

The logo for ERS Congress 2024, featuring a red circle with a white grid pattern inside, followed by the text "ERS CONGRESS | 2024" in white.

# ERS CONGRESS | 2024

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## Characteristics and clinical course of lymphangioleiomyomatosis (LAM) in patients treated with sirolimus in France

**A French real life study based on medico-administrative claim data from SNDS between 2014 and 2021**

Vincent Cottin, Lidwine Wemeau, Brioux Chardès, Jérémie Rudant, Nada Assi, Aurélie Schmidt, Hélène Denis, Yurdagül Uzunhan

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**The study was conducted by HEVA and funded by Pfizer**

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## Background

Lymphangiomyomatose (LAM) is a rare, multiple cystic lung disease, mostly affecting women, with a median age of 34 years.

A treatment with mTOR inhibitor (sirolimus) is indicated for patients with sporadic LAM with FEV1 < 70% of predicted value, or deteriorating lung function.

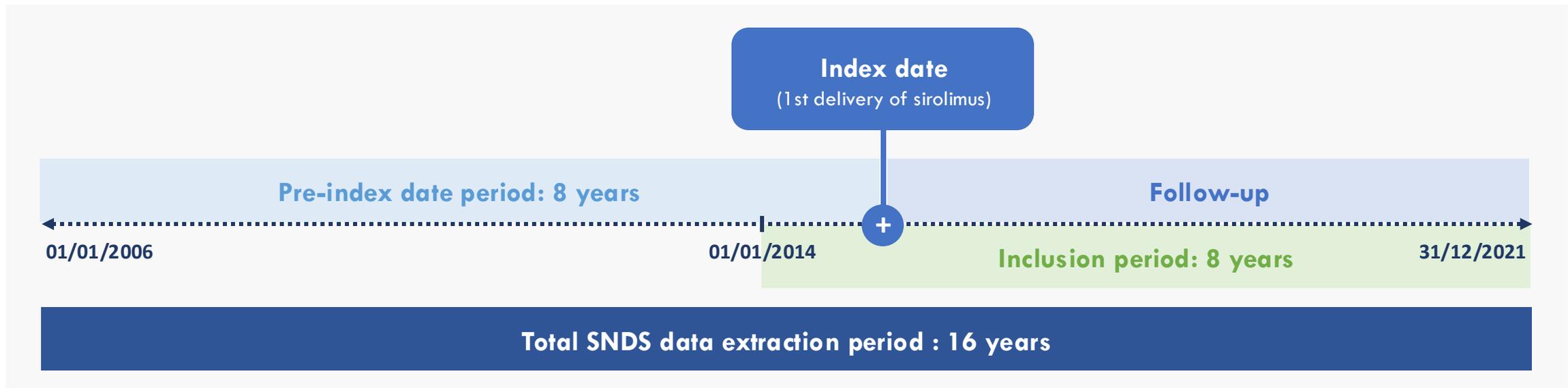
## Study objectives

- To estimate the number of LAM patients treated with sirolimus in France during the study period
- To describe their characteristics
- To describe the disease evolution (mortality, complications)

→ *This study was conducted upon request from the French health authority (Haute Autorité en Santé)*

## Observational study based on the French National Health Database (*Système national des données de santé, SNDS*), which

- covers about 99 % of the population living in France (68 M)
- collects individual pseudonymised data from beneficiaries of almost all health insurance schemes.
- contains socio-demographic data (age, sex, and area), and information on all health care expenses, including outpatient visits, reimbursed medication, medical procedures, hospital admission diagnoses and procedures, and date of death.



All adult women who received sirolimus in France between 2014 et 2021 were included in this study.

# Patients exposed to sirolimus for LAM

Since LAM does not benefit from a specific ICD-10 code, patients were identified through a stepwise algorithm considering: **sirolimus indications, uses in real-life, occurrence of events associated with the evolution of the disease.**

Data extraction from French National Health Database

**Adult women with at least one delivery of sirolimus between 01/01/2014 and 31/12/2021**

**N = 1,800**

« Possible » LAM  
N = 638

Exclusion of other sirolimus indications (transplantation, graft-versus-host disease, auto-immune disease, cancer)

« Probable » LAM  
N = 208

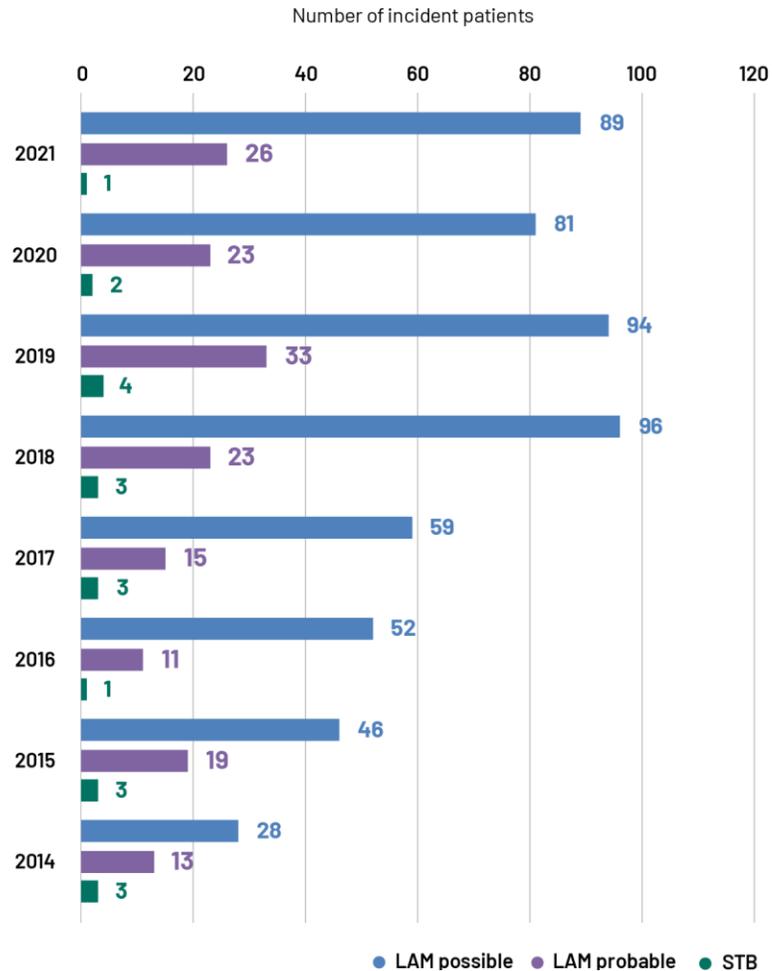
Selection of patients with at least one clinical manifestation highly suggestive of LAM (including pneumothorax, pleural drainage, pleural effusion, ascite, chronic respiratory failure, lung transplantation, or angiomyolipoma)

**Tuberous Sclerosis Complex-LAM**  
N = 33

Defined by the presence of at least one hospitalization with the ICD-10 code Q851 Tuberous Sclerosis Complex

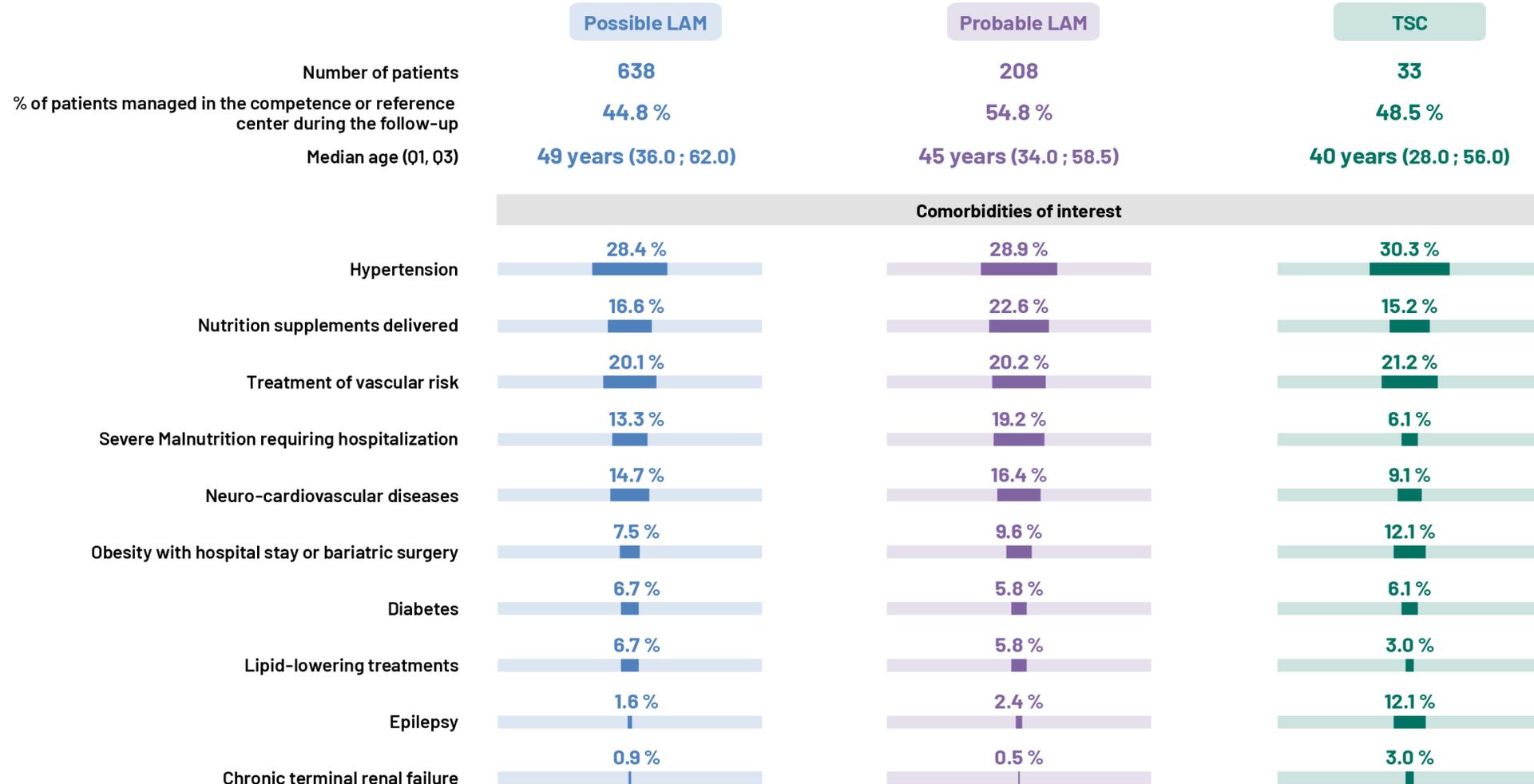


# Number of patients newly treated with sirolimus for LAM in France between 2014 and 2021



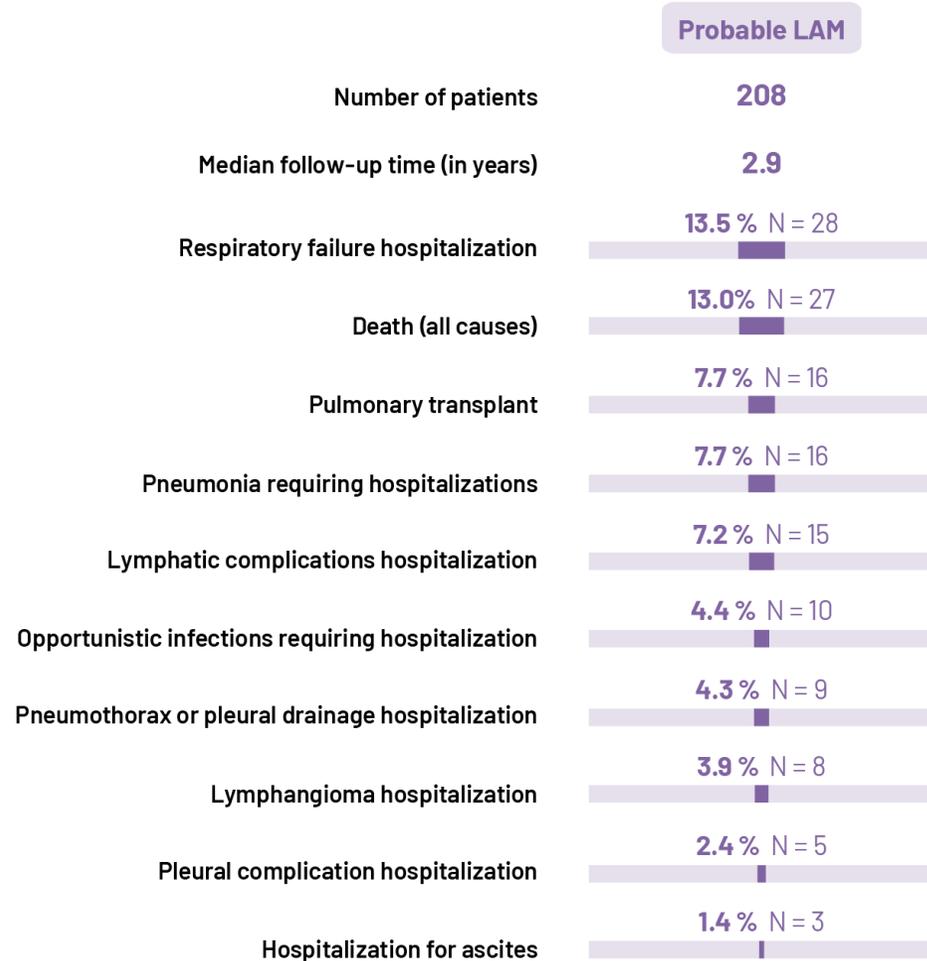
**15-33 patients with probable LAM  
newly treated with sirolimus per year**

# Patients characteristics and comorbidities



# Evolution of LAM

## Frequency of event occurrence during the follow-up



Probability of survival at 5 years  
post sirolimus initiation :

**84 % (CI 95 %: 76 %; 90 %)**

## Strengths

Exhaustive database of the French Health Insurance Insurance (SNDS)

- Approximately 68 million insured persons
- Complete including primary care (consultations, biological tests, drug dispensing) and hospitalisations (in public and private institutions)

No recall bias

Long-term follow-up

## Limitations

Identification of LAM patients through proxys/algorithms based on hospitalisation

- No ICD-10 code for LAM
- Coding is operator-dependent and may vary with coders or institutions
- Results of clinical examinations and tests are not available (PFTs, VEGF-D, etc)
- Possible underestimation of non-severe cases that would not require an hospitalisation (may be mitigated by the long-term follow-up)

Untreated LAM, and LAM patients receiving everolimus were not studied

# Conclusion

This study based on real-life data estimated

- Up to 33 new patients with LAM initiating sirolimus per year
- About ~200 patients with LAM receiving sirolimus in France ( $\sim 6/10^6$  women)
- 84% probability of survival at 5 years in patients with LAM and receiving sirolimus
- No signal for unexpected complications or infections