

THE LAM FOUNDATION
2008 INTERNATIONAL RESEARCH CONFERENCE



WESTIN HOTEL - DOWNTOWN CINCINNATI
FRIDAY, APRIL 4 – SUNDAY, APRIL 6, 2008
SCIENTIFIC SCHEDULE

CONFERENCE CHAIR, FRANCIS X. MCCORMACK, MD
BASIC SCIENCE CHAIR, GEORGE THOMAS, PHD ~ CLINICAL SCIENCE CHAIR, RAY YEUNG, MD

FRIDAY MORNING, APRIL 4TH – 7:30AM–12:00PM

TAFT I & II– LEVEL III

SESSION I: TSC FROM MAN TO MOUSE

Session Chair: *Brendan Manning, PhD*

7:30 – 8:00 CONTINENTAL 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 LAM patient Kim Benedict

8:15 – 8:40 Joel Moss, PhD, MD: "Factors affecting LAM cell biology and disease pathogenesis"

8:40 – 9:05 Jeffrey Myers, MD: "The Pathology of LAM"

9:05 – 9:30 Pier Paolo Pandolfi, MD, PhD: "Aberrant TOR signaling in the pathogenesis of LAM/TSC and human cancer"

9:30 – 9:55 David Kwiatkowski, MD: "TSC and LAM: cell lines, mouse models, and therapeutic trials"

9:55 – 10:20 **BREAK/POSTER VIEWING**

PRESIDENTIAL III–LEVEL III

10:20 – 10:45 Brendan Manning, PhD: "Signaling defects triggered by loss of the TSC1- TSC2 complex"

10:45 – 11:00 Jane Yu, PhD: "Estrogen and MEK-dependent pulmonary metastasis of tuberin-deficient cells—a mouse model of LAM"

11:00 – 11:15 Gustavo Pacheco-Rodriguez, PhD: "Chemokine-enhanced chemotaxis of LAM cells with mutations in the tumor suppressor TSC2 gene"

11:15 – 11:30 Elena Goncharova, PhD: "Combined targeting of RhoA GTPase by simvastatin and mTOR/S6K1 by rapamycin abrogates TSC2-null tumor growth"

11:30 – 11:45 Primal de Lanerolle, PhD: "Role of Myosin Light Chain Kinase in LAM Cell Physiology"

11:45 – 12:00 Lyn Moir, PhD: "TSC2 modulates cell attachment to extracellular matrix proteins in pulmonary lymphangiomyomatosis"

12:00 – 1:00 **BOX LUNCH/POSTER VIEWING**

PRESIDENTIAL III–LEVEL III

FRIDAY AFTERNOON, APRIL 4TH – 1:00PM–6:00PM**TAFT I & II– LEVEL III****SESSION II: RELATED HAMARTOMA SYNDROMES****Session Chair: *David Kwiatkowski, MD***

- 1:00 – 1:05 Comments and Patient Intro by Session Chair
1:05 – 1:10 Comments by LAM patient Mary Brown
1:10 – 1:35 Robert Abraham, PhD: "Drugging the mTOR pathway for treatment of proliferative diseases"
1:35 – 2:00 Nancy Ratner, PhD: "NF1 signaling in MPNSTS"
2:00 – 2:25 Karen Cichowski, PhD: "TORC1 is essential for NF1- dependent malignancies"
2:25 – 2:50 Reuben Shaw, PhD: "AMPK control of mTOR signaling"

2:50 – 3:15 BREAK/POSTER VIEWING**PRESIDENTIAL III–LEVEL III**

- 3:15 – 3:40 Frank McCormack, MD: Past and Future Lung Trials for LAM
3:40 – 4:05 Laura Schmidt, PhD: "Birt-Hogg-Dube syndrome: molecular genetics, folliculin functional studies and mouse models"

4:05 – 6:00 POSTER DISCUSSION/WINE & CHEESE:
Cheryl Walker, PhD & Geraldine Finlay, MD

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

- 6:30 – 6:35 Welcome/Special Presentations Leslie Sullivan-Stacey, JD, President/CEO
6:35 – 6:45 Musical performance: String Quartet
6:45 – 7:40 DINNER
7:40 – 8:00 Keynote Speaker Mary Snow, BS, CNN Correspondent
8:00 – 8:20 Patient/Scientific Award Presentations Leslie Sullivan-Stacey, JD/Frank McCormack, MD
8:20 – 8:25 Musical performance: String Quartet
8:25 – 8:45 The Impact of patient advocacy on LAM science Frank McCormack, MD, Scientific Director
8:45 – 9:30 DESSERT SOCIAL FOR ALL ATTENDEES **GIBSON FOYER–LEVEL III**

SATURDAY MORNING, APRIL 5TH – 7:30AM–12:00PM**TAFT I & II–LEVEL III****SESSION III: LAM PROGRESSION****Session Chair: *Elizabeth Henske, MD***

- 7:30 – 8:00 CONTINENTAL BREAKFAST
8:00 – 8:05 Comments and Patient Intro by Session Chair
8:05 – 8:10 Comments by LAM patient Madeline Nolan
8:10 – 8:35 Jeffrey Whitsett, MD: "Cell lineages in lung development"
8:35 – 9:00 Andrew Folpe, MD: "PEComas: clinical and pathologic features"
9:00 – 9:25 Kari Alitalo, MD, PhD: "Molecular basis of lymphangiogenesis"
9:25 – 9:50 Kuniaki Seyama, MD, PhD: "Lymphangiogenesis in LAM"

9:50 – 10:10 BREAK

- 10:10 – 10:35 Yue Xiong, PhD: "WD40 protein 5BW5 promotes ubiquitination of tumor suppressor TSC2 by DDB1-CUL4-ROC1 ligase"
10:35 – 11:00 Ray Yeung, MD: "TSC, mTOR and cytoskeleton"
11:00 – 11:15 Cleo Bonnet, PhD: "Hamartin and tuberlin functionally co-operate with polycystin-1 and help maintain the structure of the primary cilium"
11:15 – 11:30 Brian Siroky: "Disruption of primary cilia and flow modulation of mTOR signaling in TSC2-deficient renal epithelial cells"
11:30 – 11:45 Aristotelis Astrinidis, PhD: "Role of TSC proteins in control of centrosome amplification, mitotic progression and cytokinesis"
11:45 – 12:00 Magdalena Karbowniczek, MD, PhD: "TSC1 and Rheb specify cell fate in drosophila, and regulate notch activity in LAM patient-derived cells"

12:00 – 1:00 LUNCH**FOUNTAIN ROOM–LEVEL II**

SATURDAY AFTERNOON, APRIL 5TH – 1:00PM–4:55PM

TAFT I & II–LEVEL III

SESSION IV: TARGETS AND THERAPEUTICS

Session Chair: *Nancy Ratner, PhD*

- 1:00 – 1:05 Comments and Patient Intro by Session Chair
- 1:05 – 1:10 Comments by Bruce Komarowski, husband of LAM patient
- 1:10 – 1:35 Elizabeth Henske, MD: "Rapamycin/TORC1-Independent targets of Rheb"
- 1:35 – 2:00 Jan-Ake Gustafsson, MD, PhD: "Antiproliferative actions of estrogen receptor beta - possible implications for LAM"
- 2:00 – 2:25 Cheryl Walker, PhD: "Dysregulation of cell cycle control in LAM"
- 2:25 – 2:50 David Plas, PhD: "Apoptosis control by tumor suppressors downstream of Akt/PKB"

- 2:50 – 3:10 **BREAK**

- 3:10 – 3:35 David Sabatini, MD, PhD: "mTORC1 versus mTORC2"
- 3:35 – 4:00 George Thomas, PhD: "Nutrient signaling in the mTORC1 signaling pathway"
- 4:00 – 4:25 Simon Johnson, MD: "TESSTAL trial of sirolimus in TSC and LAM"
- 4:25 – 4:40 Andrey Parkhitko, PhD: "Role of autophagy in the pathogenesis of TSC"
- 4:40 – 4:55 Blanco Camoretti-Mercado, PhD: "Modulation of tuberin and serum response factor association by TGF beta"

SATURDAY EVENING – BREATH OF HOPE GALA – 6:00PM–12:00AM

Gala Chair: *Laureen McCorkle*

- 6:00 – 7:00 Silent Auction
- 7:00 – 12:00 Dinner/Program/Dancing

GIBSON FOYER– LEVEL III
PRESIDENTIAL BALLROOM–LEVEL III

SUNDAY MORNING, APRIL 6TH – 8:30AM–10:30AM

PRESIDENTIAL III–LEVEL III

- 8:30 – 10:30 Gregory Downey, MD: Working breakfast to discuss future directions for LAM