# Sirolimus Multicentre International Lymphangioleiomyomatosis Efficacy and Safety trial (the MILES trial): Canadian component

Submission date Recruitment status Prospectively registered 04/02/2008 No longer recruiting [ ] Protocol [ ] Statistical analysis plan Registration date Overall study status 04/02/2008 Completed [X] Results [ ] Individual participant data Last Edited Condition category 29/10/2013 Cancer

Plain English summary of protocol

Not provided at time of registration

## Contact information

Type(s)

Scientific

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## Additional identifiers

ClinicalTrials.gov (NCT) NCT00414648

Protocol serial number MCT-83051

## Study information

#### Scientific Title

Sirolimus Multicentre International Lymphangioleiomyomatosis Efficacy and Safety: Canadian component - a placebo-controlled, randomised, parallel group trial

#### Acronym

**MILES** 

#### **Study objectives**

- 1. Implement a double-blind, placebo-controlled, 'intention to treat' based, multicentre protocol for the determination of the safety and efficacy of sirolimus in patients with lymphangioleiomyomatosis (LAM)
- 2. Determine the correlation between changes in lung function and questionnaire-based assessments of dyspnoea, quality of life, fatigue, and degree of health impairment in LAM trial patients who are taking sirolimus or placebo

#### Ethics approval required

Old ethics approval format

#### Ethics approval(s)

- 1. Research Ethics Board of University Health Network, Toronto (Canada) approved on 07/12/2007 (ref: 07-095-A)
- 2. Institutional Review Board of Niigata University Medical and Dental Hospital (Japan) approved on the 24/07/2007 (ref: NH19-003)
- 3. Institutional Review Board (Federalwide Assurance # 00002988) of Cincinnati Childrens Hospital Medical Center, Cincinnati (USA) approved on 11/01/2008 (ref: 04-11-17)

Ethics approval from Australia pending.

#### Study design

International, multicentre, placebo-controlled, randomised, parallel group trial

#### Primary study design

Interventional

#### Study type(s)

Treatment

#### Health condition(s) or problem(s) studied

Lymphangioleiomyomatosis (LAM)

#### **Interventions**

This is a multicentre, international, intention to treat, placebo-controlled, randomised parallel, drug/diagnostic, efficacy and safety trial with study participant, study investigator, caregiver, outcome assessor, and data analyst blinded.

- 1. Experimental group: sirolimus (rapamycin), daily dose of 2 mg during 12 months; follow up only for additional 12 months
- 2. Control group: matching placebo of active treatment, 1 pill daily for 12 months; follow up only for additional 12 months

Contact for public queries same as below. Please note that you can also contact Dr Lianne Singer at lianne.singer@uhn.on.ca for details on this trial (scientific and public queries).

#### **Intervention Type**

Drug

#### Phase

Not Applicable

#### Drug/device/biological/vaccine name(s)

Sirolimus (rapamycin)

#### Primary outcome(s)

FEV1 slope response, measured at 12 months.

#### Key secondary outcome(s))

Measured at 12 months:

- 1. Six minute walk testing
- 2. Quantitative CT of the lung
- 3. Quality of life questionnaires (St George Respiratory Questionnaire, 36-item short form health survey [SF-36])
- 4. Forced vital capacity (FVC)
- 5. Diffusing capacity of the lung for carbon monoxide (DLCO)

#### Completion date

31/12/2010

## **Eligibility**

#### Key inclusion criteria

- 1. Women, aged 18 years and older
- 2. Signed and dated informed consent
- 3. Diagnosis of LAM as determined by biopsy (lung, abdominal mass, lymph node or kidney) and chest computed tomography [CT] scan findings compatible with LAM; or compatible chest CT scan findings in the setting of tuberous sclerosis, angiomyolipomata (diagnosed by CT, magnetic resonance imaging [MRI] or biopsy), or chylous pleural effusion (verified by thoracentesis)
  4. Forced expiratory volume in one second (FEV1) less than or equal to 70% of predicted during
- 4. Forced expiratory volume in one second (FEV1) less than or equal to 70% of predicted during baseline visit after bronchodilator

### Participant type(s)

Patient

#### Healthy volunteers allowed

No

### Age group

Adult

### Lower age limit

18 years

#### Sex

Female

### Key exclusion criteria

- 1. History of myocardial infarction, angina or stroke related to atherosclerosis
- 2. Pregnancy or breast feeding, inadequate contraception
- 3. Significant haematologic or hepatic abnormality (i.e., transaminase levels greater than 3 times the upper limit of normal range, haematocrit [HCT] less than 30%, platelets less than 80,000 /cumm, adjusted absolute neutrophil count less than 1,000/cumm, total white blood cell count [WBC] less than 3,000/cumm)
- 4. Intercurrent infection at initiation of sirolimus
- 5. Recent surgery 2 months (involving entry into a body cavity or requiring sutures)
- 6. Use of an investigational drug within the last 30 days
- 7. Uncontrolled hyperlipidaemia
- 8. Previous lung transplantation
- 9. Inability to attend scheduled clinic visits
- 10. Inability to give informed consent
- 11. Inability to perform pulmonary function testing
- 12. Creatinine greater than 2.5 mg/dl (greater than 220 µmol/L)
- 13. Chylous ascites sufficient to affect diaphragmatic function on pulmonary function tests (PFTs)
- 14. Pleural effusion sufficient to affect pulmonary function based on the opinion of the Site Investigator (generally greater than 500 cc)
- 15. Acute pneumothorax within the past 2 months (as pneumothorax can alter baseline PFT results)
- 16. History of malignancy in the past two years, other than squamous or basal cell skin cancer
- 17. Use of oestrogen containing medications

### Date of first enrolment

01/06/2007

#### Date of final enrolment

31/12/2010

### Locations

#### Countries of recruitment

Australia

Canada

Japan

United States of America

#### Study participating centre

#### 11C-1183 Toronto, Ontario Canada M5G 1L7

## Sponsor information

## Organisation

University Health Network (Toronto) (Canada)

#### **ROR**

https://ror.org/042xt5161

## Funder(s)

#### Funder type

Research organisation

#### **Funder Name**

Canadian Institutes of Health Research (CIHR) (Canada) - http://www.cihr-irsc.gc.ca (ref: MCT-83051)

## **Results and Publications**

Individual participant data (IPD) sharing plan

## IPD sharing plan summary

Not provided at time of registration

## Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Results article	results	28/04/2011		Yes	No
Results article	results	01/08/2013		Yes	No
Study website	Study website	11/11/2025	11/11/2025	No	Yes