SAFETY AND EFFICACY OF SARACATINIB IN SUBJECTS WITH LYMPHANGIOLEIOMYOMATOSIS (LAM) [SLAM-2]

Why is this research being done? This study is being done to find out what is the right dose of a drug called saracatinib that is best tolerated by women with LAM. It is also being done to see how well saracatinib works in LAM. This drug has been tested in subjects with certain types of cancer but is not currently approved by the United States Food and Drug Administration (FDA). Saracatinib may work in cancer by preventing the growth, movement and invasiveness of cancer cells. The investigators hope that it might work in LAM by slowing the growth and movement of LAM cells. The use of saracatinib to treat LAM is considered experimental.

What is the purpose of Phase 2 of this study? Saracatinib is being investigated as a possible new treatment for LAM. This study is being done to evaluate safety and efficacy on 125mg of saracatinib in subjects with LAM.

Who is conducting the research study? A group of institutions led by Baylor College of Medicine conducts this research study. The study is supported by the National Institutes of Health. The drug saracatinib is provided by AstraZeneca a drug manufacturing company. This study is being done in Massachusetts, Illinois, Ohio, Texas and California.

Who will be included in this study? Women 18 years and older who have LAM.

How long will you be in the research study? You will be in the research study for up to 12 months. Study drug will be given for up to 9 months.

What is involved in the research study? You will be 125 mg saracatinib for up to nine months.

Study Visits: The study schedule will include about 7 visits

Study Procedures (what will happen to you): below is a brief outline Medical History and Physical Examination Questionnaires Chest X-rays, Pulmonary Function tests, 6-minute walk test, Blood draws, Urine analysis, Urine pregnancy test, Bone density studies, CT of chest and MRI of chest.

Are there direct benefits to taking part in the research study? If you agree to take part in this research study, there may not be a direct medical benefit for you. You may benefit from the physical exams, pulmonary function tests, and other study procedures. The information learned from this research study may benefit other patients with LAM in the future. Saracatinib may or may not stabilize or improve lung function.

What are the potential risks and discomforts of the research study? There may be some risks and discomforts, including potentially serious risks, involved with participation in the study. Saracatinib has been used in many clinical trials and its safety profile is well understood. A detailed list of possible side effects will be provided to those interested in knowing more about this study.

Will I get all the facts about the study? Those who are interested in participating will be given a consent form that thoroughly explains all of the details of the study. The form covers all of the procedures, the potential risks and benefits, who to contact with questions or concerns and more. A member of the study staff will review the consent form with participants to ensure all questions are answered. Study procedures will not begin until this form is signed.

Who should I contact for more participant information?

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