prior to transplant, irrespective of the use of rituximab (R), did and 25 m post-ASCT, respectively. The treatment immediately patients relapsed). The treatment related mortality was 0% and not reached (NR). The relapse rate was 65% (11 out of 17 ASCT was 66 months and the median overall survival (OS) was of 58 months, the median progression free survival (PFS) after [VGPR] and 6 partial responses [PR]). After a median follow-up 100% (3 complete responses [CR], 8 very good partial responses [VGPR] and many macroglobulinemia (WM) is a rare and incurable lymphoproliferative disease. The advent of novel therapies such as proteasome inhibitors and monoclonal antibodies has expanded the therapeutic armamentarium for treatment of WM. Due to the rare nature of the disease, most reported studies on the use of autologous stem cell transplant (ASCT) in WM are small and retrospective in nature. Since the advent of novel agents, few studies have reported whether these agents influence the disease course in the ASCT setting. Herein we present outcomes of patients with WM who underwent ASCT at three Mayo Clinic sites.

Methods: Records of all patients with WM who underwent ASCT between 8/2005 and 11/2017 were reviewed. Time-to-event analyses were performed from ASCT using the Kaplan-Meier method. Response criteria from the 6th International WM Workshop were used.

Results: Patient characteristics are described in Table 1. Two patients had large cell transformation; one prior to ASCT and one post-ASCT. The overall response rate to transplant was 100% (3 complete responses [CR], 8 very good partial responses [VGPR] and 6 partial responses [PR]). After a median follow-up of 58 months, the median progression free survival (PFS) after ASCT was 66 months and the median overall survival (OS) was not reached (NR). The relapse rate was 65% (11 out of 17 patients relapsed). The treatment related mortality was 0% and relapse mortality was 12%. The 2 deaths in the cohort were the patients who had large cell transformation and they died at 18 and 25 m post-ASCT, respectively. The treatment immediately prior to transplant, irrespective of the use of rituximab (R), did not impact the PFS (median PFS 47 m with prior R v 66 m without prior R, p=0.82, Fig 1A). Similarly, prior exposure to a bortezomib (V)-based regimen did not impact the PFS (median NR v. 47 m, p=0.19 respectively, Fig. 1B). Achieving ≥VGPR after ASCT did not result in superior PFS compared to patients who achieved a PR (47 m vs. NR, p=0.59, respectively, Fig. 1C). Patients who had ASCT after ≥2 lines of therapy had an inferior PFS compared to patients who had ASCT ≤2 lines of therapy (41 m vs. 112 m, p=0.03, respectively, Fig 1D). Patients with large cell transformation at any point, had inferior PFS after transplant compared to those who did not (10m vs. 66m, p=0.0001, respectively, Fig 1E). The median time to next treatment (mTTNT) after ASCT was 49 m. There were no differences in mTTNT whether patients achieved ≥VGPR or PR after ASCT (49m vs. 39m, p=0.86, respectively, Fig 1F).

Conclusions: ASCT for patients with WM is a safe and efficacious treatment modality with an ORR of 100% and affords eligible patients a median treatment-free interval of 4 years. The use of V or R prior to ASCT does not impact the depth of response or PFS after ASCT. To obtain the maximum PFS benefit, ASCT should be performed earlier in the disease course, prior to receiving more than 2 lines of therapy.