Clinical Trial Terminated Early

Full title of the clinical Trial: Plerixafor for stem cell mobilization in patients with multiple myeloma who mobilize moderate to optimize collection results - a randomized, placebo-controlled, double-blind study

EudraCT Number: 2016-004122-41

Sponsor: Medical University of Vienna
Represented by (name): **aoUniv. Prof. Dr. Nina Worel**

Reason for premature termination of the clinical trial:

A sample size of 42 patients per group was calculated to provide a power of 80% to detect a 30% difference between the 2 treatment arms (placebo and plerixafor). The calculations were performed with a significance level α 0.05Unfortunately.

Unfortunately, only 21 patients (10 assigned to plerixafor and 11 to placebo) were enrolled at two study sites between February 2018 and 2021. The main factors limiting recruitment were the approval of daratumumab for standard first-line therapy of newly diagnosed MM patients, which has been associated with impaired stem cell mobilization outcomes and the COVID-19 pandemic. Daratumumab-containing induction regimens have been associated with reduced peripheral blood CD34+ cell yields, an increased need for plerixafor rescue, a higher number of apheresis sessions, and lower overall stem cell collection efficiency compared with daratumumab-free regimens. Consequently, a larger proportion of patients either failed to meet the study's predefined mobilization criteria or required plerixafor as part of standard clinical care, thereby substantially reducing the pool of eligible participants. Therefore, after an interim analysis of the first 21 patients it was decided to stop further enrollment.

Study results (if available):

Twenty-one patients were enrolled before premature study termination due to changes in first-line MM therapy and COVID-19 pandemic.

Peripheral blood CD34+ cell counts before study treatments (day 4) was similar between groups (median and range, 29.7 CD34+/µL, 16.9-34.8/µL in the plerixafor vs 22.0 CD34+/µL, 15.3-38.4/µL in

the placebo group; p=0.11). Plerixafor-treated patients had significantly higher PB CD34+ cell counts (median 93.3 CD34+/µL, range 53.4-140 CD34+/µL) than placebo-treated patients (median 29.3 CD34+/µL, range 11.0-56.2 CD34+/µL; p<0.001).

The fold increase in PB CD34+ cell count was also significantly higher in the plerixafor group than in the placebo group, with a median of 3.6-fold (range, 2.2-6.4) versus 1.2-fold (range, 0.5-2-8), respectively (p<0.001). On the first apheresis day all patient in the plerixafor group had PB CD34+ cell counts >50/µL compared with only one patient (9%) in the placebo group, respectively. In the placebo group 9 patients (82%) had >20 CD34+ cells/L before start of collection.

All patients in the plerixafor group reached the primary endpoint of ≥6x106/kg CD34+ cells in one apheresis day versus none of patients in the placebo group, respectively (p <0.001). The number of apheresis days required to collect ≥6x106/kg CD34+ cells in the placebo group was a median of 2 (range, 2-3).

Mean $CD34^+$ yield was significantly higher with plerixafor ($10.4 \pm 1.9 \times 10^6$/kg vs. $2.9 \pm 1.3 \times 10^6$/kg; p<0.001). Plerixafor-mobilized grafts contained significantly higher numbers of primitive hematopoietic progenitor subsets and demonstrated increased CFU-GM formation, indicating enhanced clonogenic potential. Despite marked differences in graft quantity and quality, engraftment kinetics, graft sustainability, and immune reconstitution were comparable between groups.

Date and Signature of Sponsor representative:

Vienna, 20th August 2026 aoUniv. Prof. Dr. Nina Worel