



Potential Diagnosis And Treatment For LAM Lung Disease

Researchers from Cincinnati Children's Hospital Medical Center and University of Cincinnati College of Medicine reported on a study testing the drug sirolimus in patients with LAM or tuberous sclerosis complex (TSC) with angiomyolipomas, benign kidney tumors common to both diseases. Approved to help transplant patients fight organ rejection, sirolimus treatment resulted in a 50 percent reduction in tumor growth; a significant improvement in lung function was observed in LAM patients.

In addition, a letter published in the same issue of NEJM reports on preliminary data to support the use of a serum marker test to confirm a diagnosis of LAM. The disease has traditionally required a lung biopsy or CT scan for confirmation of diagnosis, contributing to diagnosis complications.

"These studies represent significant advances for LAM patients," said Leslie Sullivan-Stacey, J.D., President and CEO of The LAM Foundation, a supporter of both studies. "The LAM Foundation has been the driving force behind major breakthroughs in LAM research over just the last decade, and we now have scientific evidence to support further study of treatments and diagnostic tools. The sirolimus study already is serving as the basis for other studies in TSC and LAM, including the first-ever LAM treatment trial, now enrolling patients."

In addition to these U.S. studies, a second letter to the editor from researchers in the United Kingdom reports on a Phase II study of sirolimus in patients with TSC and sporadic LAM. An editorial authored by Drs. Elahna Paul and Elizabeth Thiele of Massachusetts General Hospital in Boston, expresses enthusiasm and caution in the interpretation of the sirolimus research.

Researchers say that if validated as a biomarker, the serum test may improve the ability to conduct trials more quickly. Furthermore, identification of a biomarker for LAM may have treatment implications for other diseases with similar pathways that affect millions of Americans, including breast cancer, diabetes, obesity and even autism.

The studies are published in the Jan. 10 edition of the New England Journal of Medicine.

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