Ongoing Clinical Trials & Studies

Study of the Disease Process of LAM

Joel Moss, MD, PhD, National Institutes of Health

This study is designed to determine the disease processes involved at the level of cells and molecules to develop more effective therapies for LAM. Researchers intend to identify the proteins and genes that contribute to the process of lung destruction in affected individuals.

If you are interested in learning more please contact:
Nora Quade, RN
Research Nurse Specialist, National Institutes of Health
(301)-496-3632 (o),
nora.quade@nih.gov
Study sites: Bethesda, MD
Visit https://go.usa.gov/xuuz9 for more details.

Helen Green Research Travel Grant

Patients who are participating in the above study at the NIH may be eligible to apply for a *Helen Green Research Travel Grant* to cover travel expenses. To learn more about the grant and the guidelines, contact Patient Services at **patientservices@thelamfoundation.org** or (513) 777-6889.

National Disease Research Interchange (NDRI)

NDRI's Private Donor Program gives individuals and their families an opportunity to provide an invaluable resource for researchers working to discover and advance new treatments or cures. A variety of tissue samples from women with LAM, including lung, kidney, uterus, blood and chyle fluid are currently needed by LAM researchers. If you are having surgery and would be willing to make a tissue donation or would like to learn more about the tissue donation process, please contact Patient Services at **patientservices@thelamfoundation.org** or (513) 777-6889.

Currently Enrolling Clinical Trials & Studies

Discovery of Sirolimus Sensitive Biomarkers in Blood

Joel Moss, MD, PhD, National Institutes of Health

This study is an observational study designed to determine if blood and urine markers after 1 dose and again after 3 months can be used to evaluate the correct dose of sirolimus for people with LAM. Women ages 18-90 with LAM whose doctors have decided they should start taking a 2 mg dose of sirolimus to treat it and who are able to travel to NIH are eligible to participate.

If you are interested in learning more, please contact:
Nora Quade, RN
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(301)-496-3632 (o),
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Currently Enrolling Clinical Trials & Studies

Bronchodilator Effects of Nebulized Versus Inhaled Albuterol In Subjects With Lymphangioleiomyomatosis

Angelo M Taveira-DaSilva, MD, National Heart, Lung, and Blood Institute (NHLBI)

LAM is a progressive lung disease that usually strikes women during their childbearing years, usually between the onset of puberty and menopause. Doctors at the National Institutes of Health (NIH) are conducting a research study to compare two methods, albuterol (study drug) given in metered dose inhaler and sprayer, and to determine which of these two methods best improves lung function in women with LAM.

About the Study

- Participants will have a physical exam and medical history
- 3-day overnight stay our hospital (NIH Clinical Center)
- Participants will take albuterol using a hand held inhaler and nebulizer during their hospital stay

Who can participate?

- Adult women who have impaired lung function because of LAM

AND

- With no known allergy to albuterol

If you are interested in learning more, please contact:
Nora Quade, RN
Research Nurse Specialist, National Institutes of Health
(301)-496-3632 (o),
nora.quade@nih.gov
Study sites: Bethesda, MD
Visit https://go.usa.gov/xE9rK for more details.

Multi-Center International Durability and Safety of Sirolimus in LAM (MIDAS) Trial

Francis X. McCormack, MD, University of Cincinnati Medical Center

The MIDAS trial continues to recruit patients eager to contribute to LAM research. This study aims to follow women with LAM who are currently taking, have previously failed or been intolerant of, or are considering treatment with mTOR inhibitors sirolimus or everolimus as part of their clinical care. All patients with LAM are eligible. Women with TSC over the age of 18 are eligible whether lung cysts are present are not. There are currently 17 participating LAM clinics with more to come. Over 150 patients have signed consent with the University of Cincinnati with the intention to eventually transfer to their LAM clinic of choice.

If you are interested in learning more please contact:
Sue McMahan
LAM Research Program Manager**,** University of Cincinnati College of Medicine
(513) 558-4376 (o)
susan.mcmahan@uc.edu

Study sites: Palo Alto, CA, Denver, CO, Jacksonville, FL, Atlanta, GA, Chicago, IL, Boston, MA, Ann Arbor, MI, Rochester, MN, St. Louis, MO, Rochester, NY, Cincinnati, OH, Cleveland, OH, Philadelphia, PA, Charleston, SC, Nashville, TN, Dallas, TX, Houston, TX, Salt Lake City, UT, Seattle, WA, Portland, OR.
Visit https://clinicaltrials.gov *and search for study number* ***NCT02432560.***

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Currently Enrolling Clinical Trials & Studies

Multicenter Interventional LAM Early Disease (MILED) Trial

Francis X. McCormack, MD, University of Cincinnati Medical Center

The MILED Trial is a new research study designed to answer the question of whether we should be starting sirolimus at low doses earlier in the course of disease, before symptoms develop and while lung function is still normal, similar to the manner in which we treat diabetes and high blood pressure early to prevent future heart and kidney complications.

You may be eligible if you:

- Are an adult woman with LAM
- Have an FEV1 greater than 70% predicted
- Are not currently taking sirolimus

During the study, participants will:

- Attend 8 study visits over 2 years (about one visit every 4 months)
- Complete blood tests, a physical exam, and pulmonary function tests at visits
- Answer questions about breathing, fatigue and quality of life
- Take one pill every day (1 mg sirolimus or a sugar pill) throughout the study
- Record their pill taking and any side effects in an electronic diary

Participants will receive:

- Physical exams, pulmonary function tests, a chest x-ray, and laboratory tests free of charge
- Study drug (either 1 mg sirolimus or placebo) throughout the study
- Reimbursement for travel expenses to attend each study visit

If you are interested in learning more please contact:

Sue McMahan
LAM Research Program Manager, University of Cincinnati College of Medicine
(513) 558-4376 (o)
susan.mcmahan@uc.edu

Study sites: Palo Alto, CA, Denver, CO, Atlanta, GA, Chicago, IL, Boston, MA, Cincinnati, OH, Philadelphia, PA, Nashville, TN.

Visit https://clinicaltrials.gov** and search for study number **NCT03150914.

Resveratrol and Sirolimus in LAM Trial (RESULT)

Nishant Gupta, MD, & Francis X. McCormack, MD, University of Cincinnati Medical Center

Marina K. Holz, PhD, Yeshiva University

This is an open-label, phase II study of escalating doses of resveratrol in patients on a stable dose of sirolimus. Twenty-five patients will be enrolled. The primary endpoint will be to determine if there is a change in serum VEGF-D after 24 weeks of combined resveratrol and sirolimus as compared to the VEGF-D level in sirolimus alone. Secondary endpoints include PFTs, QOL assessments, safety and adverse effect profiles. Key inclusion criterion: LAM patients on sirolimus for at least 20 weeks, with stabilization of VEGF-D levels post sirolimus as tested by at least 2 lab draws at least 12 weeks apart. Total trial duration is 3 years.

If you are interested in learning more please contact:

Becky Ingledue
Clinical Research Coordinator, University of Cincinnati College of Medicine
(513) 558-0027 (o)

Rebecca.ingledue@uc.edu

Study site: Cincinnati, OH

Visit https://clinicaltrials.gov** and search for study number **NCT03253913.

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