

Biomarkers for LAM

GOAL of Workshop:

1. Develop novel biomarkers to assist in non-invasive diagnosis of LAM.
2. Develop novel measures and tools to optimize treatment dosing as well as track disease stage / progression and treatment response in patients with LAM.
3. Develop a prognostic severity scale specific to LAM.
4. Define disease-specific phenotypes that may be associated with differential rate of disease progression and treatment response.
5. Develop alternate surrogate biomarkers beyond lung function to serve as end points in clinical trials studying the efficacy of novel treatment options in LAM.

A biomarker is anything that indicates a patient's medical state. A biomarker should be able to be measured accurately and reproducibly.

Project ideas and focus areas likely to move forward from this workshop:

1. Evaluate the use of VEGF-D levels to predict response to treatment and disease progression.
2. LAM Share. Start with the data that is out there, figure out how to link patients across studies, and share datasets. Patients are participating in multiple biorepositories, clinical trials, and surveys. The scientific community could make more rapid advances if we were able to share our data, especially since so many of these patients are in more than one trial.
3. Produce a LAM risk calculator and search for predictive, prognostic, surrogate biomarkers.
4. Imaging - the use of imaging as a diagnostic and prognostic biomarker. Imaging low-dose CT scans can show the amount of the lung impacted by cysts. Novel PET imaging could add to our understanding of disease, but radiotracers need validated.
5. Clinical trial design and evaluation - Use of trial designs beyond the randomized placebo-controlled trial was posted on the board. N of 1 trials, where a patient serves as her own control, or factorial analyses were mentioned. A better understanding of patient variation, and choosing patients with extreme discordant phenotypes, would enable us to draw statistically-valid conclusions from smaller sample sizes.
6. Single Cell RNA Sequencing (ssRNAseq)
7. Menstrual cycle, estrogen, and LAM:
8. Nutrition: the role that nutrition can play in health and disease progression.
9. Home Spirometry - We accept FEV1 as a biomarker of lung stability or disease progression. Could more frequent testing, via a home spirometry technique, give us more timely information about disease stabilization or progression?