



LAM 101

**Hard to Pronounce ... Harder to Live With ...
Easy to Make a Difference**

What is LAM?

- **Lymphangioleiomyomatosis (LAM)** is a rare, fatal, multi-system disease affecting women in their childbearing years.
- LAM involves the lungs, kidneys (lesions called angiomyolipomas or AMLs) and lymphatics.
- LAM causes destruction throughout the body which is frequently attributed to migration and clustering of “LAM cells.”
- Over time, patients may progress to respiratory failure as cysts and nodules take over normal lung.
- The cause of LAM and the mechanism of lung destruction in LAM are poorly understood.
- One gene (TSC 2) has been identified in lung tissue, AMLs and circulating cells. Work to identify additional genetic and other changes involved in disease progression is now underway.

Who is affected?

- LAM affects women of all races, nations and economic backgrounds.
- Between 30,000 and 50,000 women worldwide are believed to have sporadic pulmonary LAM.
- Approximately 250,000 women worldwide have the form of LAM linked to a genetic disease called Tuberous Sclerosis Complex (TSC).
- LAM is more sex-specific than breast cancer.

What are common symptoms of LAM?

- Early symptoms of LAM may include shortness of breath, collapsed lung(s), chest pain, abdominal discomfort, and frequent coughing.
- Over time, women with LAM may experience complications due to leaky lymphatics (chylous or pleural effusions), become less active and require supplemental oxygen full-time.
- LAM is commonly **mis-diagnosed** as asthma, emphysema or bronchitis.
- LAM is **under-diagnosed** as it requires a high-resolution CT scan for a diagnosis. There is increasing thought that otherwise healthy, non-smoking women with one or more pneumothoraces should have a CT scan to rule out LAM.

Prognosis?

Course is variable: Women with LAM can progress to respiratory failure in less than two years or more than 20 years.

How is LAM managed?

- No therapy has been proven to effectively slow or stop disease progression.
- A few experimental medicines are now being investigated as possible treatments. (MILES Trial – rapamycin; RAD001)
- Current management practices are focused on relieving symptoms and preventing complications in the multiple systems and organs affected by LAM.
- Supplemental oxygen use is common among women with LAM.
- Lung transplantation, although quite risky, is currently the only life-extending option for LAM patients.

What are key barriers and opportunities when it comes to effectively treating and curing LAM?

- The LAM Treatment Alliance (LTA) is aggressively funding a diverse stream of strategic research priorities: The need for biomarkers with faster read-outs than pulmonary function tests is a critical priority in LAM research that will accelerate the ability to conduct small, fast clinical trials in humans. Gaining robust insight into LAM genomics and a greater understanding of the mechanism of lung destruction in LAM are also vital.
- The LAM Treatment Alliance is committed to aggressively driving development of community resources and tools to accelerate LAM research. These tools include supporting an effective data ecosystem, a transparent and efficient LAM tissue program, development of a LAM cell line and an animal model that recapitulates the disease and/or serves as an efficient bioreactor for growing cells.

What is the LAM Treatment Alliance (LTA)?

- The LAM Treatment Alliance (LTA) is a Boston-based non-profit working through global partnerships with patients, academic researchers, foundations, government institutions, biotech and pharmaceutical companies to aggressively drive collaborative treatment research for patients living with LAM today.
- The LTA works through seminars, summits, ad hoc meetings, grants, contracts and clinical care-focused partnerships to grow and foster community and strategically fund high-impact research.
- The LTA is guided by a Nobel-caliber Scientific Advisory Board (SAB) including: Robert Langer, ScD (Chair); Lewis Cantley, PhD; George Demetri, MD; Jan-Ake Gustafsson, MD, PhD; Sten Lindahl, MD, PhD; and Steve Shapiro, MD.
- The LTA's patient-centered approach to fast-tracking medical research has been applauded by *The Wall Street Journal*, *The New York Times*, the *Charlie Rose Show* and others.
- To learn more about the LAM Treatment Alliance's approach to advancing LAM research, go online to www.CureLAM.org.

What can researchers and physicians do to help accelerate LAM Research?

Get involved in LAM research:

- Email the LAM Treatment Alliance about your interest in getting involved: afarber@LAMTreatmentAlliance.org.
- Attend or participate in the monthly LAM/TSC Seminar Series remotely via live broadcast from Boston (www.LAMTSCSeminarSeries.org) by sending your email address to info@LAMTreatmentAlliance.org.

Help increase awareness in the Medical/Research Community:

- Contact the LAM Treatment Alliance if you would like to participate in our international LAM Speakers' Bureau.
- Learn more about LAM and help increase the number of accurate diagnoses.
- Contribute to LAM data ecosystem / International LAM Registry: info@LAMTreatmentAlliance.org for info
- Foster patient participation in advancing research by directing patients to www.CureLAM.org and to www.LAMsight.org

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