

להלן תמצית תוצאות המחקר בתרופת הרפאמיצין

בחולות עם LAM

What do the final results mean for women with LAM?

What the final results mean for women with LAM is that sirolimus is effective in stabilizing lung function in selected patients with moderately severe lung impairment due to LAM. There were more side effects in the sirolimus group than in the treatment group.

Do the final results mean that all LAM patients should be taking sirolimus?

No. This is a drug which can produce significant side effects. Not every LAM patient should receive sirolimus. Women should discuss the use of sirolimus with their pulmonologist or with the pulmonary specialist at a LAM Clinic.

Does sirolimus treatment last?

In the MILES study, the effect of sirolimus only persisted while the drug continued. After sirolimus was stopped, the lung function of the LAM patients in the study declined at a rate that was similar to the placebo group.

How long should sirolimus continue in a patient with LAM?. We do not know the answer to this question, since the treatment period in MILES was only one year. This will be a question for future studies.

Were there other changes in the study after treatment with sirolimus?

Yes, LAM patients treated with sirolimus during the study experienced improvement in some measures of quality of life and functional performance. They did not experience improvement in the distance that they were able to walk, however, and measurements of gas exchange (also known as diffusing capacity or DLCO) did not appear to change. Also, the serum VEGF-D level decreased in LAM patients during sirolimus treatment. Serum VEGF-D is a protein that is elevated in the blood of most LAM patients. It is useful for making a diagnosis of LAM, but it is not yet clear if the VEGF-D level correlates with rate of progression of lung disease or response to therapy.

What were the most common side effects of sirolimus?

The LAM patients in the study who took sirolimus had more side effects than the women who were on placebo. The most common side effects experienced by LAM patients who were on sirolimus were: mouth ulcers, diarrhea, nausea, increased cholesterol levels, acne like rash, and leg and foot swelling. There was no difference in the incidence of serious side effects between the groups, however.

In general, the side effects of sirolimus only lasted while the drug continued.

How much sirolimus did the LAM study patients take on a daily basis?

The daily dosage of sirolimus averaged about 2 mg per day (two 1 mg sirolimus tablets).

Some patients required more, and some patients required less. The serum levels of sirolimus were measured and the dosage was adjusted to maintain the drug levels in the blood in the therapeutic range of 5-15 ng/ml

¹ בישראל חלק מהחולות עם LAM משתמשות בסרטיקן (everolimus) וחלק ברפאמיצין (Sirolimus). התרופות הן מאותה משפחה ודומות בהשפעתן.