m^2. Patients undergoing unrelated donor transplants received antithymocyte globulin, 30 mg/kg, on days -2, -1, and +6. The last 8 Busulfan doses were adjusted to achieve areas under the concentration × time curve of 600 – 900 micromol.min/liter. Family donor transplant recipients (n = 9), received Cyclosporine/Methotrexate/Leukovorin as GVHD prophylaxis; unrelated donor transplant recipients (n = 19) received Cyclosporine/Steroid prophylaxis; one patient underwent syngeneic BMT. With a minimum of 12 months and a median of 66 months follow-up, 19 of the 29 (66%) survive event-free and 22 of the 29 (76%) survive overall. Five patients relapsed. Three patients in relapse at the time of BMT achieved CR but relapsed at 5, 5 and 6 months. Two patients with Ph+ ALL relapsed 15 and 26 months after BMT and survive event-free 42+ and 39+ months, respectively, after 2nd transplants with TBI-containing regimens. One patient with AML in CR2 suffered rejection of an unrelated cord blood but survives event-free 63+ months after autologous stem cell infusion. Four died of transplant-related complications (multi-organ failure, disseminated adenovirus infection, enterococcal sepsis, and hemolytic-uremic syndrome with Aspergillosis). Toxicity was considerable and included mucositis requiring parental nutrition (29 of 29), hemolytic uremic syndrome (n = 5), pulmonary hemorrhage (n = 3), veno-occlusive disease (n = 3), seizures (n = 1).

Our data suggest that although this is a moderately toxic regimen, Busulfan/Melphalan shows promising activity as preparative therapy for infants and very young children with leukemias, avoids total body irradiation, and produces outcomes similar to those previously reported for patients in this age group.

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UNRELATED DONOR CORD BLOOD STEM CELL TRANSPLANTATION FOR LEUKOCYTE ADHESION DEFICIENCY (LAD)

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Leukocyte adhesion deficiency (LAD) is a rare disorder characterized by the congenital absence of CD18 resulting in the loss of expression of leukocyte CD11/CD18 integrins on myeloid cells with resultant life threatening infections. Hematopoietic stem cell transplantation currently provides the only available cure for this disorder. Although there have been reports of some success in transplantation using related family member donors or unrelated marrow donors with either myeloablative or non-myeloablative prepartative regimens, there is little experience with the use of umbilical cord SCT. We present here the first report of a patient transplanted with unrelated donor cord blood (UCB) for severe LAD type 1. The patient was diagnosed at 2 months of age when he presented with failure to thrive, fever, mastoiditis, and lethargy. Laboratory evaluation revealed a WBC of 165,000 (predominantly neutrophils) and flow cytometry documented the absence of neutrophil expression of CD11b and CD18. He was treated with antibiotics and surgical drainage of his mastoid bone. Since no family donors were identified, and given the need to proceed with transplant expeditiously and to minimize the risk of GVHD, unrelated donor cord blood transplant was pursued using a non-myeloablative preparative regimen. A large (16.5×10^7 TNC/kg and 9.5×10^5 CD34 cell/kg) 10 of 10 antigen matched unrelated donor cord blood unit was identified. The preparative regimen included Campath-1H3 (10 mg/d iv days -21 to – 19), fludarabine (30 mg/m^2/d iv days -8 to -4) and melphalan (140 mg/m^2 iv day -3). GVHD prophylaxis consisted of CSA + mycophenolate. He engrafted and was transfusion independent by day 16. He had no evidence of GVHD, and donor-host studies revealed 40% donor on day 15. His donor chimerism fell to 16% by STR on day 41 confirmed 100% recipient by VNTR. His cyclosporine was discontinued and an extensive viral screen showed no evidence of CMV, EBV, HHV6, adenovirus, or Hepatitis B. Over the next several months, his donor chimerism slowly rose to 48% where it has remained stable for the past 7 months with 44% of his granulocytes positive for CD11b/CD18. He remains clinically well off all immune suppression 13 months post-transplant with normal growth and development.

Conclusion: UCB is a viable source of stem cells for LAD patients.

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DURATION OF HOSPITALIZATION IN THE FIRST 100 DAYS FOLLOWING REDUCED INTENSITY CONDITIONING FOR NON-MALIGNANT DISORDER TRANSPLANTS IN CHILDREN

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Background and Objective: A novel reduced intensity conditioning (RIC) regimen was used for hematopoietic stem cell transplantation (HSCT) in childhood non-malignant disorders in an effort to reduce regimen related organ toxicities and the incidence and severity of graft-versus-host disease (GVHD). Recipient immunosuppression was used to achieve donor cell engraftment and post-transplant monitoring included infection surveillance until immune reconstitution. This retrospective study evaluates duration of hospitalization within the first 100 days following HSCT in RIC recipients. **Methods:** Thirty patients (7 months to 17 years) with hemoglobinopathies (4), bone marrow failure (7), immune dysfunction (11), metabolic disorders (7), and mitochondrial myopathy (1) underwent HSCT following conditioning with alemtuzumab (48 mg; day -21 to -19), fludarabine (150 mg/m^2; day -8 to -4), and melphalan (140 mg/m^2 [24] or 70 mg/m^2 [6] on day -3). Doses were lower for patients <10 kg. Stem cell sources included peripheral blood (PBSC) (4), cord cells (UCB) (5), and bone marrow (BM) (21). Seven patients (5 UCB; 2 BM/PBSC) received 1–2 antigen mismatched products. GVHD prophylaxis included a calcineurin inhibitor, methotrexate (except in 4 UCB transplants) and methyl prednisolone. **Results:** Durations of hospitalization in the first 100 days is shown in Table 1; all had donor cell engraftment. Median number of in-patient days was 22.5 for the group and was similar for unrelated and related HSCT (Table 1). UCB recipients stayed longer. Eight patients were hospitalized for >30 days (3 unrelated and 1 related BM, 4 mismatched UCB) for hemolysis (1), organ failure (3), GVHD (3) and infection. Infections which required hospitalization included bacteria (6), non-CMV viruses (3), CMV reactivation (3), CMV disease (2), and C. difficile (4). Three patients died before day 100 of GVHD (1), progressive disease (1), and infection (1). **Conclusions:** Recipients of this RIC regimen had an acceptable duration of hospitalization in the first 100 days post-transplant. Infectious complications and GVHD accounted for prolonged hospitalization (>30 days) in the majority of patients.

Table 1. *In-patient stay in the first 100 days following HSCT*

Recipient Details (number)	Median days of hospitalization (range)	Mean days of hospitalization
Total (30)	22.5 (12–88)	29.5
Alive beyond 100 days (27)	22 (12–84)	27
Died prior to day 100 (3)	51 (18–88)	51.7
UCB (all unrelated, mismatched) (5)	46 (21–88)	50.3
BM + PBSC recipients (25)	21.5 (12–84)	26.3
Unrelated BM or PBSC (HLA-matched) (11)	27 (14–84)	32.1
Unrelated BM or PBSC (mismatched) (2)	20 (15–25)	20
Matched related donors (12)	21 (12–42)	21.8