



The LAM Treatment Alliance *Announces* **Request for Proposals**

The LAM Treatment Alliance is accepting proposals for clinical research studies related to the treatment of patients with Lymphangioleiomyomatosis (LAM). LAM is a rare, currently untreatable, multi-system disease involving the lungs, kidneys and lymphatics and primarily affecting women in their reproductive years. Mortality is commonly due to respiratory failure associated with progressive cystic and nodular lung destruction. Lung transplantation is currently the only widely accepted option for women with this disease. There is variation in age of onset (mean age 34 yrs) and disease progression. LAM can occur as either a sporadic disease or in association with Tuberous Sclerosis Complex, a more common autosomal dominant disorder affecting males and females and multiple organs including the lung, brain, eyes, heart, kidneys and skin.

The LAM Treatment Alliance is a 501(c)3 not-for-profit medical research organization whose mission is to fast-track bench-to-bedside research dedicated to finding an effective treatment and/or cure for LAM. The purpose of this RFP is to encourage and fund experienced, motivated investigators to conduct clinical studies designed to improve the therapeutic options for women with LAM. This is a time-line driven, time-sensitive award focused on the feasibility of getting a well-designed, logical, therapy-focused, proof-of-concept study with a clear scientific rationale up and running by mid-2010. Studies with a strong translational component incorporating the exploration of biomarkers sensitive to treatment response are encouraged. The study will be conducted by study investigator(s) with maximum support from LTA-sponsored Contract Research Organization (CRO). Competitive scientific review and evaluation of preliminary and full proposals will include careful consideration of potential impact in fastest time (time-line, feasibility, protocol design, compound(s) selected, and parameters measured).

APPLICATION PROCEDURE

Protocol Concept Submission Deadline: November 6, 2009

- Please complete the attached Protocol Concept Submission Form
- Please include an NIH formatted biographical sketch of the principal and additional study investigators (including a bibliography of publications over the past three years)

Documentation should be sent via email to the LAM Treatment Alliance attention:

Jennifer Cook, PhD
Director, Research Operations
LAM Treatment Alliance
JCook@LAMTreatmentAlliance.org

Upon receipt of the above documentation, the LAM Treatment Alliance will evaluate all Protocol Concept Submission materials and notify investigators as to whether they are invited to submit a Draft Protocol Summary/ Detailed Protocol Synopsis as the next stage of the process.

Draft Protocol Summary/Detailed Protocol Synopsis

- Full Draft Protocol Summary/Detailed Protocol Synopsis invitations will be announced, via email, during the week of November 23, 2009. Draft Protocol Summary/ Detailed Protocol Synopses MUST be submitted within 4 weeks of invitation date; those submitting a full Draft Protocol Summary/Detailed Protocol Synopsis will receive \$1500.00 (USD) as compensation for their efforts.
- In addition to the Draft Protocol Summary/Detailed Protocol Synopsis, a budget outline must be included for the conduct of this study at your institution. This should include approximate start-up and close-out costs as well as estimated per patient investigator grants, with the understanding that these are cost estimates which the site will not be strictly held to and which are subject to change based on the final protocol.
- Competitive scientific review as well as feasibility assessments of the documentation provided will be conducted by the LAM Treatment Alliance.
- If your Draft Protocol Summary/Detailed Protocol Synopsis is selected for conduct by the LAM Treatment Alliance, the author(s) of the protocol summary will be the principal investigator(s) for the study.
- The study will be LTA sponsored with potential co-sponsors.
- For maximum efficiency, the LTA will actively work to select the best experience-matched CRO to help facilitate the process of transforming the protocol summary into a full protocol. The CRO will assist investigator(s) with additional site selection and qualification; collection and review of essential documentation; negotiation of contracts and investigator grants; assurance of compliance with human subjects protections/ regulations; conducting site monitoring, data management; statistics and report writing.

Materials should be sent via email to the LAM Treatment Alliance, attention:

Jennifer Cook, PhD
Director, Scientific Operations
LAM Treatment Alliance
JCook@LAMTreatmentAlliance.org

Upon receipt of the above materials, the LAM Treatment Alliance will evaluate them and notify investigators as to whether their protocol summary has been selected for clinical conduct. Compensation payments will also be made at that time.

Awarding of Grant

- Award announcements are expected to be made during the week of January 4, 2010.

Questions and Additional Information:

All questions and requests for additional information during this process should be directed to Jennifer Cook at JCook@LAMTreatmentAlliance.org

A cumulative question and answer log will be maintained by the LAM Treatment Alliance and shared with all participants throughout the process.