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### Allogeneic hematopoietic stem cell transplantation with reduced intensity conditioning in children with inherited diseases: a single centre experience

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#### Abstract

**Background:** Allogeneic hematopoietic stem cell transplantation (alloHSCT) is an effective approach to treatment of some inherited diseases, but the efficacy of different conditioning regimens (CR) is controversial. Conventional myeloablative conditioning (MAC) is associated with high levels of treatment related toxicity (TRT) and with significant treatment related mortality (TRM). Reduced intensity conditioning (RIC) significantly decreases the development of different complications and reduces TRM. But RIC is associated with an increased frequency of graft failure or allograft rejection in these diseases.

**Purpose:** To estimate the efficacy of RIC in patients (pts) with inherited diseases.

**Patients:** From May 2006 to May 2010 MUD alloHSCT was performed in 11 pts with the following diseases: Wiscott-Aldrich syndrome, 2, Krabbe disease, 1, metachromatic leukodystrophy (ML), 1, X-linked adrenoleukodystrophy (xALD), 1, malignant osteopetrosis, 1, Diamond-Blackfan anemia (DBA), 1, Hurler syndrome, 1, and Fanconi anemia (FA), 3. Median pt age was 5.9 years. We used Fludarabine (Flu) 150 mg/m<sup>2</sup> + Melphalan 140 mg/m<sup>2</sup> in 5 pts; Flu 150 mg/m<sup>2</sup>+Cyclophosphamide (Cy) 600 mg/m<sup>2</sup> in 1 pt with DBA; and Flu 150 mg/m<sup>2</sup>+Cy 40 mg/kg in 3 pts with FA. aGvHD prophylaxis was by CsA + MTX + ATGAM.

**Results:** Three-year overall survival is 75%. Ten pts engrafted on D+14 (range D+12–19) with full donor chimerism achievement on D+30. 8 pts are alive without any signs of disease with follow-up at 1–49 months. The pt with ML died on D+37 from progression disease; 2 pts (1 with FA, 1 with xALD) died from severe gut aGvHD. During RIC nobody developed severe toxicity (III–IV st according WHO classification).

**Conclusion:** RIC is effective in children with inherited diseases, has low TRT and is a promising alternative to MAC.

**Keywords:** alloHSCT, inherited diseases, reduced intensity conditioning

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