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Clinical characteristics and prognosis of patients with lymphangioleiomyomatosis

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Background/Aims: Lymphangioleiomyomatosis (LAM) is a rare cystic lung disease that primarily affects women. Although sirolimus has become the standard of care for progressive LAM, the factors involved in the natural history of LAM are poorly understood. This study aims to identify the clinical features and prognosis of LAM patients, and compare these features based on method of treatment. **Methods:** Medical records were retrospectively reviewed of patients diagnosed with LAM from 2005 to 2018 at Severance Hospital, South Korea. The diagnosis of LAM was made according to 2010 European Respiratory Society (ERS) criteria. The patients were classified into observation or treatment group, where those with progressive disease received either sirolimus or lung transplantation (LTx). Baseline clinical features, treatment methods, pulmonary function tests (PFT), and survival were analyzed. **Results:** Total 54 patients (52 definite, 2 probable) were enrolled in this study. Most patients (53/54, 98.1%) were female, and the median age at diagnosis was 36.5 years. Among 52 (96.3%) patients who were classified as definite LAM, 36 (66.7%) patients were biopsy-proven. Median follow-up duration was 4.92 years. All patients presented with characteristic features of LAM on high-resolution computed tomography; 36 (66.7%) with pneumothorax; 27 (50%) with abdominal lymphadenopathy (LN); 18 (33.3%) with angiomyolipoma; 8 (14.8%) with tuberous sclerosis complex; 6 (11.1%) with chylothorax. Of the 36 patients in treatment group, 23 patients (20 sirolimus, 3 Afinitor) received mTOR inhibitor and 13 patients underwent LTx. 18 patients remained under medical observation. Younger age at diagnosis, presence of abdominal LN, worse baseline PFT parameters were associated with treatment initiation rather than observation. The overall survival rates at 5 and 10 years after diagnosis were 92% and 74.7%, respectively. **Conclusions:** LAM is a rare disease with a diverse clinical course, but with relatively low mortality. This study reveals that younger age at diagnosis, abdominal LN, poor baseline lung function are factors associated with LAM treatment.

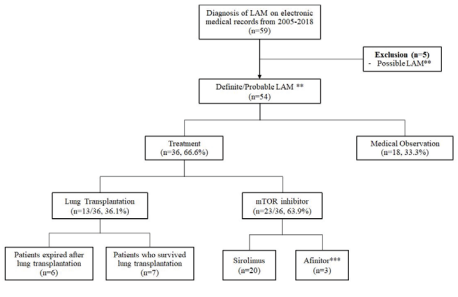

Figure 1. Flowchart of Patient Selection, Study Groups and Outcomes.
** Diagnosis based on ERS guidelines (2010, ERJ) on LAM
*** All patients treated with Afinitor had Tuberous Sclerosis Complex-associated Angiomyolipoma

Table 1. Baseline Characteristics of 54 patients with Definite/Probable LAM

	All (n=54)	mTOR inhibitor (n=23)	Lung transplantation (n=13)	Observation (n=18)	p value
Females	53 (98.1)				
Age at diagnosis, years	36.5 [31.75 - 42.25]	34.5 [28.25 - 38.75]	37.00 [32.00 - 41.50]	42.0 [36.0 - 47.0]	**0.033**
Menopause before diagnosis**	4 (9.5)	0 (0.0)	0 (0.0)	4 (26.7)	**0.019**
Median f/u duration (years)	4.89 [1.53 - 8.43]	3.77 [1.99 - 8.92]	6.56 [4.04 - 9.49]	2.21 [0.97 - 8.37]	0.112
Mortality	6 (11.1)				
Diagnosis					
Definite	52 (96.3)	22 (95.7)	13 (100.0)	17 (94.4)	1.000
Probable	2 (3.7)	1 (4.3)	0 (0.0)	1 (5.6)	
Biopsy-proven	36 (66.7)	11 (47.8)	13 (100.0)	12 (66.6)	**0.003**
Transbronchial biopsy	2/36 (5.3)				
Video-assisted Thoracic Surgery	32/36 (84.2)				
Extrathoracic sites	4/36 (10.5)				
Clinical Characteristics					
Charateristic HRCT	54 (100.0)	23 (100.0)	13 (100.0)	18 (100.0)	
Pneumothorax	36 (66.7)	17 (73.9)	9 (69.2)	10 (55.6)	0.470
Abdominal Lymphadenopathy	27 (50.0)	13 (56.5)	9 (69.2)	5 (27.8)	0.053
Angiomyolipoma	18 (33.3)	9 (39.1)	2 (15.4)	7 (38.9)	0.325
Presence of TSC	8 (14.8)	5 (21.7)	0 (0.0)	3 (16.7)	0.236
Chylothorax	6 (11.1)	5 (21.7)	1 (7.7)	0 (0.0)	0.077

mTOR = mechanistic target of rapamycin, HRCT = high-resolution computed tomography, TSC = Tuberous sclerosis complex
Data shown in n (%) or Median [IQR]. Significance level at p < 0.05
** Data available for n = 42 in all patients, n = 21 in mTOR inhibitor, n = 6 in LTx, n = 15 in Observation, respectively.