

THE LAM FOUNDATION  
2010 INTERNATIONAL RESEARCH CONFERENCE



SCIENTIFIC SCHEDULE

CONFERENCE CHAIR, FRANCIS X. MCCORMACK, MD  
BASIC SCIENCE CHAIR, ELIZABETH HENSKÉ, MD  
CLINICAL SCIENCE CHAIR, LISA YOUNG, MD

FRIDAY MORNING, APRIL 9<sup>TH</sup> – 7:30AM–12:00PM

TAFT I

SESSION I: BIOMARKERS AND IMAGING - *Session Chair: Cheryl Walker, PhD*

- 7:30 – 8:00 BREAKFAST/POSTER VIEWING
- 8:00 – 8:05 Frank McCormack, MD: Welcome
- 8:05 – 8:10 Comments and Patient Intro by Session Chair
- 8:10 – 8:15 Comments by Rich Schad, husband of LAM patient Karyn Schad
- 8:15 – 8:40 David Mankoff, MD, PhD: "Molecular imaging as a tool for bench-to-bedside research and therapy"
- 8:40 – 9:05 Bruce Kristal, BS, PhD: "Validated biomarkers of caloric restriction in rats: Markers of disease risk in humans?"
- 9:05 – 9:20 Jane Yu, PhD: "Evidence from expression profiling, proteomics, metabolomics and FDG-PET of an estrogen-dependent metabolic switch in tuberin-null cells"
- 9:20 – 9:45 Michael Welsh, MD: "A porcine model of Cystic Fibrosis exhibits lung manifestations of the human disease"
- 9:45 – 10:00 William Hardie, MD: "Inhibition of PI3K reverses transforming growth factor-alpha induced pulmonary fibrosis"
- 10:00 – 10:25 BREAK/POSTER VIEWING PRESIDENTIAL III
- 10:25 – 10:50 Kazunori Tobino, MD: "Imaging studies of LAM"
- 10:50 – 11:05 Jared Hagaman, MD: "Screening for lymphangioliomyomatosis with high-resolution CT in young, nonsmoking women presenting with spontaneous pneumothorax is cost-effective"
- 11:05 – 11:20 Masaki Hirose, PhD: "Serial measurement of serum vascular endothelial growth factor (VEGF)-D in patients with lymphangioliomyomatosis"
- 11:20 – 11:45 Cheryl Walker, PhD: "ATM signals to TSC2 in the cytoplasm to regulate mTORC1 in response to ROS"
- 11:45 – 12:00 Po-Shun Lee, MD: "TSC2 dependent up-regulation of COX2 and prostaglandin production in LAM"
- 12:00 – 1:00 LUNCHEON BUFFET/POSTER VIEWING PRESIDENTIAL III

FRIDAY AFTERNOON, APRIL 9<sup>TH</sup> – 1:00PM–6:00PM

TAFT

SESSION II: LUNG DESTRUCTION - *Session Chair: Greg Downey, MD*

- 1:00 – 1:05 Comments and Patient Intro by Session Chair
- 1:05 – 1:10 Comments by LAM patient Janice Burnham
- 1:10 – 1:35 Vera Krymskaya, PhD: "TSC2, mTORC2 and actin cytoskeleton: relevance to lung cyst formation"
- 1:35 – 2:00 Caroline Owen, MD, PhD: "Lung pathologies in LAM: Lessons from COPD"
- 2:00 – 2:25 Eric White, MD: "PTEN and the fibroblast in pulmonary fibrosis: a rationale for studies in LAM"
- 2:25 – 2:50 Danielle Morse, MD: "What can we learn about LAM from other chronic lung diseases?"
- 2:50 – 3:10 BREAK/POSTER VIEWING PRESIDENTIAL III
- 3:10 – 3:35 Akiko Hata, PhD: "MicroRNA regulation of smooth muscle phenotypes"
- 3:35 – 4:00 Franco Bonetti, MD: "LAM, PECOMAS, and cathepsin K"
- 4:00 – 6:00 POSTER DISCUSSION/WINE & CHEESE: PRESIDENTIAL III  
Drs. Greg Downey & Brendan Manning

**FRIDAY AWARDS BANQUET / DESSERT SOCIAL – 6:30PM–9:00PM****PRESIDENTIAL BALLROOM**

Jill Raleigh, Foundation Director

- 6:30 – 6:35 Welcome
- 6:35 – 7:15 Dinner
- 7:15 – 7:45 Keynote Speaker Elizabeth Cohen, CNN Senior Medical Correspondent
- 7:45 – 8:45 Award Presentations Sue Byrnes, Research Programs/Frank McCormack, MD, Scientific Director
- 8:45 – 9:00 Celebrating 15 Years of Progress Frank McCormack, MD, Scientific Director
- 9:00 – 10:00 DESSERT SOCIAL FOR ALL ATTENDEES **GIBSON FOYER**

**SATURDAY MORNING, APRIL 10<sup>TH</sup> – 7:30AM–12:00PM****TAFT****SESSION III: SIGNALING - *Session Chair: Kuniaki Seyama, MD, PhD***

- 7:30 – 8:00 BREAKFAST
- 8:00 – 8:05 Comments and Patient Intro by Session Chair
- 8:05 – 8:10 Comments by LAM patient Sue Heckman
- 8:10 – 8:35 Brendan Manning, PhD: "Loss of the TSC tumor suppressors reprograms cellular metabolism to promote proliferation"
- 8:35 – 9:00 Channing Der, PhD: "Role of Rheb in TSC pathogenesis and as a therapeutic target"
- 9:00 – 9:25 Arnold Kristof, MD: "Mammalian target of rapamycin controls the nuclear trafficking of signal transducer and activator of transcription-1 (STAT1)"
- 9:25 – 9:40 Marilyn Glassberg, MD: "Doxycycline reverses 17beta-estradiol mediated effects on estrogen receptor alpha and beta via PI3K pathway in LAM"
- 9:40 – 9:55 Elena Goncharova, PhD: "Combination of rapamycin and simvastatin abrogates TSC2-null tumor growth and prevents tumor recurrence"
- 9:55 – 10:15 BREAK
- 10:15 – 10:40 Joel Moss, MD, PhD: "Phenotypic characterization of disseminated cells with TSC2 loss of heterozygosity in patients with lymphangioleiomyomatosis (LAM)"
- 10:40 – 11:05 Kun-Liang Guan, PhD: "Regulation and function of the TSC-mTOR pathway"
- 11:05 – 11:20 Andrey Parkhitko, MS: "Role of autophagy in the pathogenesis of TSC and LAM"
- 11:20 – 11:35 Aristotelis Astrinidis, PhD: "Pharmacologic inhibition of polo-like kinase 1 (PLK1) decreases the viability and survival of TSC1 and TSC2 null cells via induction of autophagy"
- 11:35 – 12:00 David Kwiatkowski, MD, PhD: "Lymphangioleiomyomatosis (LAM): insights from PEComa and mutational analyses"
- 12:00 – 1:00 LUNCH **FOUNTAIN ROOM–LEVEL II**

**SATURDAY AFTERNOON, APRIL 10<sup>TH</sup> – 1:00PM–4:25PM****TAFT****SESSION IV: TRIALS - *Session Chair: Simon Johnson, MD***

- 1:00 – 1:05 Comments and Patient Intro by Session Chair
- 1:05 – 1:10 Comments by LAM patient Alanna Nelson
- 1:10 – 1:35 Bruce Korf, PhD, MD: "Developing a national collaborative network for clinical research in neurofibromatosis"
- 1:35 – 1:50 Professor Alfredo Gorio: "Genetic, molecular, and pharmacological advances in human TSC2 cells. The outcome of the Italian experience"
- 1:50 – 2:15 Carlyne Cool, MD: "Translational research from a lung tissue repository"
- 2:15 – 2:30 Young-Kwon Hong, PhD: "An exquisite cross-control mechanism among endothelial cell fate regulators directs the plasticity and heterogeneity of lymphatic endothelial cells"
- 2:30 – 2:55 Denise Adams, MD: "Vascular Anomalies and mTOR inhibition"
- 2:55 – 3:20 BREAK
- 3:20 – 3:35 Pedro Medeiros Jr., PhD: "Sleep hypoxemia in patients with LAM: relationship to pulmonary function and 6MWT"
- 3:35 – 4:00 Geraldine Finlay, MD: "Insights into therapeutic strategies from TSC animal models"
- 4:00 – 4:15 Justin Babcock: "Targeting mTOR-induced endoplasmic reticulum stress: a rational treatment for sporadic AML/LAM?"
- 4:15 – 4:40 George Retsch-Bogart, MD: "Cystic Fibrosis Foundation's Therapeutics Development Network: Insights for LAM"

**SATURDAY EVENING – BREATH OF HOPE GALA – 6:30PM–12:00AM****PRESIDENTIAL BALLROOM**

- 6:30 – 12:00 Dinner/Auction/Program/Entertainment

**SUNDAY MORNING, APRIL 11<sup>TH</sup> – 8:30AM–11:00AM****PRESIDENTIAL III**

- 8:30 – 11:00 Roundtable Discussions