Abstract: PB2213

Title: RETROSPECTIVE ANALYSIS OF 45 UNSELECTED PATIENTS WITH HISTIOCYTOSIS CONFIRMS PROGNOSTIC RELEVANCE OF ORGAN INVOLVEMENT: SINGLE-CENTER EXPERIENCE

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Background:

The histiocytosis is rare disorder characterized by the accumulation of macrophage, dendritic cell, or monocyte-derived cells in various tissues and organs of children and adults. Their clinical behavior ranges from mild to disseminated and, sometimes, life-threatening forms. Due to the rarity of the disease in adults, LCH is usually treated with modified pediatric protocols.

Aims:

In this study we aimed to review our clinical findings and treatment outcomes in a retrospective series of adult of L(Langerhans) and R(Rosai Dorfman) group histiocytosis patients treated over a period of 15 years.

Methods:

We retrospectively evaluated clinical findings and outcomes of treatment in a retrospective series of 45 adult patients, treated at our center between 2007-2022.

Results:

Forty-five patients with histiocytic neoplasia were included in the study. 86% (n=39) of the patients were diagnosed with Langerhans Cell Histiocytosis (LCH), 11% (n=5) with Erdheim-Chester Disease (ECD) and 2% (n=1) with Rosai Dorfman (RD). The median age of diagnosis was 32 (18-62), and the F/M ratio was 20/25. In 51% (n=23) of the patients, PET-CT was evaluated at the time of diagnosis, while approximately half of them had localized organ involvement, multi-system or multifocal involvement of a single system was detected in 39% (n=9) of the patients. Clinical characteristics of the LCH cohort showed in table 1. In 2 patients with risky organ involvement; bone marrow (n=1), spleen (n=2) and liver (n=1) lesions were found. In 26% (n=12) cases with central nervous system involvement; pituitary involvement (n=3), diabetes insipidus (n=8), hypogonadism (n=1), and cranial mass (n=3) were found. Treatment approaches and outcomes by disease category showed in table 2. Molecular tests were performed in 35% (n=16) of patients, and mutation was detected in 14 of them. We used targeted drug therapies BRAF (n=5) and MEK (n=2) inhibitors in these patients. Complete response was obtained in patients receiving targeted therapy. Two LCH patients were treated with allogeneic stem cell transplantation, one of them because of concomitant diagnosis of MDS/MPN and the other because of refractory disease. Both are in remission at follow-up. Median overall survival for the cohort as a whole was 92.1 % at 5 years and median EFS was 171.7 months (95% CI 51.1-292.2). There were no significant differences in 5-years OS between SS-LCH, MS-LCH, and pulmonary-LCH (NR vs 89.7 % vs 66.7 %; p=0.12)(Figure-1). Seven patients died, and only two of them were due to refractory disease.

Summary/Conclusion:

Based on the results of our single-center experience, PET-CT is very important in determining organ involvement and evaluating the outcome of treatment among patients with histiocytosis. The response was obtained with targeted drugs and the side-effect profile was well tolerated. It’s important to note that isolated pulmonary involvement may cause very serious respiratory failure resulting in death. Nevertheless, we report an unexpected interesting new finding which although did not reach significance, addresses a key unmet need in LCH and will inform future studies.

Table 1 Clinical characteristics of the LCH cohort					
No. of patients		SS-LCH 17	MS-LCH 20	pLCH 3	Total 39
Age	median 30(18–57)	33(18-51)	26.5(19-4 7)	26 (24-57)	
Gender	Males Females	12 5	8 12	2 1	22 18
Site of disease	Bone	10	13		23
	Special sites	1			1
	Skin/muco sa	4	7		11
	Lung	1	14		15
	Lymph nodes	0	6		6
	Bowel	0	1		1
	CNS	1	7		8
	Liver/spl een	0	2		2
Diabetes insipidus		0	6	0	6
SS-LCH = single-sy stem Langerhan s cell histiocyt osis; MS-LCH = multisyst em Langerhan s cell histiocyt osis; pLCH = primary pulmonary LCH; CNS = central nervous system					

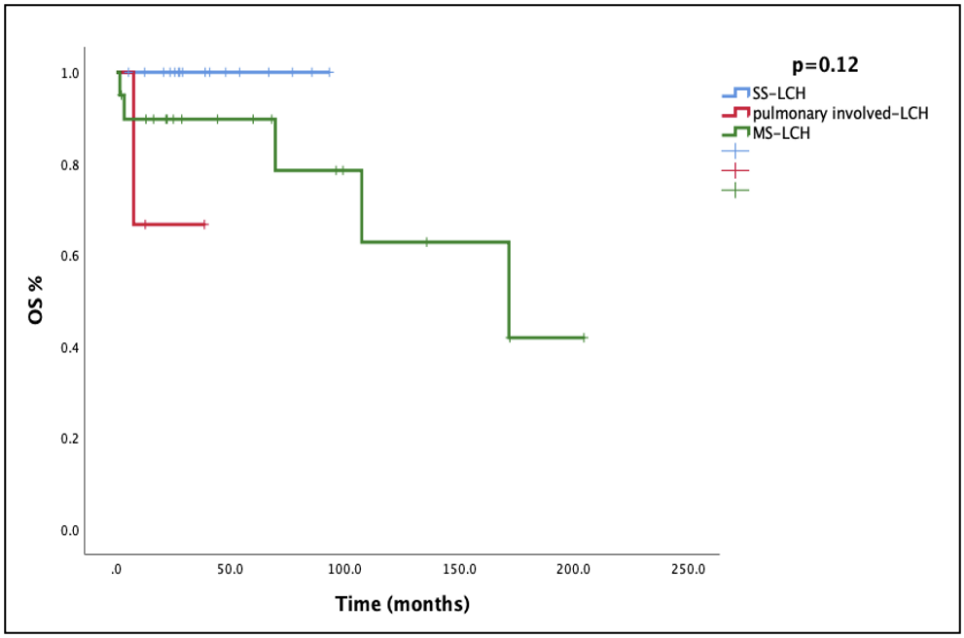

Figure-1. 5-years OS between SS-LCH, MS-LCH, and pulmonary-LCH

Keywords: Langerhans Cell Histiocytosis, Histiocytosis