

# 16<sup>th</sup> International Cord Blood Symposium Scientific Program and Invited Speakers

**Thursday, June 14, 2018**

**Welcoming Remarks and Keynote: Novel Therapies and the Regulatory Challenges They Bring**

- 8:30 am            FDA's Perspectives for RMAT and the 21st Century Cures Act  
Wilson Bryan, MD, Director, Office of Tissues and Advanced Therapies, CBER
- 9:00 am            Gene Therapy for Hemoglobinopathies  
Mark Walters, MD
- 9:30 am            Unproven Therapies: From Do-It-Yourself Gene Editing to Stem Cell Tourism  
Leigh Turner, PhD
- 10:00 am           Refreshment Break with Exhibitors

**Outcomes of Cord Blood Transplant**

- 10:30 am           "Best of the European Studies" - Highlights from EBMT and Eurocord  
Annalisa Ruggeri, MD, PhD
- 11:00 am           Cord Blood Transplantation in HIV Infected Patients Using CCR5-/- Units  
Koen van Besien, MD, PhD
- 11:30 am           HHV-6 Prophylaxis with Foscarnet: Implications for Cord Blood Transplantation  
Claudio Brunstein, MD
- 12:00 pm           Lunch with Exhibitors**

### **Novel Cord Blood Derived Cellular Therapies and Their Clinical Applications**

- 1:30 pm Cord Blood Derived NK Cells for Multiple Myeloma  
Nina Shah, MD
- 2:00 pm Cord Blood Derived Virus Specific T Cells for the Treatment of Infections Post Allogeneic Stem Cell Transplantation  
Patrick Hanley, PhD
- 2:30 pm Large Scale Bioreactor Generated Expansion of Cord Tissue Derived MSCs for Clinical Use  
Confirming Speaker

### **3:00 pm Refreshment Break with Exhibitors**

### **Simultaneous Session A: Cord Blood Expansion: Clinical Trials and Outcomes**

- 3:30 pm Phase II Trials with MGTA-456 Single Cord Blood Unit Transplants  
John Wagner, MD
- 4:00 pm Nicord Single Unit Expanded Cord Blood Transplantation - Final Results  
Mitchell Horwitz, MD
- 4:30 pm Single UM 171 Expanded Cord Blood Transplantation - Results of a Clinical Trial  
Sandra Cohen, MD

### **Simultaneous Session B: The Corporate Match: Connecting Available Units from Cord Blood Banks with their Industry Partner End Users**

This session will serve as a 'mixer' where facilitators will discuss topics such as the types of raw materials available for research and commercial development, the development of donor consents, and the benefits of synergies - acknowledgement, credibility and profit sharing.

Facilitators: Donna Regan MT(ASCP)SBB, Terri Tibbot, MS CTBS; Georgina Michael, MSN, RNFA, FNP-BC, CWCN

### **5:30-7:00 pm Welcome Reception and Poster Viewing**

**Friday, June 15, 2018**

**Controversies in Cord Blood Transplantation**

8:30 am High Resolution Typing vs CD34+ Enumeration in Cord Blood Unit Choice  
Mary Eapen, MD, MS and Jaime Sanz, MD

9:15 am Is HLA Matching at the C Locus Necessary?  
Karen Ballen, MD and Claudio Brunstein, MD

**10:00 am Refreshment Break with Exhibitors**

**Workshop A: National Cord Blood Inventory: Crisis or Opportunity?**

10:30 am HRSA - The RAND Report - Response and Next Steps  
Karen Ballen, MD

11:00 am The Role of the Cord Blood Bank in Developing New Products  
Brian Freed, PhD

11:30 am Consolidation of Public Cord Blood Banks: A Case Study  
Donna Regan MT(ASCP)SBB

**Workshop B: Translational Projects using Placental Derived Cells**

10:30 am CRISPR/Cas9 Gene Editing: A Primer  
Mathew Porteus, MD, PhD

11:00 am Exosomes of Placental Derived MSC Stimulate Angiogenesis  
Jan Nolta, PhD

11:30 am Perinatal Cells in Gene Therapy for Hemophilia  
Confirming Speaker

**12-1:30 pm Lunch with Exhibitors**

**Workshop C: Top 5 Manufacturing Challenges**

Rebecca Haley, MD,; Mike Halpenny; Andromachi Scaradavou MD

This workshop will explore the top 5 Manufacturing challenges. Topics include:

- The establishment of product specifications including the handling out of specification units, emerging infectious diseases, licensed products that fail upon release and are issued under IND
- Post thaw testing options, best practices and non destructive testing methods
- Potency and sterility lessons learned from the NMDP Cord Blood Advisory Group
- Facility considerations and the consideration of outsourcing production
- The role of GMP and efforts to decrease the cost of goods

**Workshop D: Selecting Cord Blood Units**

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| 1:30 pm | Selecting Optimal Cord Blood Units: Limitations of Cell Dose<br>Martin Maiers                                  |
| 1:55 pm | Adoptive Immunotherapy with Cord Blood Grafts Targeting<br>Shared IPA and/or NIMA<br>Andromachi Scaradavou, MD |
| 2:20 pm | Update on 10-CBA Protocol: An IND Study<br>John Miller, MD, PhD  |

**2:45 pm**            **Refreshment Break with Exhibitors**

**3:15 pm**            **Oral Abstract Presentations**  
Kate Brown, PhD

**Workshop E: Delayed Clamping - Balancing Product Quality and Donor Safety**

- 4:15 pm      Reconciling Procurement Practice with Clinical Recommendations  
(ACOG and AAP)  
Rebecca Haley, MD
- 4:40 pm      Impact of Delayed Clamping on Public Cord Blood Banking  
Andromachi Scaradavou, MD
- 5:05 pm      Delayed Clamping and Its Impact on Product Volume and  
Evaluation of Collection Devices  
Kate Girard, RN, MSN

**Workshop F: New Models for Public/Private Partnerships in Hematopoietic Stem Cell Banking**

- 4:15 pm      Procurement of HSCs from Deceased Donor Bone Marrow:  
Implications for Minority Recipients  
Prakash Rao, PhD
- 4:40 pm      Partnering with Patient Advocacy Organizations for Directed  
Donor Banking  
Joanna Tilley, PhD
- 5:05 pm      Redefining Cord Blood Banking: Charting New Paths in Asia  
Mrinalini Chaturvedi, MBBS, MD, MBA, PGDMLE

**Adjourn**

**Saturday, June 16, 2018**

**Regenerative Medicine Applications of Cord Blood/Tissue Derived Therapies**

8:30 am Cord Blood Derived Cells for the Treatment of Hypoplastic Left Heart Syndrome  
Confirming Speaker

9:00 am Cord Tissue Derived MSCs for the Treatment of Lupus in Adults  
Gary Gilkeson, MD

9:30 am Autologous Human Cord Blood for Traumatic Brain Injury in Children  
Charles Cox, MD

10:00 am Update on Clinical Trials for Brain Injury and Dysfunction  
Confirming Speaker

**10:30 am Refreshment Break**

**Regenerative Medicine Applications of Perinatal Tissue Derived Therapies**

11:00 am A Dozen Years of Clinical Trials Using Perinatal Cells  
Fran Verter, PhD

11:30 am Cryopreserved Placental Membrane for Wound Care  
Georgina Michael, MSN, RNFA, FNP-BC, CWCN

12:00 pm The Use of Placental Expanded Cells in Critical Limb Ischemia  
Confirming Speaker

**12:30 pm Networking at Noon**  
Lunches will be available for attendees to take with them or meet with colleagues at the conclusion of the Symposium.