## SUPPLEMENTARY DATA

## Results

*Patients and Donors*

Over the same time frame, we did also see patients in whom a haploidentical donor was not utilized, either due to lack of available healthy family member or "older" patients who did have a fully matched sibling. We used the identical conditioning and GVHD platform given the greater potential for GVHD in patients over age 25 years or with the use of an unrelated donor. Ten patients were treated in this way. The median age of the patients at the time of transplant was 29 (range 23-61) years and 60% were male. The SAA was presumed acquired in all patients, with the exception of patient 1 who had measured telomeres less than the first percentile in length for age by Flow-FISH[33] but no genetic mutation identified. At the time of transplant, 100% of the patients had evidence of clonality, including the short telomeres. No patients had HLA antibodies requiring apheresis against their donors. All patients were transfusion dependent of red cell and platelets at the time of transplant. The median age of the donors was 29 (range 23-42) years and 80% of the donors were male. Of the patients, three self-identified as non-white. All patients had good performance status: Karnofsky >70 % and ECOG 0 or 1 at time of transplant. The median time from diagnosis until transplant was 12 (3-165) months as they are a mix of R/R and TN patients.

*Toxicity*

All patients are alive and well, and nine are fully engrafted with 100% donor chimerism. One patient with only an URD suffered secondary GF in setting of inability to tolerate mycophenolate and calcineurin inhibitor as well as an adenovirus infection at Day 200. Of the four TN patients, the latter three received 400cGy TBI.

*Grafts, Engraftment, and Chimerism*

The marrow grafts had a median nucleated cell count of 4.08 $x10^8$ /kg recipient ideal body weight (range, 2.4-5.85 $x10^8$), a median CD34+ cell count of 3.58 $x10^6$ /kg recipient ideal body weight (range, 1.9-8.3 $x10^6$) and a median CD3+ cell count of 3.43 $x10^7$/kg recipient ideal body weight (range, 2.8-8.38 $x10^7$).

The median time to neutrophil recovery over 1000 × $10^3$/$mm^3$ for 3 consecutive days was very similar to the haplo grafts 17 (range, 14-41) days. The median time to red cell engraftment in all patients was 30 days (range, 20-115 days) and the median time to last platelet transfusion to keep platelets counts over 50 × $10^3$/$mm^3$ was 29 days (range, 22-180 days). Supplementary table 2 shows the engraftment data including the chimerism values obtained in peripheral blood or marrow.

*Clinical outcomes in treatment-naïve SAA patients using non-haploidentical donors*

With a median follow-up of 19 (range 8-71) months (by reverse Kaplan Meier method), for the entire group. The patient with secondary graft failure remains transfusion dependent but infection free. At the time of writing, only a single patient remains on immunosuppression and he is planned to stop at 1 year. All evidence of clonal hematopoiesis (PNH clones or karyotypic abnormalities) present before BMT resolved after transplant.

## Tables

Supplementary Table 1A
Baseline characteristics of patients treated with matched related and unrelated donors or mismatched unrelated donors

Supplementary Table 1B
Engraftment Data and clinical outcomes of patients treated with matched related and unrelated donors or mismatched unrelated donors

Supplementary Table 1A

| # | Age/ Sex | Previous therapy | Severity | Clonality | | Donor | ABO Compatbility | CMV status | TBI Dose |
|---|---|---|---|---|---|---|---|---|---|
| | | | | PNH | MK | | | | |
| 1 | 38 F* | Danazol | SAA | - | 46, XX Short telomeres | URD, 9/10 | Matched | Matched | 200 |
| 2 | 25 F | HiCY | vSAA | - | 46, XX | URD, 10/10 | Mismatched | Matched | 200 |
| 3 | 23 M | HiCY | vSAA | + | 46, XY | URD, 10/10 | Matched | Mismatched | 200 |
| 4 | 25 F | ATG/ CsA x3 | SAA | + | 46, XX | URD, 10/10 | Matched | Mismatched | 200 |
| 5 | 61 F | ATG/ CsA, splenectomy | vSAA | + | 46, XX | URD, 10/10 | Matched | Mismatched | 200 |
| 6 | 24 M | ATG/ CsA/ EPAG | vSAA | + | 46, XY | URD, 10/10 | Matched | Mismatched | 200 |
| 7 | 38 M | None | SAA | + | 46, XY | Sister, 34 F | Matched | Matched | 200 |
| 8 | 44 M | None | vSAA | + | 46, XY | Brother, 42 M | Matched | Mismatched | 400 |
| 9 | 32 M | None | vSAA | + | 46, XY | Sister, 29 F | Matched | Mismatched | 400 |
| 10 | 23 M | None | SAA | + | 46, XY | URD, 9/10 | Matched | Mismatched | 400 |

Supplementary Table 1B

| # | Age/ Sex | Neutrophil engraftment | Red cell engraftment | Platelet engraftment | Day 60 T Cell | Day 60 PB | Day 360 T Cell | Day 360 PB | Graft Failure | aGVHD | cGHVD | CMV reactivation | EBV reactivation | Clonality PNH | Clonality MK | Alive | Follow up (Months) |
|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|
| 1 | 38 F | 17 | 20 | 22 | 100 | 100 | 100 | 100 | No | No | No | No | No | - | 46 XY | Yes | 71 |
| 2 | 25 F | 16 | 25 | 26 | 94 | 100 | 100 | 100 | No | No | No | No | No | - | 46 XY | Yes | 48 |
| 3 | 23 M | 20 | 27 | 33 | 95 | 95 | 100 | 100 | No | No | No | Yes | No | - | 46 XY | Yes | 44 |
| 4 | 25 F | 41 | 115 | 180 | 22 | 100 | 80 | 95 | No | No | No | No | Yes | - | 46 XY | Yes | 27 |
| 5 | 61 F | 17 | 37 | 29 | 100 | 100 | 100 | 100 | No | No | No | No | No | - | 46 XY | Yes | 12 |
| 6 | 24 M | 20 | NR | NR | 82 | 95 | | | Secondary (Adenovirus) | No | No | No | Yes | - | 46 XY | Yes | 8 |
| 7 | 38 M | 15 | 31 | 26 | 100 | 100 | 100 | 100 | No | No | No | No | No | - | 46 XY | Yes | 28 |
| 8 | 44 M | 16 | 30 | 27 | 100 | 100 | | | No | No | No | No | No | - | 46 XY | Yes | 7 |
| 9 | 32 M | 15 | 40 | 52 | 100 | 100 | | | No | No | No | Yes | No | - | 46 XY | Yes | 7 |
| 10 | 23 M | 14 | 29 | 32 | 100 | 100 | | | Yes | Yes | No | No | No | - | 46 XY | Yes | 9 |