

Supplementary file 1: Details on the four HSCT

1 – HSCT 10.09.2008

Conditioning regimen: Fludarabin + Busulfan + Thiotepa

GVHD profilaxis: Cyclosporine

Cells infused (HLA-identical sister as donor): $4.2 \times 10^8/\text{kg}$

Engraftment: 500 neutrophils/ μl at day + 15

2 – HSCT 27.10.2010

Conditioning regimen: Aracytine + Melphalan

GVHD profilaxis: Cyclosporine

Cells infused (HLA-identical sister as donor): $3.5 \times 10^8/\text{kg}$

Engraftment: 500 neutrophils/ μl on day + 12

3 – HSCT 10.12.2012

Conditioning regimen: Aracytine + Melphalan

GVHD profilaxis: Cyclosporine + ATG

Cells infused (unrelated cord blood donor): $5.2 \times 10^7/\text{kg}$

Engraftment: 500 neutrophils/ μl on day + 17

4 – HSCT 27.6.2018

Conditioning regimen: Thiotepa + Threosulfan + Fludarabin + ATG

GVHD prophylaxis: alpha/beta T lymphocyte and CD+19 in vitro depletion

Cells infused (partially matched mother as donor): TCN $2.2 \times 10^8/\text{kg}$, CD34+ $6.11 \times 10^6/\text{kg}$, T CD3+ $5.94 \times 10^6/\text{kg}$, TCR $\alpha\beta$ + $22 \times 10^3/\text{kg}$, TCR $\gamma\delta$ + $5.94 \times 10^6/\text{kg}$

Engraftment: day +9, platelet engraftment day +17