

Overview: The Animal Model Summit will focus on overcoming the challenges of modeling LAM. Without a useful model for the disease, we struggle to scale-up efforts to explore therapeutic options in an effective and efficient way. At this meeting, we will bring together LAM experts with leaders in relevant fields to present those from outside the field with a comprehensive look at some of the approaches to modeling the disease that have been / are being attempted. We will map out next steps toward overcoming hurdles we face and learn how the LAM Treatment Alliance can concretely support these initiatives.

Date: April 9, 2008

Time: 10:00 AM – 4:00 PM

Location: Harvard Medical School, New Research Building, Room #258

SUMMIT DELEGATES:

- Myles Brown, MD, *Dana Farber Cancer Institute/ Harvard Medical School, USA*
- Catherine Butterfield, *Vascular Biology Program, Children's Hospital Boston/ Harvard Medical School, USA*
- Lewis Cantley, PhD, *BIDMC Division Signal of Transduction, BIDMC Cancer Center/ Harvard Medical School, USA*
- John (Sean) Clohessy, PhD, *BIDMC Division of Genetics/ Harvard Medical School, USA*
- Jennifer Cook, PhD, *Dana Farber Cancer Institute/ Harvard Medical School, USA*
- Sandra Dabora, MD, PhD, *Brigham and Women's Hospital/ Harvard Medical School, USA*
- Thomas Darling, MD, PhD, *Uniformed Services University of the Health Sciences, USA*
- Alfredo Gorio, PhD, *University of Milano, ITALY*
- Elizabeth Henske, MD, *Fox Chase Cancer Center, USA*
- Carla Kim, PhD, *Children's Hospital Boston Stem Cell Program/ Harvard Medical School, USA*
- David Kwiatkowski, MD, PhD, *Brigham and Women's Hospital/ Harvard Medical School, USA*
- Steven Niemi, DVM, *Massachusetts General Hospital/ Harvard Medical School, USA*
- Michael Nurok, MD, PhD, *Brigham and Women's Hospital/ Harvard Medical School, USA*
- Pier Paolo Pandolfi, MD, PhD, *BIDMC Cancer Center, Cancer/Genetics Program/ Harvard Medical School, USA*
- Craig Peacock, PhD, *Sidney Kimmel Comprehensive Cancer Center/ Johns Hopkins University, USA*

- David Sabatini, MD, PhD, *Whitehead Institute for Biomedical Research/ Broad Institute/ MIT Center for Cancer Research, USA*
- Sarah Short, PhD, *Vascular Biology Program, Children's Hospital Boston/ Harvard Medical School, USA*
- Cheryl Walker, PhD, *University of Texas MD Anderson Cancer Center, USA*
- Vicky Whittemore, PhD, *Tuberous Sclerosis Alliance, USA*
- Kwok-Kin Wong, MD, *Dana Farber Cancer Institute/ Harvard Medical School, USA*
- Sima Zacharek, PhD, *Children's Hospital Boston Stem Cell Program/ Harvard Medical School, USA*

SUMMIT AGENDA:

April 9, 2008 (10:00 AM – 4:00 PM)

- ▶ 10:00 AM – 10:05 AM **Summit Introduction**
 - ▶ 10:05 AM – 10:15 AM **Summit Mission - Delegate Introductions**
Pier Paolo Pandolfi, MD, PhD (Facilitator) & Summit Delegates
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SESSION I: Where We Are Now: Disease Theory and Animal Models of LAM

- ▶ 10:15 AM – 10:25 AM **Disease Theory Revisited & Implications for Treating LAM**
Elizabeth Henske, MD
- ▶ 10:25 AM – 10:35 AM **Efforts to Model LAM & Why It Matters**
David Kwiatkowski, MD, PhD
- ▶ 10:35 AM – 10:40 AM **What is the Litmus Test for a LAM Animal Model?**
Pier Paolo Pandolfi, MD, PhD
- ▶ 10:40 AM -11:05 AM **Model Presentation & Brief Discussion**
Sandra Dabora, MD, PhD

- ▶ 11:05 AM - 11:30 AM Model Presentation & Brief Discussion
Alfredo Gorio, PhD
- ▶ 11:30 AM - 11:55 AM Model Presentation & Brief Discussion
Craig Peacock, PhD
- ▶ 11:55 AM - 12:20 PM Model Presentation & Brief Discussion
David Sabatini, MD, PhD
- ▶ 12:20 PM - 12:45 PM Model Presentation & Brief Discussion
Thomas Darling, MD, PhD
- ▶ 12:45 PM - 1:10 PM Model Presentation & Brief Discussion
David Kwiatkowski, MD, PhD
- ▶ 1:10 PM - 1:35 PM Model Presentation & Brief Discussion
Elizabeth Henske, MD

▶ LUNCH BUFFET 1:35 PM – 1:45 PM

SESSION II. Where We Are Headed: Priorities Moving Forward

- ▶ 1:45 PM – 4:00 PM Making Sense of the Existing & In-Progress Models
 - The Litmus Test for a LAM Animal Model, Revisited
Pier Paolo Pandolfi, MD, PhD
 - Research Initiatives to Advance Critical Next Steps?
David Kwiatkowski, MD, PhD (Facilitated Discussion)