

## A multicenter analysis of clinical features and long-term outcomes of POEMS syndrome in Korea

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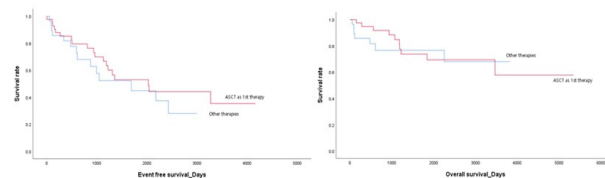
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**Background/Aims:** POEMS syndrome is a rare disease that is characterized by polyneuropathy, organomegaly, endocrinopathy, monoclonal protein, and skin changes. There are few reports regarding the syndrome due to its low incidence.

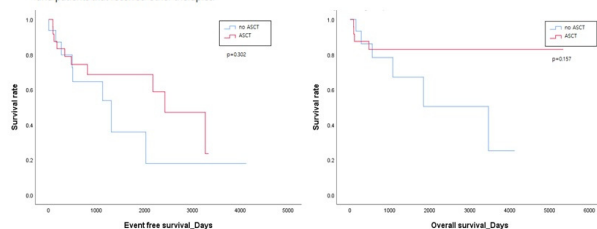
**Methods:** This study is a multicenter retrospective study that analyzed the clinical characteristics and event free survival(EFS), overall survival(OS) of 71 POEMS syndrome patients(pts) from eight institutions, from 2000-2020.

**Results:** The median age at diagnosis was 52(range, 28-77). Neurologic symptoms were present in all pts (100%), 77% showed organomegaly, 72% showed endocrinopathy, 93% presented with monoclonal gammopathy and 58% showed skin changes. Volume overload and Castleman disease were found in 76%, 21% of pts, respectively. VEGF levels were available in 32 pts with median 1245(Range 102~12900).(Table 1) Median follow up duration was 1949 days(range 230-5540). Of 71 pts, 12 received only local radiotherapy(RTx), 5 underwent chemotherapy(CTx) and local RTx, and 14 received CTx. Among the other 40 pts, 28 received autologous stem cell transplant(ASCT) without previous CTx and 12 were treated with CTx and ASCT. Pts were stratified to low and high risk groups by age, presence of volume, and kidney function. There was no significant difference in EFS and OS rate between pts that received ASCT and the other group (Figure1). Subgroup analyses revealed that the high-risk POEMS patients who received upfront ASCT showed a trend of increased overall survival rate. (p=0.157) (Figure2) In the low-risk group, there was no significant difference in EFS rate and OS rate depending on the treatment received (Figure3).

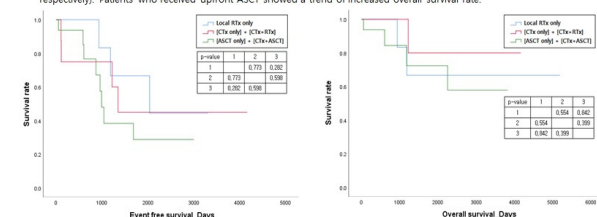
**Conclusions:** Regarding POEMS syndrome, establishing treatment guidelines based on statistics is currently necessary. In high-risk POEMS patients, performing ASCT may be considered since patients who received ASCT showed an increased overall survival rate. For lower risk POEMS patients, undergoing treatment with local RTx may be a considerable treatment option since there is no significant survival benefit of performing CTx and ASCT, which may present with more complications than local RTx.



**Figure 1.** Kaplan-Meier event free survival (EFS) and overall survival(OS) curve for all patients with newly diagnosed POEMS syndrome. (p=0.469, p=0.704, respectively). There was no significant difference in OS and EFS between patients that received ASCT as 1<sup>st</sup> therapy and patients that received other therapies.



**Figure 2.** Kaplan-Meier event free survival and overall survival curve for high-risk POEMS patients. (p=0.302, p=0.157, respectively). Patients who received upfront ASCT showed a trend of increased overall survival rate.



**Figure 3.** Kaplan-Meier event free survival and overall survival curve for low-risk POEMS patients depending on treatment strategies. (p-values as above). There was no statistically significant difference between the 3 treatment groups.

**Table 1.** Clinical characteristics of patients with newly diagnosed POEMS syndrome

Clinical characteristics (N,%)	All patients (N=71)	Low risk group (N=31)	High risk group (N=40)
<b>Demographic features</b>			
Age > 50 years	38 (53)	18 (58)	20 (50)
Male	45 (63)	19 (61)	26 (65)
<b>POEMS features</b>			
Polyneuropathy	71 (100)	31 (100)	40 (100)
<b>Organomegaly</b>			
Hepatomegaly	40 (56)	9 (29)	31 (78)
Splenomegaly	31 (44)	16 (51)	15 (38)
Lymphadenopathy	24 (34)	5 (16)	19 (48)
Endocrinopathy	51 (72)	21 (68)	30 (75)
Monoclonal gammopathy	66 (93)	29 (93)	37 (93)
Skin changes	41 (58)	15 (48)	26 (65)
<b>Volume overload</b>			
Ascites	25 (35)	2 (6)	23 (58)
Pleural effusion	27 (38)	1 (3)	26 (65)
Pericardial effusion	16 (23)	0 (0)	16 (40)
Pulmonary Hypertension	20 (28)	0 (0)	20 (50)
Castleman's disease	15 (21)	3 (10)	12 (30)
Papilledema	19 (27)	5 (16)	14 (35)
Osteosclerosis	46 (65)	27 (87)	19 (48)
Osteolytic lesion	33 (46)	24 (77)	9 (22)
Kidney dysfunction (Cr > 1.5)	7 (10)	0 (0)	7 (17)
Serum VEGF > 2000 pg/ml	9 (13)	4 (13)	5 (13)
β2mg ≥ 5	18 (25)	2 (6)	16 (40)
<b>Therapeutic regimen</b>			
Local Radiotherapy	12 (17)	7 (22)	5 (13)
Chemotherapy only	5 (7)	5 (16)	0 (0)
Chemotherapy and Radiotherapy	14 (20)	3 (10)	11 (28)
ASCT only	28 (40)	12 (39)	16 (40)
Chemotherapy and ASCT	12 (17)	4 (13)	8 (20)

Abbreviations : ASCT, autologous stem cell transplantation.