

A risk factor analysis of outcomes after unrelated cord blood transplantation for children with Wiskott-Aldrich syndrome

Zhanna Shekhovtsova,^{1,2} Carmem Bonfim,³ Annalisa Ruggeri,^{1,4} Samantha Nichele,³ Kristin Page,⁵ Amal AlSeraihy,⁶ Francisco Barriga,⁷ José Sánchez de Toledo Codina,⁸ Paul Veys,⁹ Jaap Jan Boelens,¹⁰ Karin Mellgren,¹¹ Henrique Bittencourt,¹² Tracey O'Brien,¹³ Peter J. Shaw,¹⁴ Alicja Chybicka,¹⁵ Fernanda Volt,¹ Federica Giannotti,^{1,4} Eliane Gluckman,^{1,16} Joanne Kurtzberg,⁵ Andrew R. Gennery¹⁷ and Vanderson Rocha^{1,18} on behalf of Eurocord, Cord Blood Committee of Cellular Therapy and Immunobiology Working Party of the EBMT, Federal University of Parana, Duke University Medical Center and Inborn Errors Working Party of the EBMT

¹Hôpital Saint Louis, Eurocord, Paris, France; ²Dmitry Rogachev National Research Centre of Pediatric Hematology, Oncology and Immunology, Moscow, Russian Federation; ³Bone Marrow Transplantation Service, Hospital de Clínicas, Universidade Federal do Paraná, Curitiba, Brazil; ⁴Service d'Hématologie et Thérapie Cellulaire, Hôpital Saint Antoine, Paris, France; ⁵Pediatric Blood and Marrow Transplantation Program, Duke University Medical Center, Durham, NC, USA; ⁶Section of Pediatric SCT, King Faisal Specialist Hospital & Research Centre-Riyadh, Saudi Arabia; ⁷Programa de Hematologia Oncologia Departamento de Pediatria, Pontificia Universidad Catolica de Chile, Santiago, Chile; ⁸Servicio de Hematología y Oncología Pediátrica, Hospital Vall d'Hebron, Barcelona, Spain; ⁹Great Ormond Street Hospital Children's Charity, London, UK; ¹⁰Pediatric Blood and Marrow Transplantation Program, University Hospital Utrecht, the Netherlands; ¹¹Department of Oncology, Hematology and Stem Cell Transplantation, The Queen Silvia Children's Hospital Gothenburg, Sweden; ¹²Hematology-Oncology Division, Centre Hospitalier Universitaire Sainte-Justine, Montréal, QC, Canada; ¹³Sydney Children's Hospital Kids Cancer Centre, Randwick, Australia; ¹⁴The Children's Hospital at Westmead, Sydney, Australia; ¹⁵Wroclaw Medical University, Poland; ¹⁶Centre Scientifique de Monaco, Monaco; ¹⁷Institute of Cellular Medicine, Newcastle University, Newcastle-Upon-Tyne, UK and ¹⁸Oxford University Hospitals NHS Trust, UK

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Correspondence: zhanna.shekhovtsova@fccho-moscow.ru

Supplemental table

Table 1. WAS scoring system to define clinical phenotypes

Score	XLT				WAS	
	0.5	1	2	3	4	5
Thrombocytopenia	-/+	+	+	+	+	+
Small platelets	-	+	+	+	+	+
Eczema	-	-	(+)	+	++	(+)/+/++
Immunodeficiency	-	-/(+)	(+)	+	+	(+)/+
Infections	-	-	(+)	+	+ / ++	(+)/+ / ++
Autoimmunity and/or malignancy	-	-	-	-	-	+

WAS indicates Wiskott-Aldrich syndrome; XLT, X-linked thrombocytopenia.

Scoring system: -/(+), absent or mild; -/+, intermittent thrombocytopenia; (+), mild transient eczema or mild infrequent infections not resulting in sequelae; +, thrombocytopenia, persistent but therapy-responsive eczema, and recurrent infections requiring antibiotics and often IVIG prophylaxis; ++, eczema that is difficult to control and severe, life-threatening infections. Because patients with XLT may develop autoimmune disorders or lymphoma (albeit at a lower rate than those with classic WAS), a progression from a score of 1 or full donor to a score of 5 is possible for XLT.

Table 2. Median and range of absolute number ($\times 10^9/L$) of CD4+, CD8+ and CD19+ at first reported test confirmed IR, compared to median and percentiles (5th to 95th percentiles) of absolute numbers ($\times 10^9/L$) of normal values of CD4+, CD8+ and CD19+; number of patients survived, achieved engraftment and IR by age groups¹.

	Absolute number ($\times 10^9/L$)	5-9 mo.		9-15 mo.		15-24 mo.		2-5 yr.		5-10 yr.		10-18 yr.	
		Healthy children	Median (range)	Healthy children	Median (range)	Healthy children	Median (range)	Healthy children	Median (range)	Healthy children	Median (range)	Healthy children	Median (range)
CD4+	4.3	2.8 (1.4-5.1)	2.02 (1.9-2.1)	2.3 (1.4-4.6)	1.35 (0.7-2.6)	2.2 (0.9-5.5)	1.33 (0.09-3.9)	1.3 (0.5-2.4)	0.6 (0.28-2.7)	1.0 (0.3-2.0)	0.8 (0.3-1.2)	0.8 (0.4-2.1)	0.8 (0.4-2.1)
CD8+	1.75	1.1 (0.6-2.2)	0.8 (0.6-5.2)	1.1 (0.4-2.1)	0.4 (0.17-1.4)	1.2 (0.4-2.3)	0.6 (0.04-6.2)	0.8 (0.3-1.6)	1.05 (0.1-1.2)	0.8 (0.3-1.8)	0.36 (0.02-1.5)	0.4 (0.2-1.2)	0.4 (0.2-1.2)
CD19+	1.49	1.3 (0.7-2.5)	1.41 (0.3-1.4)	1.4 (0.6-2.7)	1.4 (0.89-2.7)	1.3 (0.6-3.1)	1.09 (0.001-2.2)	0.8 (0.2-2.1)	0.95 (0.5-2.1)	0.5 (0.2-1.6)	0.79 (0.03-1.7)	0.3 (0.2-0.6)	0.3 (0.2-0.6)

References

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