



(G-CSF + Plerixafor)

EUR 13,354 per PM patient

# COST-EFFECTIVENESS OF HEMATOPOIETIC STEM CELL MOBILIZATION STRATEGIES IN MULTIPLE MYELOMA AND LYMPHOMA PATIENTS IN THE CZECH REPUBLIC

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### Introduction

Blood stem cell mobilization, which is important as a source of hematopoietic stem cells for autologous transplantation, is performed using granulocyte colony-stimulating factor (G-CSF), but is ineffective in around 20% of patients reported as poor mobilizers (PM). Combining G-CSF with plerixafor increases the percentage of successful mobilizations. The drug has orphan drug status and is approved for lymphoma and multiple myeloma patients. Several randomized studies showed that combination of G-CSF and plerixafor can increase peripheral blood CD34+ count and the success rate as well as yield of stem cells collected compared with the administration of G-CSF alone (1,2). Further studies reported plerixafor as a very efficient mobilizing agent facilitating capture of high-quality autologous hematopoietic stem cell grafts in about 70% of poor mobilizers (3-7).

## Objective

- The objective was to compare the cost-effectiveness of three available mobilization schemes:
  - i) the use of plerixafor "on demand" (POD) even during a first mobilization attempt in all patients with inadequate response
  - ii) the standard use of plerixafor strictly within a standard re-mobilization scheme following failure of the first mobilization (SSP)
  - iii) the standard (re)mobilization scheme without plerixafor (SSNP).

#### Methods

- Decision tree models were built to compare clinical outcomes and direct costs from the payer's perspective in all three strategies (Figure 1,2 and 3).
- The outcome evaluated was the success/failure in achieving the mobilization, excluding the follow up oncological treatment. The minimum successful amount of collected stem cells was set as 2 x 106 CD34+ cells/kg.
- The baseline was the beginning of the first mobilization, while the endpoint was the final conclusion about the mobilization success (success/failure).
- The models were populated with effectiveness and resource use data from a first-of-a-kind patient registry of all patients with plerixafor administered (n=93) in 6 Czech centers.
- SSNP and the first mobilization attempt in SSP was not captured within the registry and hence the model probabilities and efficacy had to be retrieved from published sources (8,9).
- The direct costs were considered from the perspective of payer, the Czech National Health Insurance Fund.

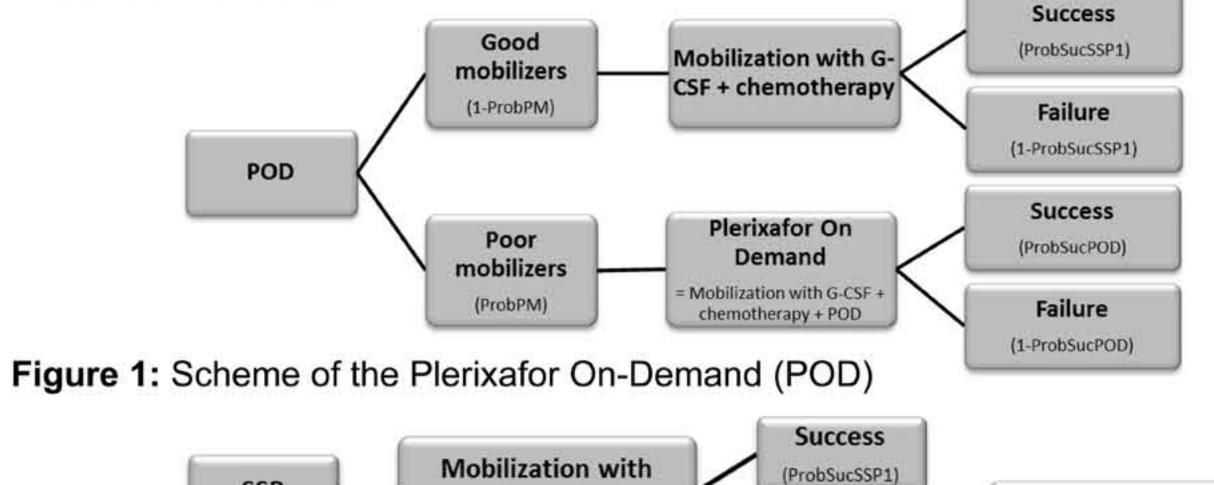
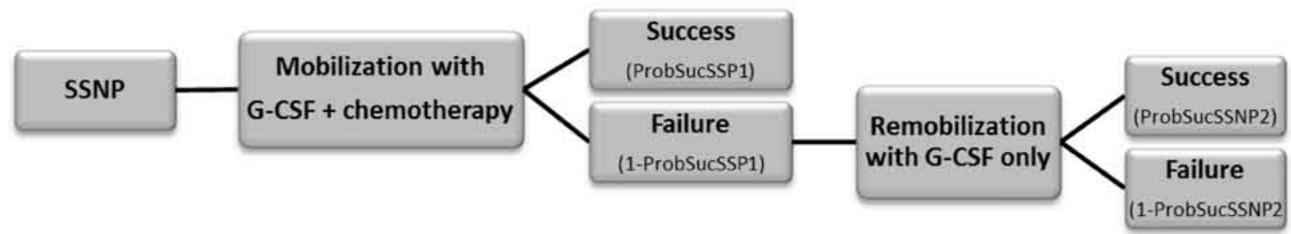


Figure 2: Standard scheme with plerixafor (SSP)

SSP



Failure

(1-ProbSucSSP1)

Figure 3: Standard (re)mobilization scheme without plerixafor (SSNP)

G-CSF + chemotherapy

# Results

- Out of the total 93 patients captured within the registry, 33 were treated with plerixafor on demand, 46 patients were enrolled in the study for re-mobilization and remaining 13 patients received plerixafor as predicted poor mobilizers and therefore were not considered for the purpose of this study.
- POD strategy has the greatest clinical benefit in terms of successfully mobilized patients over the SSP and SSNP strategies. At the same time, it has the second lowest total treatment costs associated with mobilization and therefore can be considered dominating over the SSP strategy and is only slightly more expensive (difference EUR 961) than the SSNP strategy, yet with substantially improved chance for successful mobilization in comparison to the SSNP.
- The total direct average costs per patient of the POD, SSP and SSNP strategies were estimated as EUR 5,736; EUR 6,416 and EUR 4,775, respectively. Projected percentages of successfully treated patients in the POD, SSP and SSNP strategies were 94.9%, 94.7% and 84.7%, respectively (Table 1).
- Considering the individual phases of the entire two-step process (mobilization and remobilization), the dissociated costs per patient associated with the first mobilization attempt and with the re-mobilization in respective strategies are shown in Figure 4,5 and 6.

## Results

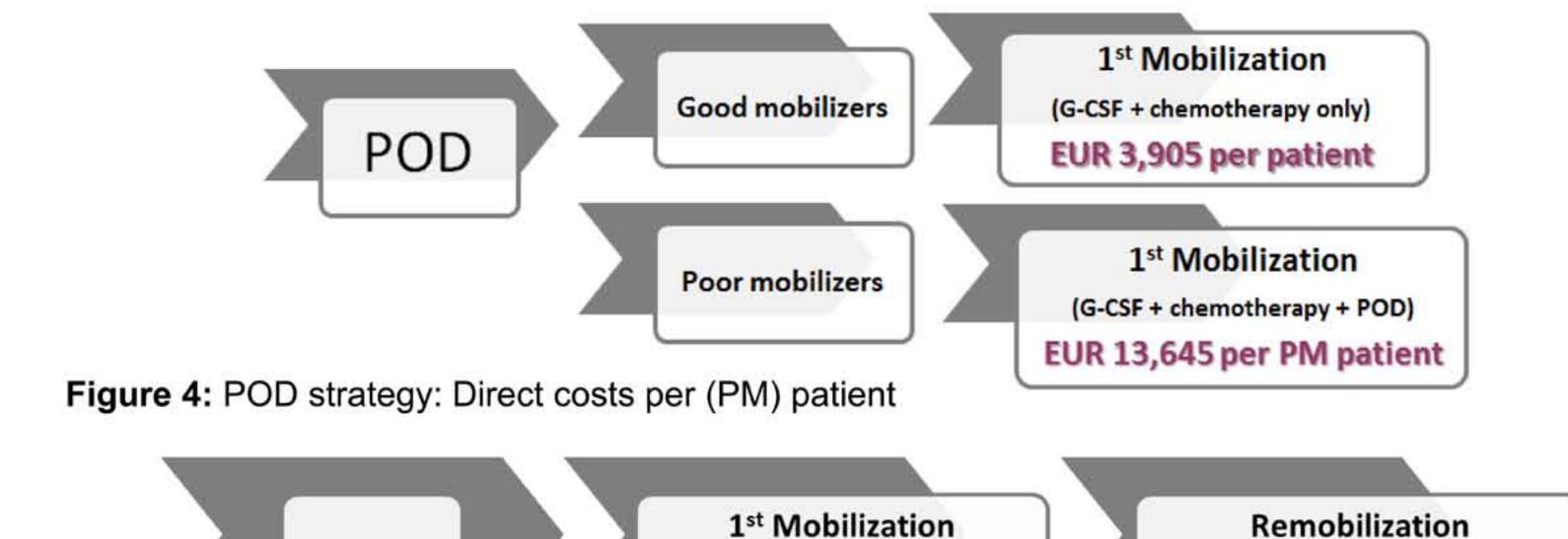


Figure 5: SSP strategy: Direct costs per (PM) patient

SSP



(G-CSF + chemotherapy only)

EUR 3,905 per patient

Figure 6: SSNP strategy: Direct costs per (PM) patient

- The overview in Figure 7 demonstrate the structure of direct costs in all three strategies.
- The cost-effectiveness expressed as the total costs per one successfully treated average patient were estimated as EUR 6,046; EUR 6,776 and EUR 5,641, respectively (Table 1).

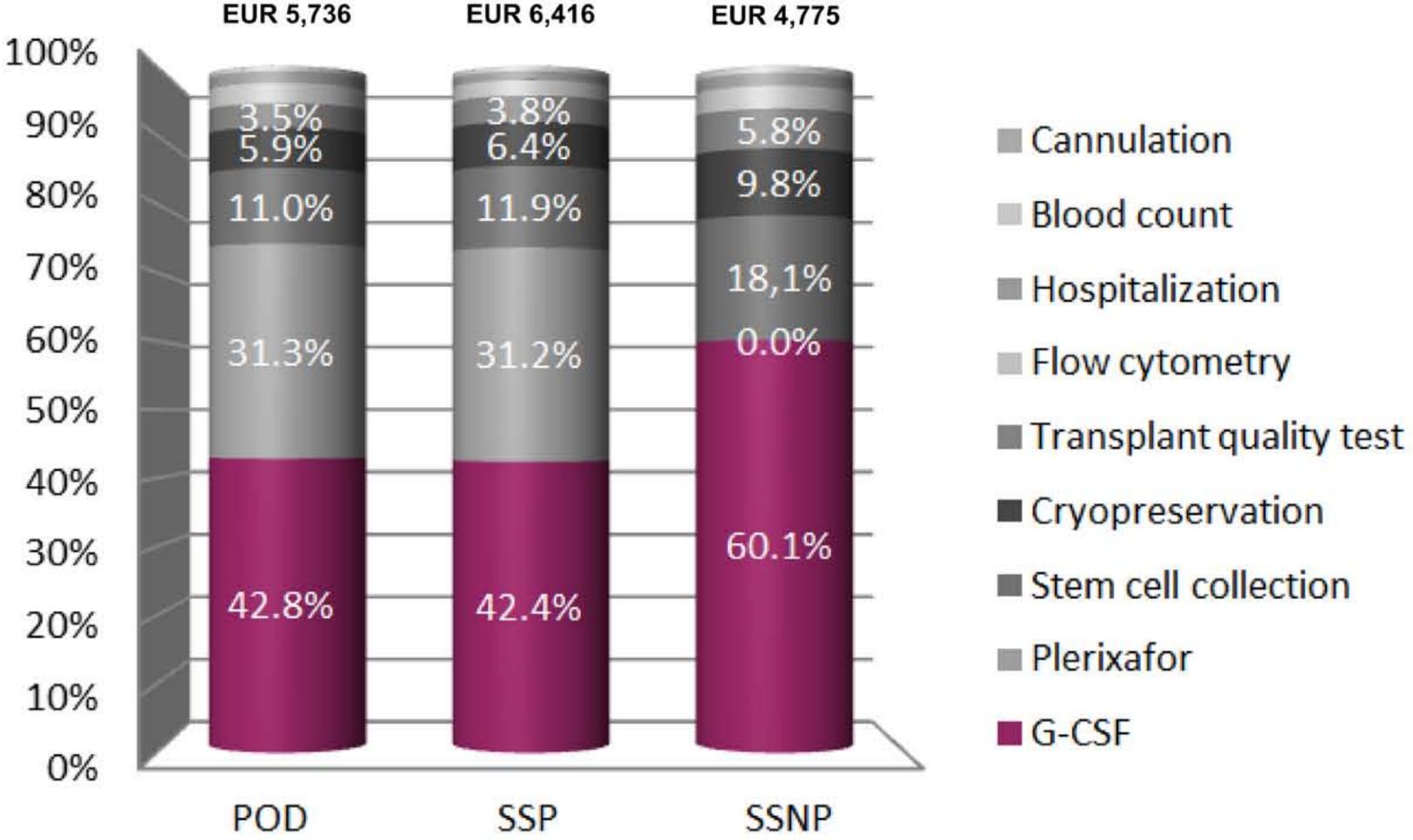


Figure 7: Direct cost structure of the POD, SSP and SSNP strategies

| Cost-effectiveness analysis                              | POD     | SSP     | SSNP    |
|----------------------------------------------------------|---------|---------|---------|
| Total average cost per patient                           | € 5,736 | € 6,416 | € 4,775 |
| Successfully treated patients (in %)                     | 94,9%   | 94,7%   | 84,7%   |
| Total costs per one successfully treated average patient | € 6,046 | € 6,776 | € 5,641 |

Table 1: Cost-effectiveness analysis

## Discussion

Success

(ProbSucSSP2)

Failure

(1-ProbSucSSP2)

Remobilization with

G-CSF + Plerixafor

- The use of plerixafor "on demand" delivers the highest success in collecting sufficient transplant (94.9 %) compared with the two other strategies.
- The total mobilization costs of mobilization without plerixafor were lower by estimated EUR 961 than with plerixafor used "on demand". However, the incremental costs for an additional successfully mobilized patient with plerixafor used "on demand" as compared to mobilization without plerixafor is EUR 94.2 only.
- Plerixafor is more cost-effective when used "on demand" during early mobilization than in subsequent re-mobilization.
- The use of plerixafor "on demand" (POD) during the first mobilization seems to be the most practical from the clinical perspective. The agent is administered only in patients inadequately responding throughout the undergoing mobilization without the need for a second mobilization. Further, the use on demand saves time and discomfort associated with patient's waiting and preparing for remobilization.
- In poor mobilizers, the POD approach allows shortening of the procedure with early proceeding to transplantation

# Conclusions

In conclusion, the analysis suggests that the use of plerixafor delivers major benefit for minimal incremental costs. If, however, plerixafor is to be included in the mobilization, it should be used "on demand" rather than in the second remobilization effort as this is a more cost-effective use.