator: Saad J. Kenderian, M.B., Ch.B.
Panelist: Mohamed A. Kharfan Dabaja, M.D., M.B.A.
- 11:20 a.m. **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 a.m. **Healthcare Innovation – Discussion**
Gloria R. Olivier, Ph.D., Rupa Pike, Ph.D., Sarah Creviston, Daniel Cushing Ph.D.
- 12:10 p.m. **Lunch and Exhibits**
- iPSC Therapy Plenary**
Moderator: Zachary Resch, Ph.D.
- 1:10 p.m. **A Vision to Grow Stem Cells into Retinal Pigment Epithelium (RPE) for Eyesight**
Kapil Bharti, Ph.D.
- 1:25 p.m. **The Path to a Cure for Type 1 Diabetes: What’s Next in Cell Therapy and Beyond?**
Quinn P. Peterson, Ph.D.

- 1:40 p.m. **Closer to Precision Cardiology With iPSCs**
Max Gnechi M.D. Ph.D.
- 1:55 p.m. **iPSC-Derived Cardiac Lineages for Heart Repair in Congenital Heart Disease**
Timothy Nelson, M.D., Ph.D.
- 2:10 p.m. **Panel Discussion: iPSC**
Moderator: Zachary T. Resch, Ph.D.
Panelist: Session Presenters
- 2:25 p.m. **Closing Remarks**
Julie G. Allickson, Ph.D., and Saranya P. Wyles, M.D., Ph.D.
- 2:30 p.m. **Adjourn**