Supplemental Table. Cardiac and laboratory parameters before and following HSCT in 41 SS or SS B0 patients with successful transplants

| Parameter | Baseline | 3 months | 6 months | One Year |
|---|---|---|---|---|
| BSA (m$^2$) | 1.87 ± 0.21 | 1.87 ± 0.23 | 1.89 ± 0.22 ^ | 1.92 ± 0.23*** |
| SBP (mmHg) | 120 ± 14 | 123 ± 12 | 122 ± 15 | 121 ± 16 |
| DBP (mmHg) | 64 ± 12 | 71 ± 10*** | 72 ± 10*** | 71 ± 12* |
| Heart Rate (beats/min) | 75 ± 14 | 74 ± 13 | 78 ± 15 | 76 ± 15 |
| TR (cm/sec) | 2.6 ± 0.34 | 2.5 ± 0.20 | 2.5 ± 0.27 | 2.4 ± 0.29* |
| RVSP (mmHg) | 33 ± 7.2 | 30 ± 4.4 | 31 ± 5.8 | 29 ± 5.5 |
| LVEDD (mm) | 51 ± 4.9 | 49 ± 4.6* | 48 ± 4.8*** | 48 ± 4.9*** |
| LVESD (mm) | 33 ± 4.2 | 34 ± 5.3 | 33 ± 6.5 | 33 ± 4.0 |
| LVEDV / BSA (mL/m$^2$) | 83 ± 22 | 69 ± 21*** | 68 ± 20*** | 65 ± 18*** |
| LV Mass Index (gm/m$^2$) | 101 ± 27 | 90 ± 25 | 86 ± 25*** | 89 ± 22** |
| LA Volume Index (mL/m$^2$) | 46 ± 14 | 36 ± 18* | 38 ± 15*** | 36 ± 12*** |
| EF (%) | 65 ± 4.9 | 57 ± 5.1*** | 58 ± 6.1*** | 60 ± 5.2*** |
| CO (L/min) | 6.1 ± 1.9 | 5.1± 1.2 | 5.7 ± 1.6 | 5.6 ± 1.9 |
| E/A | 1.7 ± 0.6 | 1.5 ± 0.5 | 1.4 ± 0.5* | 1.5 ± 0.5 |
| Sep E/e' (cm/sec) | 10.8 ± 2.6 | 9.3 ± 2.3*** | 8.7 ± 2.0*** | 9.1 ± 2.0*** |
| Lat E/e' (cm/sec) | 16 ± 3.9 | 15 ± 4.6 | 14 ± 4.2 | 14 ± 3.2* |
| Hemoglobin (g/dL) | 9.5 ± 0.9 | 11.3 ± 2.0*** | 11.7 ± 2.1*** | 12.3 ± 1.9*** |
| Total bilirubin (mg/dL) | 2.1 ± 3.1 | 0.4 ± 0.3*** | 0.6 ± 0.3*** | 0.5 ± 0.3*** |
| Serum creatinine (mg/dL) | 1.0 ± 1.5 | 1.4 ± 2.3*** | 1.5 ± 3.1*** | 1.4 ± 2.8*** |
| Reticulocyte count (%) | 3.5 ± 2.8 | 2.1 ± 1.5*** | 2.2 ± 1.3 | 2.0 ± 1.2 |
| LDH (U/L) | 338 ± 122 | 248 ± 80*** | 253 ± 127*** | 208 ± 61*** ^^ |
| 6 minute walk (meters) | 509 ± 81 | 501 ± 98 | 525 ± 113 | 544 ± 83** |

*p≤0.05, **p≤0.01, ***p≤0.005 for follow-up studies compared with baseline echo
^p≤0.05, ^^p≤0.01, ^^^p≤0.005 for 3 month vs 6 month studies and 6 month vs 12 month
BSA = body surface area; SBP = systolic blood pressure; DBP = diastolic blood pressure; TR = tricuspid regurgitation; RVSP = right ventricular systolic pressure; LVEDD = left ventricular end diastolic diameter; LVESD = left ventricular end systolic diameter; LVEDV = left ventricular end diastolic volume; LV = left ventricular; LA = left ventricular; EF = ejection fraction; CO = cardiac output; LDH = lactic acid dehydrogenase

Supplemental Figure 1. Individual changes in log NT-proBNP in successful (A) vs unsuccessful HSCT (B)
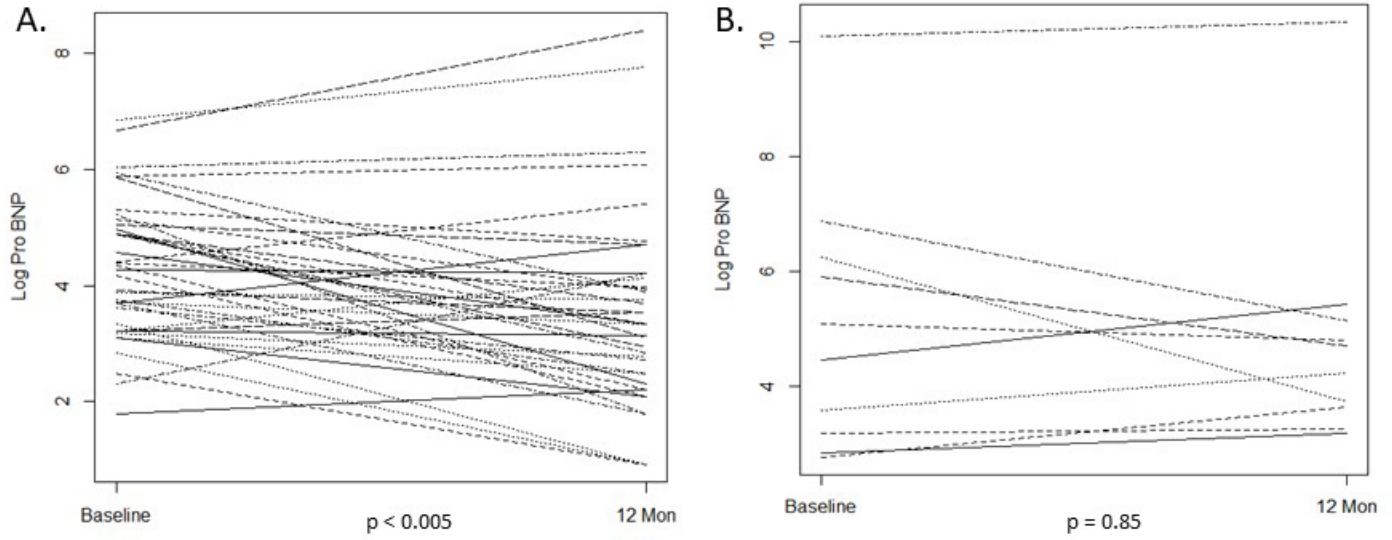
Supplemental Figure 1: Log NT-proBNP levels improved significantly in the successfully transplanted group. The small number of unsuccessful transplants did not have a significant change in their log NT-proBNP levels.

## Supplemental Figure 2. Hemoglobin and LVEDV/BSA by HSCT type in patients with successful transplants
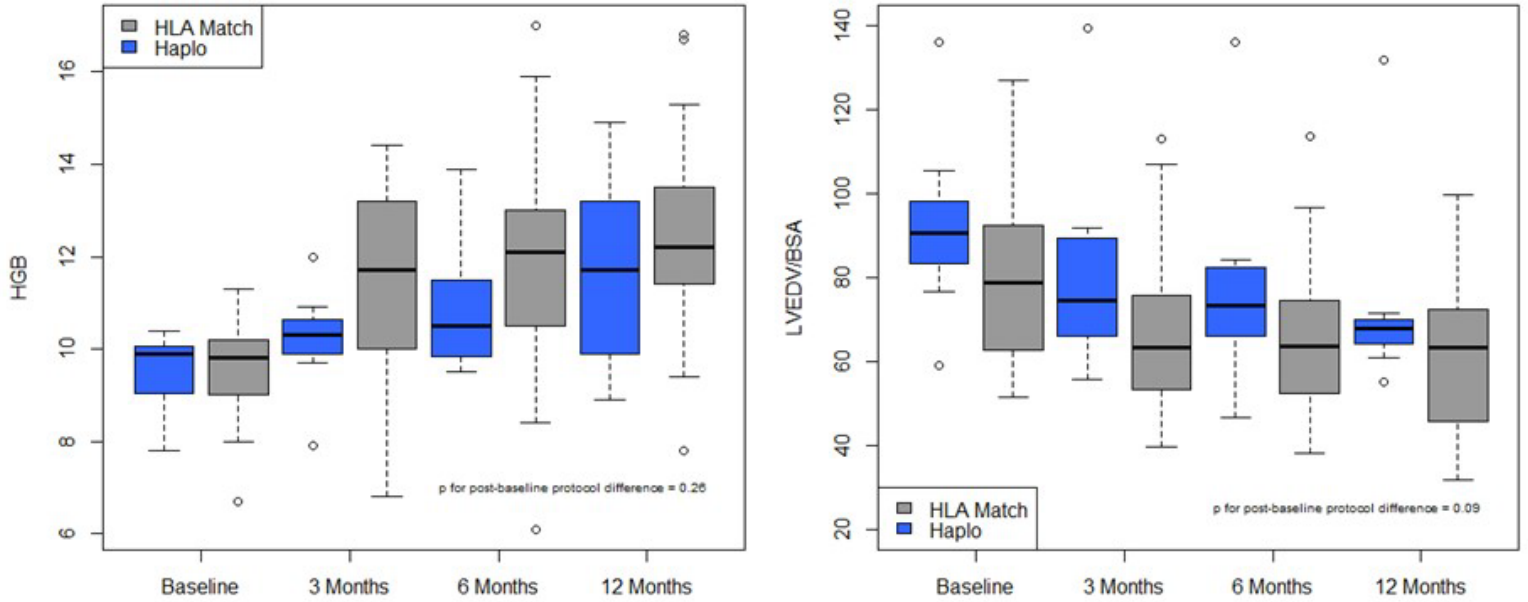


Supplemental Figure 2: The type of transplant (HLA Match vs Haplo) did not determine differences in hemoglobin levels or LV size in successfully transplanted patients.

**<u>Appendix A.</u> Comparison of successful transplants vs unsuccessful transplants using a multivariate model.**

Changes from baseline to 1 year were compared between the successful and unsuccessful transplant groups. The Figure below shows the average percent change in the two groups for several structural and functional outcomes. Except for the six minute walk distance, decreases in the percent change are consistent with patient benefit over time. For these outcomes the successful group appears to have better changes although for some of the outcomes the group difference was relatively small (e.g. LVEDD, LAVI, and 6MWD). Statistical comparisons of the individual outcomes were not significant for six of the seven outcomes, however, the p-value for the Sep E/e' difference was 0.002 (based on an analysis of covariance test). The small sample size of the unsuccessful group (N=12) lowers the statistical power for these comparisons.



Percent Change from Baseline to 12 Months
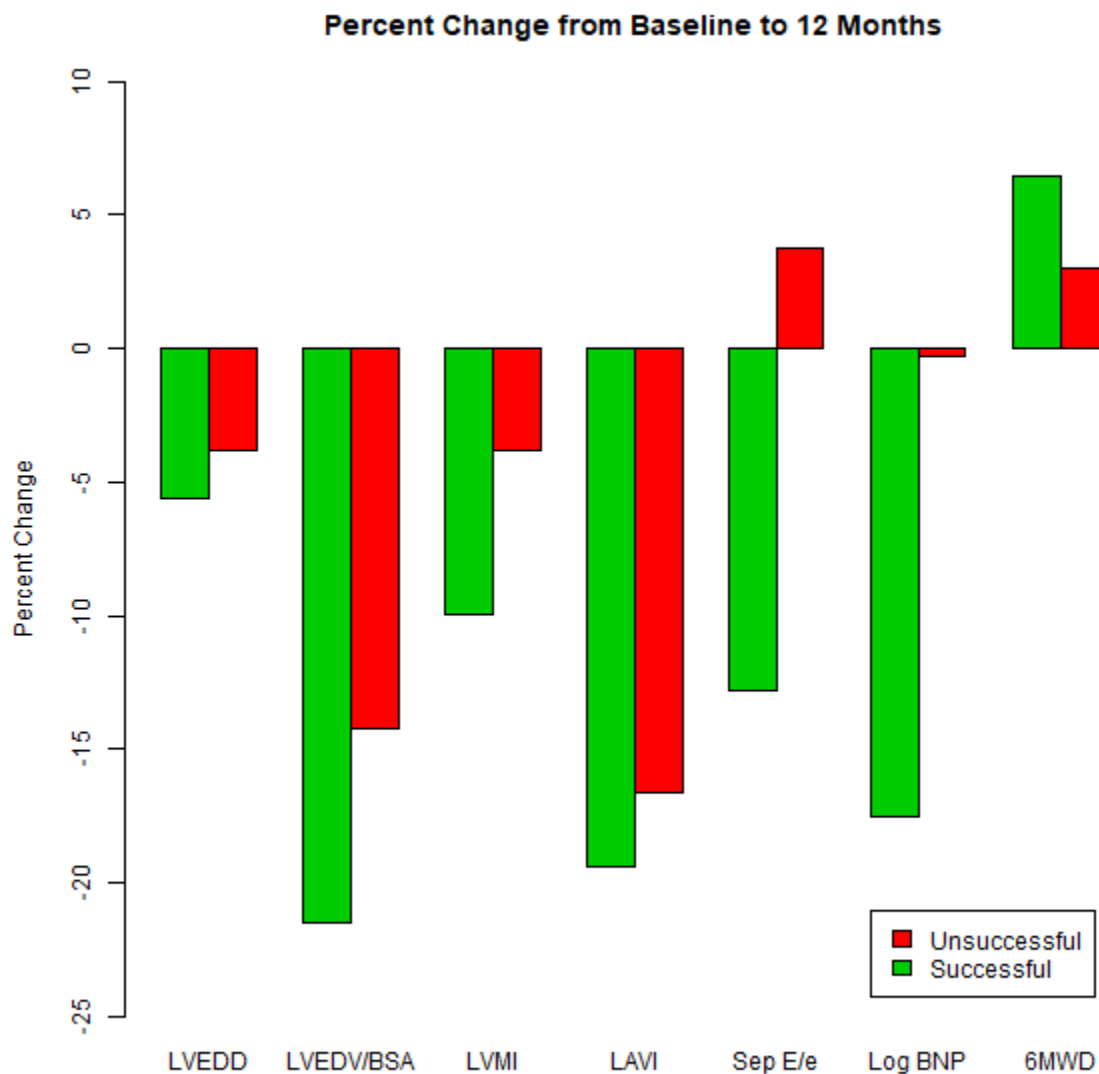
Given that each of the differences in these outcomes favored the successful group, a multivariate model was used to estimate a common successful/unsuccessful difference in the percent change

across the outcomes; the modeling of data across the outcomes can generate greater statistical power. A linear mixed effects regression model was used to model each individual's percent change as an outcome with explanatory variables of successful/unsuccessful, type of outcome (i.e. which of the seven variables), gender, and age at baseline. (For 6MWD we used -1 X percent change in 6 MWD so more negative values of percent changes were thought to be beneficial for all seven outcomes). The model is multivariate because each individual contributes their data from multiple outcomes. Regression output is shown in Table Z

Table Z – Estimated Percent Change Explained by Output Variable, Gender, Age and Success Status

|  | Estimate | Std Error | t-value | p-value |
|---|---|---|---|---|
| LVEDD | 2.54 | 4.31 | 0.59 | 0.56 |
| LVEDV/BSA | -12.07 | 4.37 | -2.76 | 0.01 |
| LVMI | -0.95 | 4.31 | -0.22 | 0.83 |
| LAVI | -11.43 | 4.38 | -2.61 | 0.01 |
| Sep E/e | -1.85 | 4.32 | -0.43 | 0.67 |
| Log BNP | -6.19 | 4.40 | -1.41 | 0.16 |
| 6MWD | 3.48 | 5.11 | 0.68 | 0.50 |
| Male | -1.61 | 3.20 | -0.50 | 0.62 |
| Age | 0.43 | 0.16 | 2.76 | 0.01 |
| Successful | -8.52 | 3.95 | -2.16 | 0.04 |

The first 7 rows of the table show estimated percent changes for the unsuccessful group in the seven output variables. Gender is not a significant factor; each additional year of age is associated with an increase of 0.43 percent change. The last line indicates those in the successful group have an estimated percent change that 8.5% more negative than those in the unsuccessful group for each outcome, and a p-value of 0.04. Further testing was done to see if an interaction term between the output type (e.g. LVEDD, LDEVD/BSA, … , 6MWD) and Success/Unsuccessful status was warranted however there was not support for the interaction (p = 0.63). The interpretation is that there is marginal evidence that for these outcome variables the magnitude of the percentage change for the successful group is greater (by approximately 8.5%) than the percentage change in the unsuccessful group. The model allows for different estimated percentages for the 7 different variables but estimates a constant difference of 8.5% between the two groups (e.g. 2.5% and -6.0% change for LVEDD in the unsuccessful and successful groups, and -12.1% and -20.6% for LVEDV/BSA).