### Schedule

### Thursday April 17 – Cole Hall

### 8:00-8:30 **Breakfast**

| Session I - Hematopoiesis and The Hematopoietic Niche |
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| Chair: Tippi MacKenzie                                |

| 8:30-8:40<br>8:40-9:40 | Introduction Alan Flake: In utero transplantation – Lessons learned in 30 years Children's Hospital of Philadelphia  |
|------------------------|--|
| 9:40-10:00             | <b>Graca Almeida-Porada</b> : Ontogeny of the human hematopoietic niche Wake Forest University   |
| 10:00-10:20            | <b>William Peranteau</b> : Ex vivo modification of donor hematopoietic cells to enhance allogeneic engraftment following <i>in utero</i> hematopoietic cell transplantation  Children's Hospital of Philadelphia |
| 10:20-10:30            | Break  |
| 10:30-11:30            | Irv Weissman: Normal and neoplastic stem cells Stanford University   |
| 11:45-1:00             | Lunch/Display Posters  |

### Session II - Hematopoietic Diseases Chair: Robert Montgomery

| 1:15-1:35 | <b>Jennifer Puck</b> : Newborn Screening for SCID and T Cell Lymphopenic Disorders<br><i>University of California, San Francisco</i>   |
|-----------|--|
| 1:35-1:55 | Elliott Vichinsky: Alpha thalassemia major: new mutations, intrauterine management, and outcomes  Children's Hospital of Oakland Research Institute  |
| 1:55-2:15 | <b>Jerry Chan</b> : Combined intrauterine and postnatal cell therapy using fetal liver and adult bone marrow cells facilitates sustained donor cell chimerism in a murine thalassemia model  *Duke - National University of Singapore* |
| 2:15-2:35 | <b>David Archer</b> : Alterations in specific immune cell subsets in children with sickle cell disease <i>Emory University</i>   |
| 2:35-3:00 | Coffee Break   |

Session III – Amniotic Fluid and Mesenchymal Stem Cell Transplantation Chair: Anna David

| 3:00-3:20 | <b>Cecilia Gotherstrom:</b> Ten year follow up after prenatal transplantation of fetal mesenchymal stem cells in a patient with severe osteogenesis imperfecta <i>Karolinska Institutet</i>       |
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| 3:20-3:40 | Pascale V Guillot: A fetal-to-fetal approach for the treatment of brittle bone disease  University College London   |
| 3:40-4:00 | Panicos Shangaris: Haematopoietic engraftment of amniotic fluid stem cells following in utero transplantation  University College London  |
| 4:00-4:20 | Steven Shaw:  potential both in vitro and in vivo and engraft after autologous in utero stem cell transplantation  University College London  |
| 4:20-4:40 | George Christ: Muscle progenitor cells for tissue engineered muscle repair (TEMR) of volumetric muscle loss (VML) injuries: cleft lip as "first-in-man" target indication  Wake Forest University |
| 4:40-5:00 | <b>Aijun Wang:</b> Engineering biomaterials and stem cells for <i>in utero</i> repair of structural birth defects  University of California, Davis  |
| 5:00-5:20 | <b>Anna David:</b> Translating a prenatal gene therapy into the clinic: experience with maternal uterine artery VEGF gene therapy for fetal growth restriction.                                   |
| 5:20-6:00 | University College London  Group Discussion (Part I)  |

### Friday April 18 – Cole Hall

8:00-8:30 Breakfast

| Session I - Transplantation Immunology Chair: Graca Almeida-Porada |   |  |  |
|--|---|--|--|
| 8:30-9:30  | Maria Grazia Roncarolo: Induction of tolerance to allogenic stem cells<br>San Raffaele Telethon Institute for Gene Therapy                                  |  |  |
| 9:30-9:50  | Qizhi Tang: Immune tolerance by design University of California, San Francisco  |  |  |
| 9:50-10:10   | <b>Tippi MacKenzie</b> : Mechanisms of tolerance induction after in utero hematopoietic cell transplantation <i>University of California, San Francisco</i> |  |  |
| 10:10-10:30  | Break   |  |  |

| Session II - Maternal/Fetal/Neonatal Tolerance Chair: Nick Fisk |  |  |  |
|---|--|--|--|
| 10:30-10:50   | Aimen Shaaban: Prenatal NK cell education as the final endorsement of allospecific tolerance  Cincinnati Children's Hospital   |  |  |
| 10:50-11:10   | <b>Trevor Burt:</b> Using fetal and adult gene expression signatures to predict immunity in the newborn <i>University of California, San Francisco</i>   |  |  |
| 11:10-11:30   | <b>Becky Adkins:</b> Dynamic epigenetic events in murine fetal and neonatal T cell ontogeny <i>University of Miami</i>   |  |  |
| 11:30-11:50   | Magnus Westgren: Differentiation and functional regulation of human fetal NK cells  Karolinska Institutet  |  |  |
| 12:00-1:15  | Lunch/Posters  |  |  |
| 1:30-1:50   | Chris Baker: Analysis of maternal microchimerism in rhesus monkeys (Macaca mulatta) using real-time quantitative PCR amplification of MHC polymorphisms  University of California, San Francisco |  |  |
| 1:50-2:10   | <b>Anna Bakardjiev:</b> Host defense mechanisms at the maternal-fetal interface <i>University of California, San Francisco</i>   |  |  |
| 2:10-2:30   | <b>Susan Fisher</b> : Maternal decidual macrophages inhibit NK cell killing of invasive cytotrophoblasts during human pregnancy <i>University of California, San Francisco</i>                   |  |  |
| 2:30-2:50   | Coffee Break   |  |  |

| Session III - Gene The Chair: Suzy Buckley | erapy Session will be held in HSW 300   |
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| 2:50-3:10                                  | Mort Cowan: Gene therapy for artemis-deficient severe combined immunodeficiency University of California, San Francisco           |
| 3:10-3:30                                  | <b>Simon Waddington</b> : Fetal gene and stem cell therapy – a skeptic's perspective <i>University College London</i>             |
| 3:30-3:50                                  | Ahad Rahim: Perinatal gene therapy rescues acute neonatal lethal neuronopathic Gaucher disease in mice  University College London |

| 3:50-4:10 | Citra Mattar: Fetal gene transfer in early gestation promotes stable expression with minimal immune toxicity and facilitates improved expression after postnatal challenge  National University of Singapore |
|-----------|--|
| 4:10-4:30 | Alice Tarantal: Translational nonhuman primate models for regenerative medicine and gene therapy: focus on the fetus and infant <i>University of California, Davis</i>                                       |
| 5:00-6:00 | Group Discussion (Part II) Reception- Stem Cell Board Room   |

| 6:00-7:30 | Reception – Stem Cell Board Room |
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|           |                                  |

# Millberry Union - City Lights Room Thursday 11:45-1:00 and Friday 12:00-1:15

## Development and Functional Competence of Fetal Dendritic Cells and Macrophages in Human Early-Mid Gestation

**Chan JKY**<sup>1,2</sup>, Shin A<sup>3</sup>, Low D<sup>1</sup>, Wasan PS<sup>3</sup>, Poidinger M<sup>3</sup>, Larbi A<sup>3</sup>, Collin M<sup>4</sup>, Choolani M<sup>2</sup>, Haniffa M<sup>3,4</sup> and Ginhoux F<sup>3</sup>

<sup>1</sup>Reproductive Medicine, KK Women's and Children's Hospital, Singapore <sup>2</sup>Yong Loo Lin School of Medicine, National University of Singapore, Singapore <sup>3</sup>Singapore Immunology Network, Agency for Science, Technology and Research (A\*STAR), Singapore <sup>4</sup>Institute of Cellular Medicine, Newcastle University, UK

## In Utero Depletion of Fetal Host Hematopoietic Stem Cells Improves Engraftment Following Neonatal Transplantation in Mice

**Derderian SC<sup>1,2</sup>,** Togarrati PP<sup>1,2</sup>, King C<sup>1,2</sup>, Moradi PW<sup>1,2</sup>, Czechowicz A<sup>3</sup>, Reynaud D<sup>1</sup>, Weissman I<sup>4</sup>, MacKenzie TC<sup>1,2</sup>.

<sup>1</sup>Eli and Edythe Broad Center of Regeneration Medicine and Stem Cell Research, <sup>2</sup>Department of Surgery, UCSF, San Francisco, CA, USA <sup>3</sup>The Department of Pediatrics, Boston Children's Hospital, Harvard Medical School, Boston, MA, USA. <sup>4</sup>Stem Cell Biology and Regenerative Medicine, Departments of Pathology and Developmental Biology, Stanford University School of Medicine, Palo Alto, CA

#### Maternal and Fetal T Cell Responses During Normal Pregnancy and Preterm Labor

**Frascoli M<sup>1, 2</sup>**, Coniglio L<sup>1, 2</sup>, Tang Q<sup>2</sup>, Gomez-Lopez N<sup>3</sup>, Romero R<sup>3</sup>, and MacKenzie TC<sup>1, 2</sup> <sup>1</sup>Eli and Edythe Broad Center of Regeneration Medicine, <sup>2</sup>Department of Surgery, UCSF, San Francisco, CA, USA, and <sup>3</sup>Perinatology Research Branch, NICHD/NIH/DHHS and Hutzel Women's Hospital, Detroit, MI, USA.

#### **Optimization of Vascular Niches to Increase Hematopoietic Engraftment**

Mokhatari S, Colletti E, Porada C, Almeida-Porada G

Wake Forest Institute for Regenerative Medicine, NC, USA

## Human Stem Cells from Early Chorion Differentiate into Podocytes and Improve Renal Glomerulopathy in Alport Mice

**Moschidou D<sup>1,3</sup>**, Patsia M<sup>1</sup>, Bou-Gharios G<sup>1</sup>, Pusey CD<sup>1</sup>, Fisk NM<sup>2</sup>, Cook HT<sup>1</sup>, David A<sup>3</sup>, De Coppi P<sup>3</sup> and Guillot PV<sup>1,3</sup>

<sup>1</sup>Imperial College London, London, UK.<sup>2</sup>University of Queensland, Brisbane, Australia. <sup>3</sup>University College London, London, UK.

## Cell Fusion Phenomena Detected after in Utero Transplantation of Ds-red-Harbouring Porcine Amniotic Fluid Stem Cells into EGFP Transgenic Mice

Peng SY<sup>1</sup>, Chen YS<sup>1, 2</sup>, Wang YH<sup>3</sup>, Lee HM<sup>1, 2</sup>, Cheng WT<sup>4,5</sup>, Wu SC<sup>4</sup>, Shaw S<sup>6,7</sup>

<sup>1</sup>Institute of Biotechnology, National Taiwan University, Taiwan <sup>2</sup>Department of Surgery, Hualien Armed Forces General Hospital, Taiwan <sup>3</sup>Department of Nursing, College of Health Sciences, Yuanpei University, Taiwan <sup>4</sup>Department of Animal Science and Technology, National Taiwan

University, Taiwan <sup>5</sup>Department of Animal Science and Biotechnology, Tunghai University, Taiwan <sup>6</sup>Department of Obstetrics and Gynaecology, Chang Gung Memorial Hospital at Linkou and Chang Gung University, College of Medicine, Taiwan <sup>7</sup>Prenatal Cell and Gene Therapy Group, Institute for Women's Health, University College London, London, UK

## Innate immune response to *Listeria monocytogenes* infection at the maternal-fetal interface **Rizzuto G**, Bakardjiev A

<sup>1</sup>Departments and Pathology and Pediatrics, Microbial Pathogenesis & Host Defense Program University of California, San Francisco

# Second and Third Trimester Human Amniotic Fluid Stem Cells Engraft After in Utero Transplantation in Immunocompetent Mice

Shaw SWS, Shangaris P, Pozzobon M, Piccoli M, Pipino C, Lee KH, Schiavo A, Maghsoudlou P, Lin J, De Coppi P and David A

Institute for Women's Health, University College London, London, London, United Kingdom;

Insitute of Child Health, University College London, London, London, United Kingdom; and Research Institute, Citta' della Speranza, Padua, Italy.

## Uterine integrity is required to maintain human fetal immunologic naiveté Snead A, Nguyen T, Yesayan M, Kahn DA

Department of Obstetrics & Gynecology, UCLA, Los Angeles, CA, USA

### **Tregs Prevent Production of Maternal Antibodies to Fetal Alloantigens**

**Marta Wegorzewska<sup>1,2,\*</sup>, Catherine Tsai<sup>1,2,\*</sup>,** Patriss Moradi<sup>1,2</sup>, Philip Norris<sup>3</sup>, Rachel Jackman<sup>3</sup>, Qizhi Tang<sup>2</sup>, and Tippi C. MacKenzie<sup>1,2</sup>

<sup>1</sup>Eli and Edythe Broad Center of Regeneration Medicine, <sup>2</sup>The Department of Surgery, University of California, San Francisco and <sup>3</sup>Blood Systems Research Institute, San Francisco, CA, USA