$CD3^+$ and $CD3^+CD4^+$ T cells above 500 cells/ml were achieved by 180 and 270 days, respectively. IgA and IgM levels achieved normal values by 180 days.

Conclusion: BPX-501 following $\alpha\beta$-T and B-cell depleted haplo-HSCT represents a highly effective transplantation strategy for pediatric pts with AL. Rimiducid was an effective treatment for pts with steroid-resistant GvHD.

Table 2
AL Efficacy Outcomes

Parameter (median)	*AML n = 46*	*ALL n = 54*
F/u	14.6 mos	13.2 mos
TRM	8.8%	4.8%
CR1	23.5%	12.5%
CR2+	0.0%	10.9%
RFS	84.7%	80.2%
CR1	76.5%	87.5%
CR2+	88.8%	78.5%
OS	91.2%	89.1%
CR1	76.5%	87.5%
CR2+	100.0%	89.1%

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Busulfan/Fludarabine- or Treosulfan/Fludarabine-Based Conditioning Regimen for Patients with Wiskott-Aldrich Syndrome – an EBMT Inborn Errors Working Party and Scetide Study

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Introduction: Excellent survival rates have been reported after allogeneic haematopoietic stem cell transplantation (HSCT) for Wiskott-Aldrich syndrome (WAS) patients. Recipient age >5 years in MUD HSCT as well as MMFD as donor were negative predictors for outcome. However, the vast majority of HSCTs in previously published studies were performed with (oral) busulfan/cyclophosphamide-based conditioning and in the early 2000 years or before.

Objectives: To compare OS and EFS after HSCT with either busulfan/fludarabine (BuFlu) ± thiotepa (TT) or treosulfan/fludarabine (TreoFlu) ± TT as recommended for primary immunodeficiencies since 2005 by the inborn errors working party (IEWP) of EBMT and ESID.

Methods: We performed a retrospective analysis via the EBMT and SCETIDE registries of WAS patients transplanted between 20006 and 2016 with these two regimens. At the time of this interim analysis, 174 patients were included, 92 (53%) with BuFlu±TT and 82 (47%) with TreoFlu±TT conditioning, with a median age of 1.6 years (0.2-30) at HSCT and a median follow-up of 32.9 months (1.5-128.9). Donors were MSD in 30, other MRD in 5, MUD (9/10 or 10/10) in 105, MMUD (<9/10) in 9 and MMFD in 25 (18 with ex-vivo T-cell depletion). Stem cell source was bone marrow in 93, peripheral blood in 62 and cord blood in 18.

Results: Two year overall survival (OS) of the entire cohort was 88.6% (95% c.i. 83.5%-93.6%). There was no significant difference in OS between BuFlu±TT or TreoFlu±TT conditioning (2-year OS 88.1% vs. 89.5%; p=0.7). Patients aged >5 years had a worse OS as compared to those 5 years or younger at HSCT (74.9% vs. 90.8%; p=0.005). The type of donor had no influence on OS: 96.4% for MSD/MFD, 86.8% for MUD/MMUD and 87.7% for MMFD (p=0.4). The rate of complete (≥90%) donor chimerism at last follow-up or before a secondary procedure (if a patient had one) was 41/42 (98%) in the BuFlu±TT group and 21/35 (60%) in the TreoFlu±TT group (p=0.0001). Twenty-six patients required a second procedure: stem cell boost in 4, donor lymphocyte infusion in 9, 2nd HSCT in 15 and splenectomy in 1. The 2-year cumulative incidence (CI) of second procedures was higher at 33.9% in the TreoFlu±TT versus 12.8% in the BuFlu±TT group (p=0.017), and 2-year EFS (events: second procedure or death) was 61.4% in the TreoFlu±TT and 75.0% in the BuFlu±TT group (p=0.2). Grade II-IV acute GVHD had the same incidence in both groups (24.4% vs. 26.3%; p=0.849) and chronic GVHD of any grade was borderline more frequent in the TreoFlu±TT group (17.2% vs 6.7%; p=0.054).

Conclusion: HSCT with either BuFlu±TT or TreoFlu±TT conditioning reliably cures almost 90% of patients with WAS regardless of donor type. Age >5 years at HSCT remains a negative risk factor. More patients were mixed chimeras and required second procedures after TreoFlu±TT than after BuFlu±TT conditioning. These data confirm the feasibility and efficacy of the regimens currently recommended by the IEWP.

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Individual Patient Dose-Escalated Low-Dose Interleukin-2 for Steroid-Refractory Chronic Graft-Vs.-Host Disease in Children and Adults: Safety, Efficacy and Immune Correlates

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