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Clinical trial examines drug combo as remission-inducing treatment options for LAM

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Researchers at the University of Cincinnati (UC) begin a new clinical trial this fall, examining the potential role of a drug combination therapy to eliminate lymphangioleiomyomatosis (LAM) cells. The trial will look at the safety and efficacy of a combined therapy using sirolimus and resveratrol as a potential remission-inducing treatment option for patients with LAM.

Sirolimus is a drug approved by the Food and Drug Administration that suppresses cell growth in LAM patients and is currently the first-line treatment option for most LAM patients. Resveratrol is a naturally occurring chemical found in the skin of grapes used to make red wine.

"The next phase in the treatment for LAM is to identify a regimen that will induce disease remission, not just suppression," says Nishant Gupta, adjunct assistant professor in the Division of Pulmonary Critical Care in the Department of Internal Medicine at the UC College of Medicine and the principal investigator of the study. "Resveratrol is a very safe drug, it has been used in multiple human trials before and is well tolerated with very few side effects."

Pre-clinical research led by Marina Holz, PhD, Doris and Ira Kukin Chair in Biology at Yeshiva University, demonstrated that the combination of sirolimus and resveratrol can kill LAM cells.

LAM is a rare but serious lung disease that occurs primarily in women of childbearing age. LAM is a low-grade tumor in which the abnormal tumor cells grow out of control and spread to restricted areas in the body, including the lungs, kidneys, lymph nodes, blood vessels and lymphatics.

"The laboratory science behind this drug combination is very strong," says Frank McCormack, MD, professor, Department of Internal Medicine and director of the Division of Pulmonary Critical Care and Sleep Medicine at the UC College of Medicine. "There is no good animal model for LAM, so all the discoveries that are directly relevant to patients have to come from humans until a better model is discovered. We learn a lot from the cells and the animal models that we have, but none of them are good enough to bypass a human study."

"I am thrilled that this grant will allow us to rapidly build upon the basic and preclinical studies that started in my lab in 2014 and led to a synergistic collaboration with our clinical partners at UC, and our industry partner Evolva, the provider of resveratrol," says Holz. "This grant will allow us to make substantial progress towards validating new therapeutic options for treatment of LAM, and will serve as a model of cross-disciplinary collaboration and rapid implementation of future clinical trials."

Gupta hopes to start enrolling study participants in October and is confident he will get a positive response.

"The LAM patient community is one of the best patient communities to work with because they are very forthcoming with respect to research, and are always willing to contribute towards scientific discovery," he says. "This is a model for how other diseases could organize their communities and work towards attaining better outcomes."