EFFICACY AND SAFETY OF COBIMETINIB USED IN MONOTHERAPY FOR ERDHEIM-CHESTER DISEASE

4CPS-122
L01-Cytostatics

Muñoz Burgos M., Desongles Corrales T.
Hospital Universitario Virgen del Rocío. Seville, Spain.

OBJECTIVE

Evaluate the efficacy and safety of the MEK inhibitor cobimetinib used in monotherapy for Erdheim-Chester Disease (ECD) patients without the *BRAF* mutation.

MATERIAL AND METHODS

Three patients received cobimetinib alone.

Efficacy → monitoring histiocytic infiltrations and metabolic response with PET-CT scans.

Safety → number and severity of side effects.

Variables: age and sex , date of diagnosis and manifestations, presence of mixed histiocytosis, *BRAF* status, previous treatment and reasons to change, date of cobimetinib initiation and dose, initial-final creatinine level, evolution of histiocytic infiltrations and side effects.

RESULTS

2 men+1woman → median age 50 y.o.
All of them → WildType-BRAF
1 patient → mixed histiocytosis
Time from diagnosis until cobimetinib initiation → 11,22 and 51 months.
Previously → pegylated interferon alfa
Change → progression PET-CT
Dose → 60mg/day for 21 days (28-day cycle)

MANIFESTATIONS

Perirenal infiltration	2
Long bones hypermetabolism	3
Retroperitoneal fibrosis	2
Cardiac involvement	1
Arterial affection	1

1. complete response with 3 cycles + creatinine level decreased significantly + stopped dialysis
2. excellent metabolic response with 3 cycles
3. stabilization of perirenal infiltration

ADVERSE EVENTS

Rash	3
Acne	2
Arthralgia	2
Diarrhoea	3
Asthenia	2
Cardiac failure	1
Erythema	1

CONCLUSION

Cobimetinib represents an option for *WT BRAF* patients. However, its toxicity is considerable. Further research is certainly warranted to better define this therapeutic alternative.

http://www.eahp.eu/24-4CPS-122