A Single Institutional Experience Using Romiplostim to Manage Treatment Related Thrombocytopenia in Pediatric Solid Tumor Patients

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BACKGROUND

- Therapy related thrombocytopenia (TRT): thrombocytopenia due to cancer-directed therapy that persisted <u>despite adequate recovery time</u> and/or resulted in <u>treatment modifications</u>

- Associated with both reduced relative dose intensity of chemotherapy treatment as well as increased platelet transfusion requirements

- Thrombopoietin receptor (TPO-R) agonists have been used for adult CIT and pediatric immune thrombocytopenia (ITP) with reassuring safety profile and clinical outcomes

METHODS

- **Design:** Retrospective study at MSKCC between 2010-2022

- **Target population:** Solid tumor patients who received romiplostim (Table 1)

- **Endpoints:** Impact of romiplostim on TRT measured through:
  - (1) the median platelet count during romiplostim treatment
  - (2) the 'time-to-platelet count recovery' after initiation of romiplostim (defined by platelet count of ≥75 µ/L)
  - Safety data

RESULTS

FIGURE 1: Median platelet count response to romiplostim
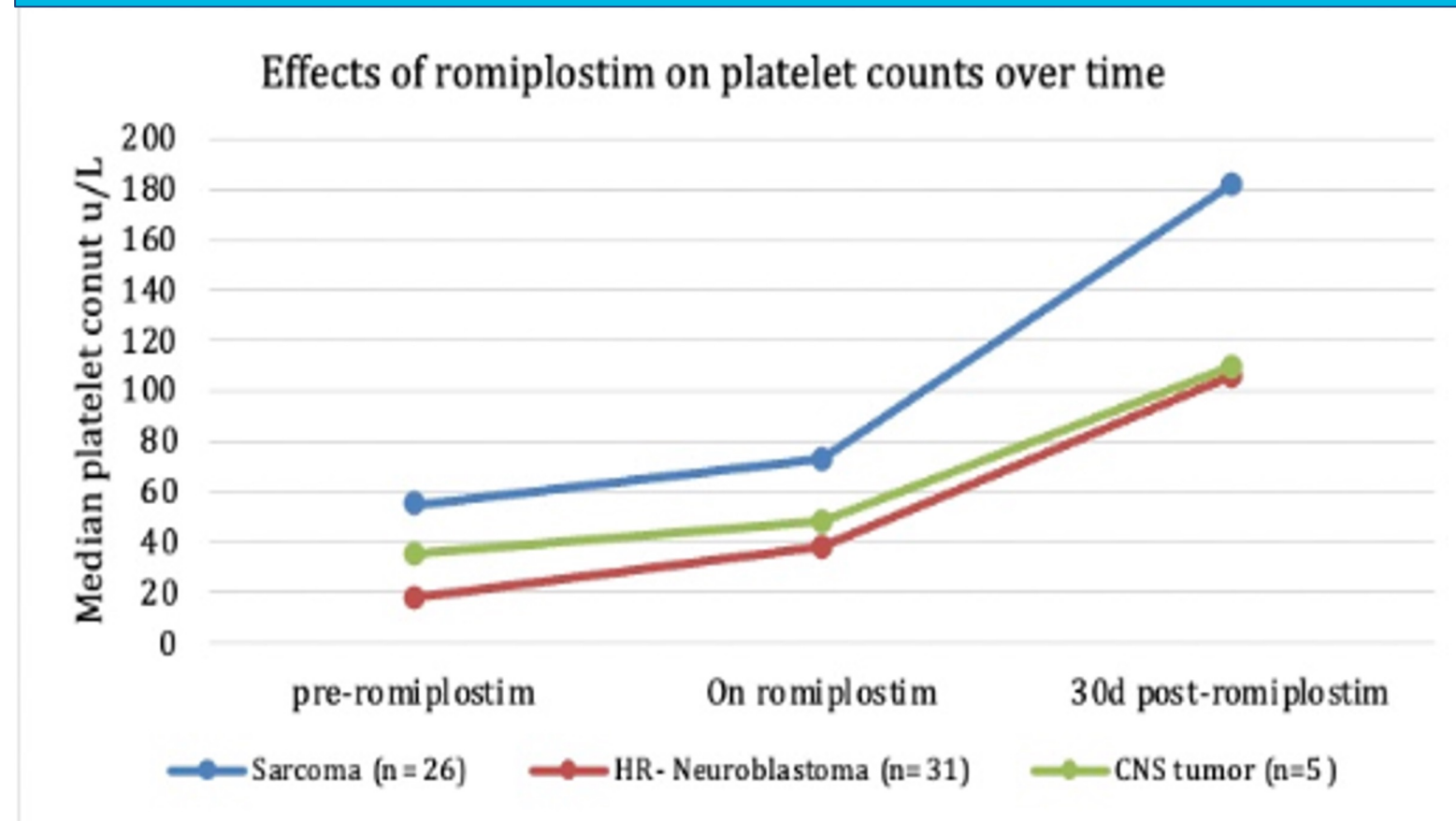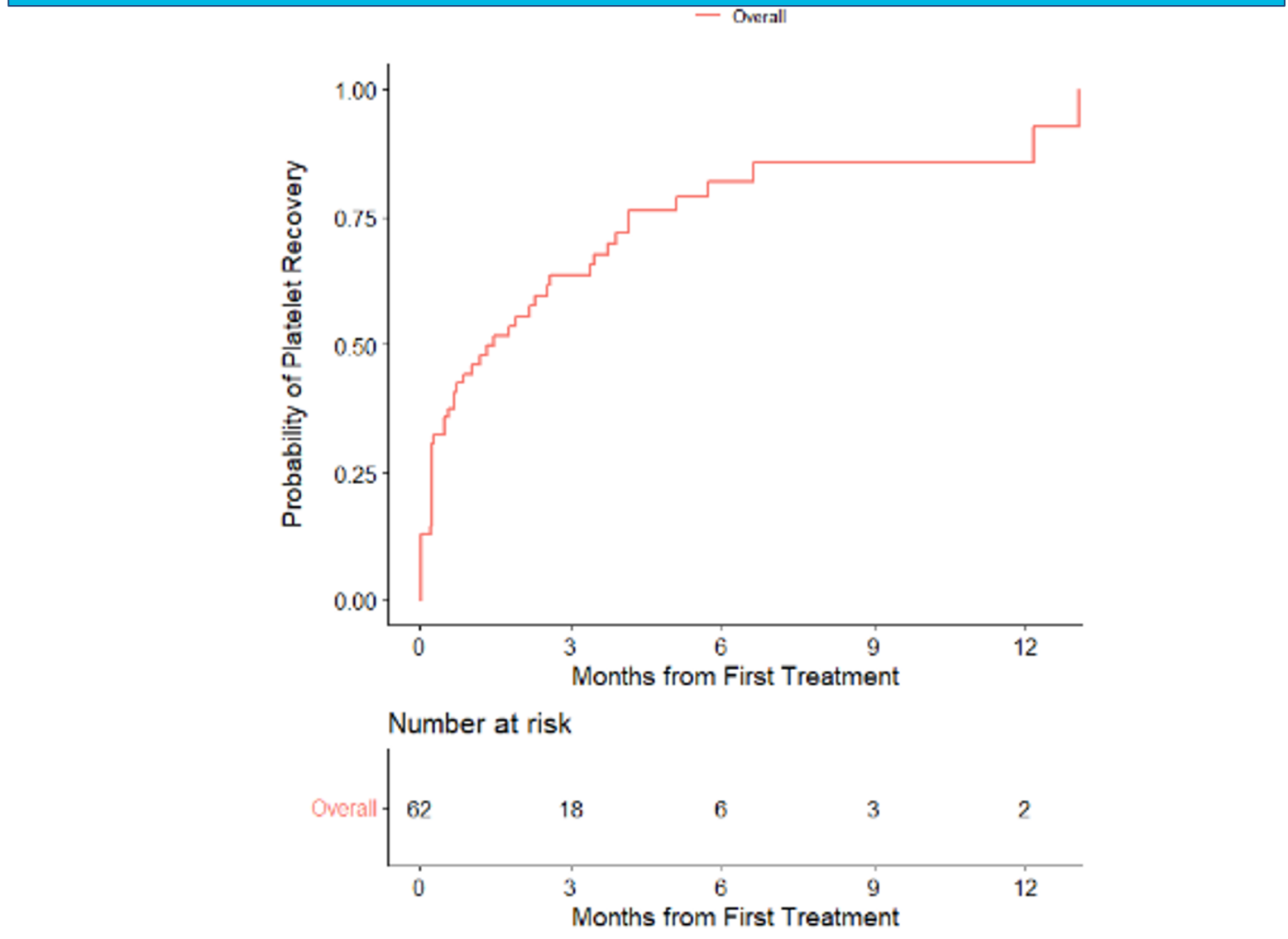

TABLE 1: Baseline Demographics

	Overall (N=62[1])	Pediatric age (< 18yo) (N=52[1])	Adult age (≥18yo) with pediatric diagnosis (N=10[1])
Age at diagnosis (years)	8.5 [1.0 – 30.0]	7.0 [1.0-16.0]	21.0 [19.0-30.0]
Histologic diagnosis:			
High Risk Neuroblastoma	31 (50%)	31 (50%)	0 (0%)
Sarcomas	26 (42%)	18 (29%)	8 (13%)
Ewing sarcoma	8 (13%)	5 (8%)	3 (5%)
Osteosarcoma	10 (16%)	7 (11%)	3 (5%)
Rhabdomyosarcoma	3 (5%)	3 (5%)	0 (0%)
Miscellaneous (spindle cell sarcomas, Wilms tumor)	5 (8%)	3 (5%)	2 (3%)
CNS tumors	5 (8%)	3 (5%)	2 (3%)
Treatment subclassifications at time of romiplostim:			
High Risk Neuroblastoma			
Chemotherapy	14 (23%)	14 (23%)	0 (0%)
Immunotherapy	5 (8%)	5 (8%)	0 (0%)
Chemoimmunotherapy	4 (6%)	4 (6%)	0 (0%)
Radionuclide therapy	8 (13%)	8 (13%)	0 (0%)
Sarcomas			
Chemotherapy	24 (39%)	17 (27%)	7 (11%)
Radiotherapy	1 (2%)	1 (2%)	0 (0%)
Immunotherapy	1 (2%)	0 (0%)	1 (2%)
CNS tumors			
Chemotherapy	3 (5%)	1 (2%)	2 (3%)
Radioimmunotherapy	2 (3%)	2 (3%)	0 (0%)

[1] Statistics presented: N (%); mean [minimum – maximum]

FIGURE 2: Probability of platelet recovery over time
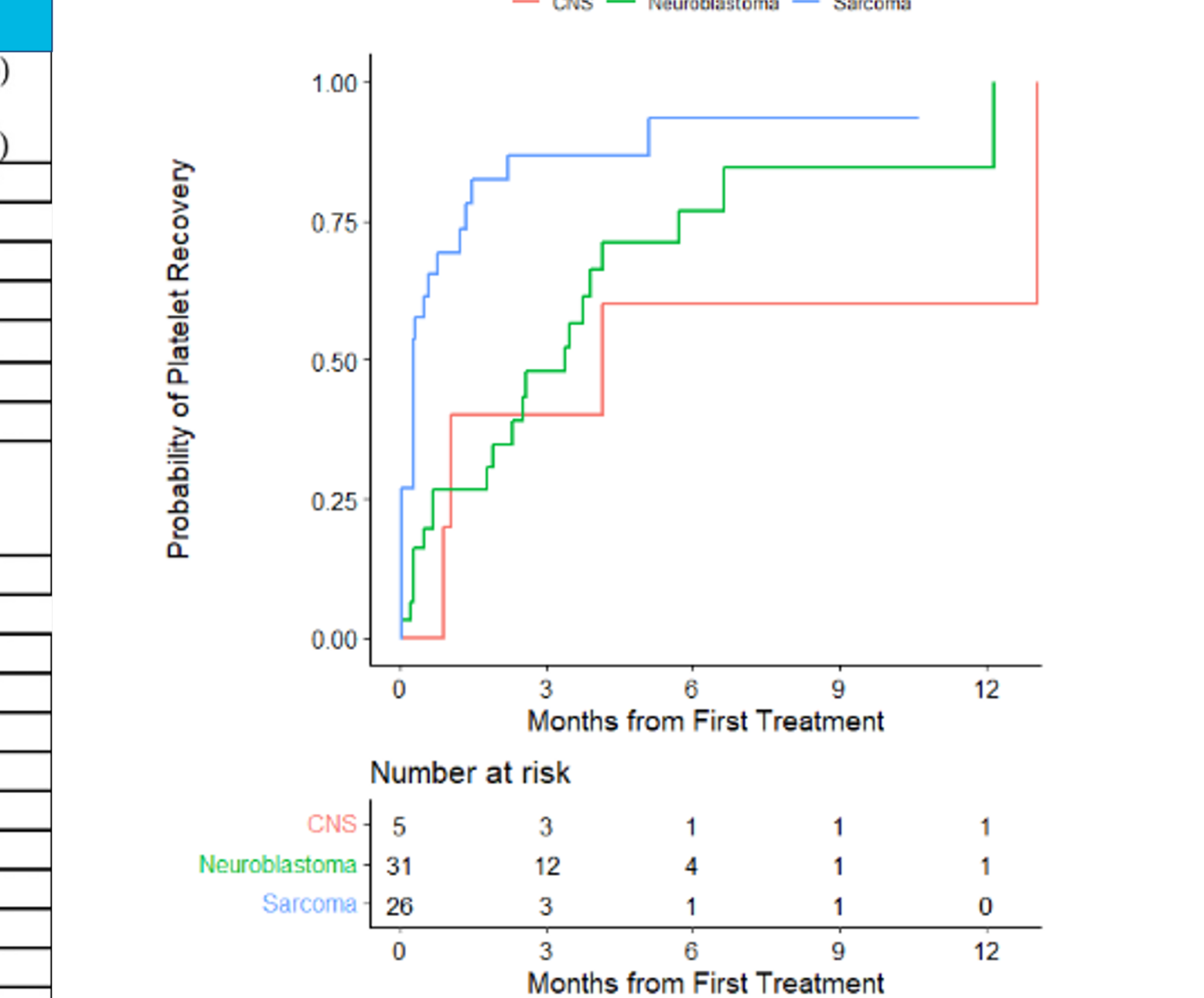
RESULTS

- The overall median time on romiplostim was 4-5 months

- The median starting dose was 3mcg/kg and median maximum dose was 8mcg/kg

- Disease subgroup was a predictor of platelet counts while on romiplostim (Figure 1)

- Median measured time-to-recovery was 45 days (Figure 2)

CONCLUSIONS

- Romiplostim used off-label in pediatric solid tumors has shown to be effective in increasing platelet counts over time and assisting in the platelet recovery

- Regardless of time treated with romiplostim, side effect profile was reassuring

NEXT STEPS

- Plan to perform a multi-institutional review to expand data pool

- Gathering more data on platelet transfusions and RDIs while on romiplostim