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ELEVENTH ANNUAL MEETING

APLASTIC ANEMIA: INSIGHT FROM A SINGLE CENTER EXPERIENCE

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MOFFITT CANCER CENTER

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INTRODUCTION

- Aplastic anemia (AA) is a rare disease with changing treatment modalities over time.
- Patients often do not respond to first line therapy and require multiple lines of therapy.
- Understanding treatment response and outcomes remains imperative.
- Treatment has also evolved over time, with the addition of thrombopoietin receptor agonists.
- Building a database of AA patients allows us to categorize treatments and outcomes for these patients

METHODS

- Single Center Retrospective Study
- Data collected on 53 patients who presented to Moffitt Cancer Center with diagnosis of AA between 1990 and 2023.
- Clinical, demographic, and genomic data were collected on eligible patients.
- All statistical analyses were performed using Kaplan-Meier statistics in SPSS

RESULTS

- Our study included 53 patients (mean age at diagnosis, 54 years) diagnosed with AA. (Table 1)
- At diagnosis, mean white blood count was 2.5, with an absolute neutrophil count of .95, a hemoglobin of 8.5, and platelet count of 24. (Table 1)
- 34/51 patients (67%) required packed red blood cell transfusion and 35/51 (69%) required platelet transfusion.
- Bone marrow cellularity was <10% in 26/48 patients (54%).
- Twenty-two patients were tested for large granular lymphocyte (LGL) clones, and 12 (55%) had clones present.
- Paroxysmal nocturnal hematuria (PNH) clones were detected in 22 out of the 42 patients tested (52%).
- T-cell clones were found in 20 out of 26 patients (77%).
- The most used first line therapies were equine ATG (eATG) + cyclosporine (CSA) + eltrombopag in 15, followed by eATG + CSA in 9, and eltrombopag only in 7.
- All 7 patients receiving eltrombopag only first line had no response.
- In patients receiving eATG/CSA with or without eltrombopag 9/24 (38%) had partial response and 7/24 (29%) had complete response; 1 patient did not have follow up data.
- No difference in response was observed between triple therapy incorporating eltrombopag compared to eATG/CSA alone.
- No difference in response was noted based on presence of LGL clone or PNH clone.
- Nine received allogeneic stem cell transplant (allo-SCT): 3 received it second line, 3 received it third line, and 3 received it fourth line or greater.
- Median overall survival was not reached in our cohort.

RESULTS

Baseline Variables	n = 53
Median Age, Years	54
Sex	
Male	31 (58%)
Female	22 (42%)
Race	
Caucasian	39 (74%)
African American	4 (8%)
Hispanic or Latino	5 (9%)
WBC, mean (x10^9/L)	2.5 (n=49)
ANC, mean (x10^9/L)	.95 (n=44)
Hemoglobin, mean (gm/dL)	8.5 (n=49)
Platelet Count (x10^9/L)	24 (n=50)
Requiring RBC transfusion	34 (n=51, 67%)
Requiring Platelet transfusion	35 (n=51, 69%)
Bone Marrow Cellularity <10%	26 (n=48, 54%)
LGL Clone	12 (n=22, 55%)
PNH Clone	22 (n=42, 52%)
T-Cell Clone	20 (n=26, 77%)

CONCLUSIONS

- AA is diagnosed primarily in symptomatic patients requiring transfusions with life threatening cytopenias.
- First treatment varies, but the most common therapies, which rely on eATG and CSA, as well as recently thrombopoietin receptor agonists had shown the most success.
- Patients often require multiple lines of treatment.

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