

LAM Foundation Media Kit

Overview

The LAM Foundation is the global leader in the fight against LAM (lymphangiomyomatosis), a rare lung disease that strikes young women, often in the prime of their lives.

The New England Journal of Medicine cites The LAM Foundation as “a model for patient advocacy.” The National Heart, Lung, and Blood Institute calls The LAM Foundation “a model for voluntary health agencies.”

LAM is one of 7,000 known rare diseases, and one of only 500 rare diseases with a treatment. LAM’s first treatment, the drug Rapamune, was approved in 2015 after a strikingly efficient global effort. Led by The LAM Foundation, the effort included researchers, clinicians, and government health agencies.

It also included hundreds of LAM patients who, in this and many others LAM initiatives, have tirelessly given of themselves to raise money for research, serve as advocates, support one another, and volunteer as research subjects—all in spite of a disease that progressively destroys their ability to breathe.

“If that isn’t altruism and courage, I don’t know what is,” said Dr. Francis McCormack, M.D., Director of the Division of Pulmonary, Critical Care and Sleep Medicine at the University of Cincinnati School of Medicine, and widely considered the world’s leading LAM researcher.

Research funded by The LAM Foundation has also **proven central to understanding other, more common diseases such as cancer, diabetes and obesity**. Still, The LAM Foundation’s work is far from complete, and **much remains to be done**, including:

- Maximizing the effectiveness of the current treatment, Rapamune, (related to optimal dosage and long term effectiveness) while continuing the search for a cure.
- More clinical trials are needed to determine if there are combination or alternate therapies for the treatment and cure of LAM.
- An untold number of women with LAM remain undiagnosed; we need to find them and shorten time to diagnosis in the U.S. and globally.
- Great opportunities exist for The LAM Foundation to serve as a model for other rare disease organizations, and **to share knowledge to propel treatments and cures in rare and common diseases**.

More on LAM Disease

With LAM, a patient's lungs are invaded by mutant smooth muscle cells, which dissolve healthy lung tissue and replace it with innumerable air-filled holes or cysts. Debate is ongoing about the links between LAM and cancer.

LAM is often misdiagnosed. Early symptoms may resemble asthma, emphysema, or bronchitis. Chest x-rays may look normal. The most useful imaging test for diagnosing LAM is a high resolution CT-Scan, which is expensive and usually not the first test ordered by physicians, especially physicians unfamiliar with LAM.

A LAM diagnosis once meant certain lung failure leading to a lung transplant or death. Now, LAM patients' outlook often centers on the word "hope." In great part, this is due to The LAM Foundation's strategy of "relentless collaboration" with researchers, clinicians, patients and government health agencies around the world.

More on Rare Diseases

There are 7,000 known rare (or orphan) diseases. A rare disease is one that affects a small percentage of the population. In the U.S., a rare or orphan disease is a disease or condition that affects less than 200,000 people. Collectively, rare diseases are a major problem—one in 10 Americans is affected by a rare disease, with the majority of them being children.

In the case of LAM, 1,400 American women and 3,500 globally have been diagnosed. Researchers estimate that at least 250,000 women worldwide have LAM, the vast majority of whom remain undiagnosed.

Of the 7,000 rare diseases, LAM is one of only 500 with a treatment, but still no cure.

Progress to find treatments and cures for rare diseases is typically slow because populations are small, research is often ignored, and costs to develop treatments and cures are unlikely to be recovered.

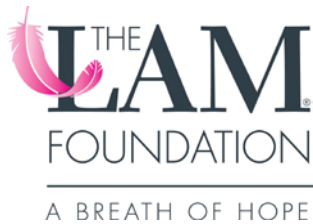
Investment in LAM and rare disease research is vital, not only for patients, but also because such research contributes to discoveries in other diseases such as cancer and diabetes.

More on the LAM Foundation

Sue Byrnes and her husband Fran founded The LAM Foundation in 1995 in Cincinnati, Ohio. The couple's daughter, Andrea, had been diagnosed with LAM the year before following several misdiagnoses.

Key accomplishments for The LAM Foundation include:

- Byrnes and followers convinced the National Heart, Lung, and Blood Institute (NHLBI) to establish a National LAM Registry and to initiate an intramural research program, a \$20 Million investment.
- The LAM Foundation developed a system to acquire and distribute LAM tissues obtained at transplantation for research, which has evolved into a large tissue repository at the National Disease Research Interchange (NDRI).



- The LAM Foundation has raised more than \$20 million, primarily by women suffering with LAM and their families, and has directed the vast majority of those funds toward research and patient advocacy.
- Seed investments by The LAM Foundation have resulted in LAM scientists receiving over \$40 Million in subsequent awards from the National Institutes of Health (NIH), Department of Defense, and other funding sources.
- The LAM Foundation funded research that provided the basis for the first ever LAM treatment trial (MILES). Results from MILES subsequently played a pivotal role in the 2015 U.S. Food and Drug Administration approval of the drug Rapamune (sirolimus) for the treatment of LAM— *the first drug approved to treat the disease.*
- The LAM Foundation also funded research that advanced knowledge of the mTOR pathway (important in regulating the cell division cycle) *that has proven central to understanding other, much more common diseases such as cancer, diabetes and obesity.*
- The LAM Foundation spearheaded the development of [31 domestic LAM clinics](#) and [24 international LAM clinics](#) to deliver coordinated diagnosis, care, and cooperative research.
- The Foundation organizes and executes an annual International LAM Research Conference. Co-funded by the National Heart, Lung, and Blood Institute, the annual conferences typically draw 300 attendees, including an average of 100 scientific investigators.

Resources

For more information about The LAM Foundation, or to arrange interviews, contact:

Sue Sherman, Executive Director, at ssherman@thelamfoundation.org or at 513.777.6889.

[LAM Foundation Awareness Video](#) – This short video provides an excellent summary through the eyes of patients, physicians, researchers and advocates. The page also has links to other videos about LAM and LAM Research.

[The LAM Foundation](#) – Foundation Website