

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

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L01-Cytostatics

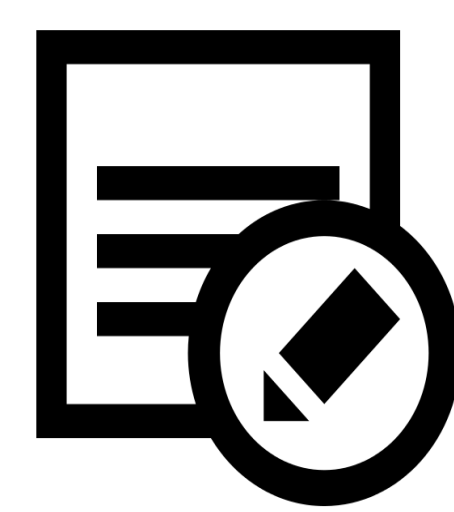
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OBJECTIVE

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

ADVERSE EVENTS

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

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.

