Cellular Therapy and Transplantation (CTT), Vol. 2, No. 5, 2009
doi: 10.3205/ctt-2009-No5-abstract05

Abstract accepted for "Joint EBMT Pediatric Working Party – 3rd Raisa Gorbacheva Memorial Meeting on Hematopoietic Stem Cell Transplantation", Saint Petersburg, Russia, September 17–20, 2009

Hematopoietic stem cell transplantation (HSCT) in patients with severe combined immunodeficiency (SCID): experiences, possibilities, and prospects

Dmitry N. Balashov, Pavel E. Trakhtman, Elena V. Skorobogatova, Yulia V. Skvortsova, Zarema M. Dyshlevaja, Irina P. Shipitsina, Alexey A. Maschan, Alexander G. Rumyantsev

Federal Research and Clinical Centre of Pediatric Hematology, Oncology and Immunology, Moscow, Russia

Correspondence: Dmitry N. Balashov, 117/2, Leninsky prosp, Moscow, Russia, 105062, Phone: +7 (495) 936-9023, Fax +7 (495) 936-9120, E-mail: bala8@yandex.ru

Abstract

HSCT is the standard, and an effective method of therapy of SCID patients. Unfortunately, the poor somatic status of children and/or infectious complications are often grounds for the modification of the existing standard approach to HSCT in SCID. In some cases, decreasing the intensity of conditioning regimens seems to be the cause of mixed chimerism, delayed immunological reconstitution, long-term (or life-long) demand for IVIG transfusions, and an increase in morbidity and mortality after HSCT. Currently there are many publications about gene therapy. It's a new and promising approach to the treatment of SCID patients. Perhaps it will be a good alternative for the standard HSCT method in the future.

Between 1997 and 2009, twelve patients (1 female and 11 males) with a median age of 6 months (range 2–12 months) received standard HSCT for SCID at the Federal Research and Clinical Centre of Pediatric Hematology, Oncology and Immunology. In 7 patients we found genetic mutations, and confirmed X1-SCID (n=5), ADA-deficient-SCID (n=1), and Omenn's syndrome (n=1). In other cases we did not discover any genetic mutations, but patients showed clinical signs and immunological phenotypes that match SCID. One patient was transplanted from an HLA-compatible sibling donor, other cases were treated with haploidentical HSCT from their parents. At present, the median follow-up is 76 months (range 3–164 months); 7 children are alive and have full donor chimerism, and five of them demonstrate full immunological reconstitution.

Currently, gene therapy is not available for most patients, but HSCT is an effective method of treatment for SCID patients.

Keywords: SCID, transplantation, conditioning, gene therapy, children