

# EFFICACY, SAFETY AND COST OF POMALIDOMIDE IN RELAPSED AND REFRACTORY MULTIPLE MYELOMA

P. Gueneau<sup>1</sup>, D. Caillot<sup>2</sup>, A. Cransac<sup>1</sup>, C. Pernet<sup>1</sup>, M. Boulin<sup>1</sup>

<sup>1</sup> Dijon University Hospital, Pharmacy, Dijon, France, <sup>2</sup> Dijon University Hospital, Haematology, Dijon, France.

## BACKGROUND

Patients with relapsed and refractory multiple myeloma (RRMM) have a median survival of about 3-6 months, pomalidomide is a very potent member of the immunomodulatory drug family.

## PURPOSE

The aim of the study was to analyse **efficacy, safety and cost** of pomalidomide in patients with RRMM.

## MATERIAL AND METHODS

All patients in whom a treatment by pomalidomide was initiated between August 2013 and October 2015 for a RRMM in our teaching hospital were included. We defined 3 groups : Patients who relapsed early (<3months), responding patients and stable patients at 3 months.

Outcomes were : **Predictive factors of early pomalidomide discontinuation (before 3rd month), Overall response rate (ORR, using International Multiple Myeloma Working Group criteria), Overall survival, Safety, Treatment cost per patient.**

## RESULTS

63 patients (male, 59%) were included (**Figure 1**). All patients received pomalidomide and dexamethasone.

A time from diagnosis to pomalidomide initiation < 3 years was independently associated with a an early pomalidomide discontinuation ( OR = 5.82;95%CI 1.51-22.4;p=0.01),

At 3 months, **ORR** was 51%. The independants risk factors of mortality from pomalidomide initiation were : **early pomalidomide discontinuation** (hazard ratio, 6.8 vs no early discontinuation ; 95%CI, 2.3-19.6;p<10-3) and **hemoglobin level below 11 g/dl** (hazard ratio, 2.7 vs  $\geq 11$  g/dl ; 95%CI, 10-7.0;p=0.04).

Median **OS** from pomalidomide initiation was **6.4 months in patients who early discontinued pomalidomide, 17.1 months in patients with a stable disease and not achieved in responders** (**Figure 2**).

24 patients required a dose decrease for adverse events. The most common grade  $\geq 3$  adverse events were **neutropenia (14%) and infections (25%)**.

The mean pomalidomide cost per patient was €79,717  $\pm$  46,296 (range 17,850 – 241,200) (**Figure 3**): **early discontinuation, € 21,700  $\pm$  15,084 ; responders, € 88,905  $\pm$  46,320 ; stable disease, € 56,394  $\pm$  38,661 ; p<10-3).**

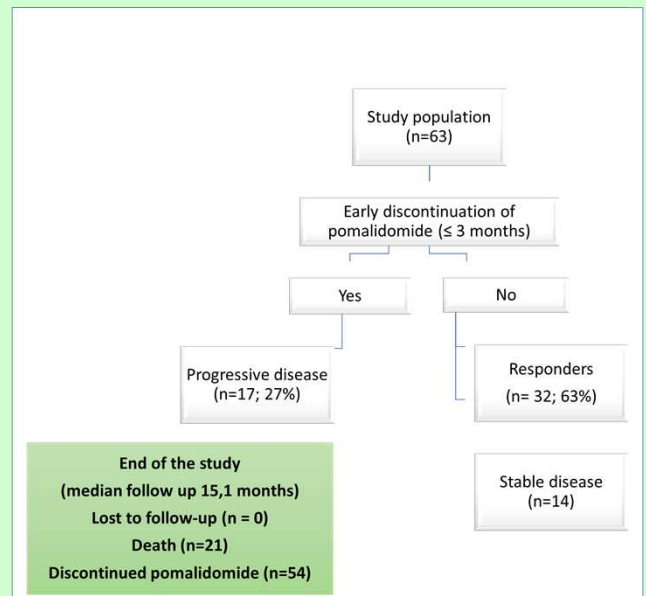


Figure 1. Flowchart of the study

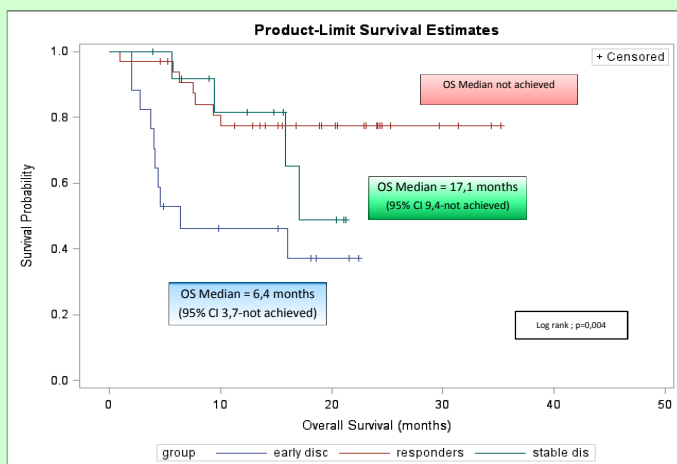


Figure 2. Kaplan Meier estimated overall survival from pomalidomide initiation by group

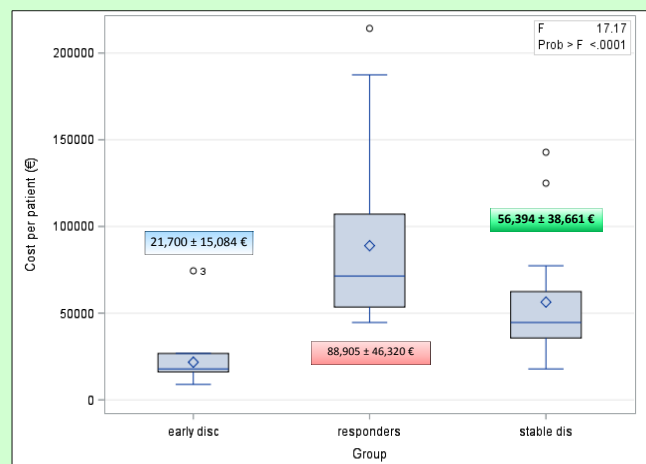


Figure 3. Treatment cost per patient by group (mean cost  $\pm$  standard deviation)

## DISCUSSION AND CONCLUSION

Compare to the MM-003 phase III trial, we reported similar safety data but a higher ORR (51%vs. 21%). We demonstrated a long-term favorable safety and efficacy profile of pomalidomide in RRMM patients even with a stable disease.