

## **Supplementary File**

### **Methods:**

#### **Mutation analysis and WASp expression**

Mutation analysis was performed by PCR-based amplification of genomic DNA using primers spanning the WASp gene. WASp expression was analyzed using western blot) or flow cytometry (FACs) (12).100µl of peripheral blood was collected in EDTA, fixed and permeabilized using Caltag fix buffer and permeability Buffers A and B according to manufactures protocol. 1 ul of anti- WASp or IgG2a isotype control (both BD Biosciences) was added, cells were incubated for 30 minutes at room temperature and then washed with PBS + 5% fetal calf serum (both Invitrogen) . Then, 1 ul biotinylated goat anti-mouse IgG2a (Southern Biotech) was added, cells were incubated for 15 minutes, washed and then 1 ul of Streptavidin-APC (BD Biosciences) was added. Cells were further incubated for 15 minutes, washed, and fixed (CellFix BD Biosciences). 10,000 lymphocyte events were acquired on a FACs Calibur and analyzed using CellQuest software (BD Biosciences). Lymphocytes were gated, and WASp expression was compared to the IgG2a control. Normal values were defined as 80% or more WASp expression above the IgG2a. Values below 5% was considered as absent WASp expression.

#### **Disease severity:**

Disease severity before transplantation was expressed as a score of 1 to 5 according to Ochs and Thrasher, 2006 and Ochs et al, 2009

#### **References:**

1. Ochs HD, Thrasher AJ. The Wiskott-Aldrich syndrome. *J Allergy Clin Immunol.* 2006; 117: 725-738.
2. Ochs HD, Filipovich, AH, Veys P, Cowan MJ, and Kapoor N. Wiskott-Aldrich syndrome: diagnosis, clinical and laboratory manifestations, and treatment. *Biol. Blood Marrow Transplant.* 2009; 15, 84–90.

#### **Chimerism analysis:**

Lineage specific chimerism was assessed using polymerase chain reaction amplification of specific polymorphic DNA sequences (short tandem repeats) in circulating lymphoid and myeloid cells.

#### **Statistical Analysis:**

Mean WAS scores and platelet counts were compared using a Student t-test. Kaplan-Meier survival curves were plotted and log-rank P values were determined for differences in rate of complications. Spearman rank correlation coefficient used to test the association between myeloid/lymphoid donor engraftment and platelet count. Graphprism 7 was used to plot the

kinetics of donor T, donor B and donor myeloid cell engraftment at different time points post-transplant. Significance was considered at  $P < 0.05$ .

**Ethical approval:**

All patients provided transplant consent according to UK paediatric BMT group regulations.