

List of current HSCT and Gene Therapy Studies within ERN RITA

Study Title (short)	Study Design	Sponsor	PI(s)	Status	Comment
HSCT for LAD	Retrospective		Shahrazad Bakhtiar, Peter Bader, Andy Gennery	manuscript in preparation	
Domino transplants	Retrospective		Marc Bierings	manuscript submitted	
HSCT for porphyria	Retrospective		Ayman Saad	data collection	Combined EBMT/CIBMTR
HSCT for CGD	Retrospective		Robert Chiesa, Mary Slatter, Tayfun Güngör	data collection	
Conditioning for HSCT for WAS (BuFlu vs TreoFlu)	Retrospective		Michael Albert	data collection	
CBT in inborn errors of metabolism	Retrospective		Robert Wynn, Jaap Jan Boelens, Su Han Lum	data collection	
HSCT for HLH in adults	Retrospective		Rafał Machowicz, Nicolaus Kröger	data analysis	
HSCT for LAL-D (Wolman's Diseases)	Retrospective		Robert Wynn, Su Han Lum, Simon Jones	in preparation	
P-CID (profound combined immunodeficiency)	Prospective		Stephan Ehl	recruiting	
SCID-SZT 2016 (HSCT registry for SCID)	Prospective		Manfred Hönig	recruiting	Germany, Austria, Switzerland only
ADA SCID Gene Therapy	Prospective	GSK	Alessandro Aiuti	Enrolment complete (data collection)	
WAS Gene Therapy	Prospective	GSK	Alessandro Aiuti	Enrolment complete (data collection)	
XCGD Mobilization	Prospective		Fabio Ciceri, Franco Locatelli	Recruiting	San Raffaele Hospital, Milan and Bambino Gesù Hospital, Rome
Stem-cell based gene therapy for recombination deficient SCID (RECOMB)	Prospective	EU	Frank Staal, Arjan Lankester	not yet recruiting	