

PRINCIPLES OF THE SCIENTIFIC STRATEGIC PLAN

LAM research has moved forward with astounding speed in the past decade, due in large part to advances in the understanding of the tuberous sclerosis signaling pathways that are deranged in this disease. A wealth of promising molecular targets that are ripe for testing in clinical trials have been identified, many of which are addressable with drugs that are FDA approved for other indications. These drugs include, but are not limited to, mTOR inhibitors (e.g. sirolimus), farnesyl transferase inhibitors, specific estrogen antagonists, endothelin receptor antagonists (e.g. Bosentan), statins (e.g. simvastatin), tyrosine kinase inhibitors (e.g. Gleevec), metalloproteinase inhibitors, and angiogenesis inhibitors (e.g. Avastin).

LAM is in an uncommon research position among human diseases that requires innovative planning; we have a large body of cellular and molecular knowledge which we anticipate will continue to rapidly accumulate with funding from federal sources, no optimal cell or animal models, and a limited pool of patients available for trials. LAM Foundation research will therefore be focused on the development and validation of drug targets, prioritization of compounds for testing, and facilitation and implementation of phase I/II clinical trials. This strategy will require closer partnerships with industry and with the NCI.

The following principles will guide The LAM Foundation Research Program going forward:

- Research will remain the central mission of the Foundation.
- Our basic research funding will continue to be based on peer-reviewed, investigator-initiated research.
- Translational studies that help bring bench discoveries to the bedside are a priority, especially those focused on development of targets for LAM trials and studies that link pivotal findings in critical, parallel research arenas (e.g TSC) to LAM.
- Our clinical research goal is “a trial for every LAM patient and every LAM patient in a trial.” Research resources will be directed toward development of biomarkers and small phase I/II trials. A new Clinical Trials Board has been recruited to complement the existing Clinical Advisory Board.
- We remain committed to the development of LAM cell and animal models.
- Letters of intent will be required for submission of LAM Foundation research proposals, to ensure that all studies forwarded for review are aligned with the theme.
- Progress in LAM research will require free access to data, and to human tissue and reagents. We will continue to develop and facilitate systems to acquire and distribute tissue.
- The agenda for the annual LAM meeting will be realigned with this translational theme.
- We will increase resources available for research through vigorous fundraising, and partnerships with academia, industry, the NIH, and other foundations and organizations.
- We will develop systems to measure LAM research outcomes including LAM Foundation citations, attributions, awards, publications, funding successes, accomplishments and discoveries, and use these data to promote the Foundation research program.
- If funding allows, LAM Clinics will be developed in geographically dispersed locations. These centers will be partially funded by The LAM Foundation and will be resources for diagnosis, treatment and trials for LAM patients.