Hematopoietic cell transplantation in inborn errors of immunity Part I: severe combined immunodeficiency

Rafaella Muratori[1*], Juliana Folloni Fernandes[2,3], Gisele Loth[1,4], Ana Karine Vieira[5], Paulo Klinger[6], Simone Franco[7], Carmem Bonfim[4,8]

1. Universidade Federal do Paraná – Hospital de Clínicas – Curitiba (PR), Brazil.
2. Universidade de São Paulo – Hospital das Clínicas, Instituto de Tratamento do Câncer Infantil – São Paulo (SP), Brazil.
3. Hospital Israelita Albert Einstein – São Paulo (SP), Brazil.
4. Hospital Pequeno Príncipe – Curitiba (PR), Brazil.
5. Universidade Federal de Minas Gerais – Hospital das Clínicas – Belo Horizonte (MG), Brazil.
6. Hospital Santa Marcelina – Associação para Criança e Adolescente com Câncer – São Paulo (SP), Brazil.
7. Hospital da Criança – Brasília (DF), Brazil.
8. Duke University – Durham (NC), United States of America.

***Corresponding author:** rafaella.oncopediatra@gmail.com

Section editor: Fernando Barroso Duarte

Received: Sep. 15, 2025 • **Accepted:** Oct. 28, 2025

ABSTRACT

Severe Combined Immunodeficiencies (SCID) are rare diseases characterized by a blockage in T-lymphocyte development. Hematopoietic Cell Transplantation (HCT) is the primary curative therapy, with the highest survival rates correlated to early diagnosis and the absence of active infections at transplant. The gold standard is a matched related sibling donor (MSD), but alternative donors like haploidentical family donors are increasingly used. Conditioning regimens vary, with reduced intensity busulfan and fludarabine often indicated. Early diagnosis via newborn screening and referral to experienced HCT centers are crucial for improving outcomes. Long-term follow-up is essential, as the cumulative incidence of late effects, such as neurologic and neurodevelopmental issues, increases over time.

Keywords: Hematopoietic Stem Cell Transplantation. Severe Combined Immunodeficiency. Primary Immunodeficiency Diseases.

INTRODUCTION

Severe combined immunodeficiencies (SCID) are a group of rare and monogenic diseases characterized by a blockage in the development of T-lymphocytes. Classical SCID is characterized by the absence of T-lymphocytes and deficient T-lymphocyte proliferation. SCID may also have defects in B-cell differentiation and/or function and/or natural killer (NK) cell differentiation.

Current Primary Immunodeficiency Treatment Consortium (PIDTC) guidelines define that patients with typical SCID must have less than 0.05×10^9 autologous T-cells/L on repetitive testing, with either pathogenic variant(s) in a SCID-associated gene, very low/undetectable T-cell receptor excision circles or less than 20% of CD4 T-cells expressing naive markers, and/or transplacental maternally engrafted T-cells. Patients with less profoundly impaired autologous T-cell differentiation are designated as having leaky/atypical SCID[1].

Omenn syndrome is a form of leaky SCID characterized by patients' expanded memory T-cells that infiltrate the skin and other tissues and produce a characteristic generalized erythematous rash, often associated with lymphadenopathy, hepatosplenomegaly, and other clinical features such as eosinophilia. The rash of Omenn syndrome can resemble the rash of graft-*versus*-host disease (GvHD). Therefore, the exclusion of maternal engraftment is essential to confirm the diagnosis.

Genetic diagnosis helps differentiate SCID from other thymic stromal defects and other causes of secondary T-cell deficiency. Non-SCID conditions may have defects in thymic function, like deletions in chromosome 22q11.2 (DiGeorge syndrome), pathogenic variants in genes such as FOXN1 and TBX1, or other underlying causes. These patients may resemble SCID regarding lymphocyte phenotype and clinical phenotype to some extent, but hematopoietic cell transplantation (HCT) is unlikely to be curative, since the error is in the thymic stromal cell development[2].

PRE-TRANSPLANT MANAGEMENT

Patients must be referred to a specialized center as soon as the diagnosis is made. Immunoglobulin (IVIg) replacement therapy and pneumocystis pneumonia prophylaxis must be started promptly, and active infections must be aggressively treated. All blood products should be irradiated and leukodepleted before transfusion to avoid GvHD and cytomegalovirus (CMV) infection. Breastfeeding from a CMV-positive mother should be discouraged. Infant CMV DNA polymerase chain reaction (PCR) studies should be conducted since the diagnosis is made, weekly for four weeks; if all are negative, testing is recommend every three weeks. If any positive CMV PCR, provide treatment with ganciclovir and/or other effective anti-CMV therapy[3].

Patients with SCID are profoundly susceptible to opportunistic infections, and live vaccines are contraindicated. The Bacille Calmette-Guérin (BCG) vaccine in these patients can promote disseminated infection by the vaccine strain and is associated with numerous complications, with increased rates of morbidity and mortality. If the patient has received BCG before diagnosis, prophylaxis with one or two drugs (isoniazid alone or combined with rifampin) is recommended. For patients presenting with local or disseminated BCGosis, four or more drugs may be necessary for the treatment. The use of pyrazinamide is not indicated as BCG is intrinsically resistant to this drug. Antituberculous drugs are potentially hepatoxic, and special care is necessary with multidrug interactions in immature livers. Patients that have received BCG might experience worsening of the disease during aplasia/engraftment and immune reconstitution inflammatory syndrome like symptoms as T-lymphocyte function is restored[4]. Table 1 summarizes the main pre-transplant management.

Table 1. Pre-transplant management.

Immediate referral to a specialized center	Human leukocyte antigens (HLA) typing (patient + parents and siblings)
Isolation precautions (hospital or home)	Immunoglobulin replacement therapy
Pneumocystis pneumonia and fungal prophylaxis Cytomegalovirus polymerase chain reaction screening	Aggressive infection treatment
Irradiated and leukodepleted blood products	Suspension of breastfeeding from a cytomegalovirus-positive mother
All live vaccines are contraindicated, including Bacille Calmette-Guérin (BCG) and rotavirus. If the patient is already vaccinated with BCG, prophylaxis is advised.	If BCG disease is present (focal or disseminated), four-drug treatment should be initiated.

Source: Elaborated by the authors.

TRANSPLANT AND DONOR CHOICE

Patients with SCID present with life-threatening infections (viral, fungal, bacterial) in the first year of life. HCT for these patients is considered a pediatric emergency and must be performed as soon as possible with the most rapid and best available donor since survival is highly correlated to the early diagnosis and the presence of infections at the time of the transplant[5]. A matched related sibling donor (MSD) (unaffected by the same disease) is the gold standard. Without an MSD, alternative donors (matched unrelated or umbilical cord blood)

may be considered if they are easily accessible[6]. Haploidentical family donors have been used since the late 1980s and are a growing alternative, since most donors are readily available.

A recent study compared inborn errors of immunity patients undergoing haploidentical transplants with either *in-vitro* depletion of CD3+TCRαβ/CD19 (TCRαβdep) cells or *in-vivo* T-cell depletion using post-transplant cyclophosphamide (PTCY). The three-year overall survival was 78% for TCRαβ and 66% after PTCY (p = 0.013). The PTCY approach had higher rates of veno-occlusive disease (VOD), while primary graft failure and second HCT were more frequent in TCRαβdep. The high incidence of graft failure among TCRαβdep recipients, needing a second transplant or other intervention, led to a comparable event-free survival between the two groups[7].

An in-depth analysis of 37 Brazilian patients who underwent a haploidentical transplant with PTCY showed an alarming number of SCID patients with severe and very severe VOD (27%), as well as BCG reactivation (34%), and elevated number of patients needed intensive care unit (35%), mechanical ventilation (27%), and dialysis (19%). Due to the extremely high morbidity of these children throughout the HCT, they must be referred to experienced referral centers. The 74% overall survival must be further improved in our country. Defibrotide should be used aggressively and promptly to treat VOD in these patients to reduce VOD-related mortality[8,9].

CONDITIONING

Conditioning regimens may vary according to SCID phenotype (presence of B and/or NK cells), genetic defect (if available), and patients' performance status. Although the most important outcome is developing a functional T-cell compartment, some degree of myeloid chimerism may help B-cell reconstitution and long-term thymic output. In very particular cases, HCT can be performed without conditioning (T-B+NK- SCID, with matched sibling donor). Some patients may not develop B-cell function, requiring lifelong IVIg replacement therapy.

Most indicated regimens (European Society for Immunodeficiencies/Inborn Errors Working Party guidelines) include a reduced dose of busulfan (pharmacokinetics is recommended)—AUC 60–70 mg*h/L—, associated with fludarabine ± serotherapy (thymoglobulin or alemtuzumab) according to the donor type (Table 2). Without myeloablative conditioning, the marrow and thymic niches occupied by host progenitor cells will not be effectively depleted, thus preventing optimal repopulation by donor equivalents. In those cases, the thymic function is often insufficiently corrected[10]. Genetic defects associated to B+ phenotype (IL2RGC, JAK3, IL7R), myeloablative conditioning and matched donors are associated with better outcomes[11]. HCTs without a conditioning therapy may promote an impaired immune function with the risk of chronic viral infections with hepatitis, warts, poor quality of life, and the need of a second transplant[12,13].

Table 2. Hematopoietic cell transplantation conditioning and graft-*versus*-host disease (GvHD) prophylaxis regimens for pediatric patients: Brazilian Society of Cellular Therapy and Bone Marrow Transplantation Consensus recommendations for inborn errors of immunity.

Donor type	Conditioning	GvHD prophylaxis
Myeloablative		
MSD	Busulfan (AUC 85–95) + fludarabine 160 mg/m^2 + ATG 5-10 mg/kg or alemtuzumab 0.5–1 mg/kg	CSA + MTX or MMF
MUD		CSA + MTX or MMF
Haplo		Cyclophosphamide 50 mg/kg +3, +4 + CSA + MMF
CB		CSA + MMF or Methylprednisolone
Reduced toxicity		
MSD	Busulfan (AUC 60–70) + fludarabine 160–180 mg/m^2 + ATG 5–10 mg/kg or alemtuzumab 0.5–1 mg/kg	CSA + MTX or MMF
MUD		CSA + MTX or MMF
Haplo		Cyclophosphamide 50 mg/kg +3, +4 + CSA + MMF
MSD	Fludarabine 150–160 mg/m^2 + melphalan 140 mg/m^2 ± ATG or alemtuzumab 0.5–1 mg/kg	CSA + MTX or MMF
MUD		CSA + MTX or MMF
Haplo		Cyclophosphamide 50 mg/kg +3, +4 + CSA + MMF

MSD: matched related sibling donor; MUD: Matched Unrelated Donor; Haplo: Haploidentical Donor; CB: Cord Blood; ATG: anti-thymocyte globulin; CSA: Cyclosporin; MTX: Metothexate; MMF: Mycophenolate mofetil. Source: adapted from the IEWP recommendations[10].

NEWBORN SCREENING

Babies with SCID diagnosed by newborn screening (NBS) and referred to HCT in the first weeks of life have been studied in Brazil. Conditioning regimens for HCT are not recommended before 6 to 8 weeks old due to concerns with chemotherapy-induced toxicities, as well as immature metabolism of other drugs. The NBS in the city of São Paulo demonstrated an incidence of SCID in our population of 1:39,500 live births, much higher than expected based on average numbers worldwide. All patients with confirmed SCID were referred for HCT, and despite rapid referral, five out of seven patients developed respiratory viral infections before HCT. All patients had HCT with haplo-PTCY, with deaths due to adenovirus infection and severe VOD, one each. HCT were performed at a median age of 113 days old, with an overall survival of 71%[14]. NBS centers should strongly reinforce the need to prevent respiratory viral infections.

GENE THERAPY

Gene therapy has the potential to replace HCT, but it is commercially available in Europe only for adenosine deaminase deficiency SCID (Strim-velis). Clinical studies for other genetic defects are ongoing[15].

LONG-TERM FOLLOW-UP

After more than 50 years since the first transplant for SCID, the long-term complications of transplanting very young children can be addressed. Updated data from the PIDTC of 399 SCID patients revealed a cumulative incidence of chronic and late effects of 25% in those alive in two years, increasing to 41% in 15 years after HCT, the most prevalent being the neurologic, neurodevelopmental, and dental issues. Much of the long-term sequelae is usually attributed to chemoradiation at conditioning, but there are also many challenging complications of unconditioned HCT in SCID patients, such as immunoglobulin replacement, attention-deficit/hyperactivity disorder, warts, and learning disabilities, that were reportedly more prevalent than in the general population[16,17]. Transplant centers should maintain a long-term follow-up clinic to prevent and treat such complications.

KEY-POINTS

- SCIDs differ in phenotype and genotype, and centers should aim for genetic diagnosis. Special attention should be paid to non-typical (leaky) SCIDs;
- A myeloid/b-cell engraftment is preferred due to increasing data demonstrating long-term complications when only the T-cell compartment is corrected;
- Although mieloablative conditioning (MAC) regimens show better overall survival, the haplo-PTCY approach leads to increased toxicity and a high incidence of VOD. Patients should be referred to experienced HCT centers if possible;
- NBS with early diagnosis and referral of babies with SCID to specialized care might improve outcomes.

CONFLICT OF INTEREST

Nothing to declare.

DATA AVAILABILITY STATEMENT

Data sharing is not applicable.

AUTHORS' CONTRIBUTIONS

Substantive scientific and intellectual contributions to the study: Loth G, Fernandes JF, Vieira AK, Klinger P, Franco S and Bonfim C. **Conception and design:** Bonfim C and Fernandes JF. **Manuscript writing:** Fernandes JF and Muratori R. **Final approval:** Muratori R, Fernandes JF, Loth G, Vieira AK, Klinger P, Franco S and Bonfim C.

FUNDING

Not applicable.

ACKNOWLEDGEMENTS

Not applicable.

REFERENCES

1. Dvorak CC, Haddad E, Heimall J, Dunn E, Buckley RH, Kohn DB, Cowan MJ, Pai SY, Griffith LM, Cuvelier GDE, Eissa H, Shah AJ, O'Reilly RJ, Pulsipher MA, Wright NAM, Abraham RS, Satter LF, Notarangelo LD, Puck JM. The diagnosis of severe combined immunodeficiency (SCID): The Primary Immune Deficiency Treatment Consortium (PIDTC) 2022 Definitions. J Allergy Clin Immunol. 2023;151(2):539–46. https://doi.org/10.1016/j.jaci.2022.10.022

2. Collins C, Sharpe E, Silber A, Kulke S, Hsieh EWY. Congenital athymia: genetic etiologies, clinical manifestations, diagnosis, and treatment. J Clin Immunol. 2021;41(5):881–95. https://doi.org/10.1007/s10875-021-01059-7

3. Dorsey MJ, Wright NAM, Chaimowitz NS, Dávila Saldaña BJ, Miller H, Keller MD, Thakar MS, Shah AJ, Abu-Arja R, Andolina J, Aquino V, Barnum JL, Bednarski JJ, Bhatia M, Bonilla FA, Butte MJ, Bunin NJ, Chandra S, Chaudhury S, Chen K, Chong H, Cuvelier GDE, Dalal J, DeFelice ML, DeSantes KB, Forbes LR, Gillio A, Goldman F, Joshi AY, Kapoor N, Knutsen AP, Kobrynski L, Lieberman JA, Leiding JW, Oshrine B, Patel KP, Prockop S, Quigg TC, Quinones R, Schultz KR, Seroogy C, Shyr D, Siegel S, Smith AR, Torgerson TR, Vander Lugt MT, Yu LC, Cowan MJ, Buckley RH, Dvorak CC, Griffith LM, Haddad E, Kohn DB, Logan B, Notarangelo LD, Pai SY, Puck J, Pulsipher MA, Heimall J. Infections in Infants with SCID: Isolation, Infection Screening, and Prophylaxis in PIDTC Centers. J Clin Immunol. 2021;41(1):38–50. https://doi.org/10.1007/s10875-020-00865-9

4. Mazzucchelli JT, Bonfim C, Castro GG, Condino-Neto AA, Costa NM, Cunha L, Dantas EO, Dantas VM, de Moraes-Pinto MI, Fernandes JF, Goes HC, Goudouris E, Grumach AS, Guirau LM, Kuntze G, Mallozzi MC, Monteiro FP, Moraes LS, Nudelman V, Pinto JA, Rizzo MC, Porto-Neto AC, Roxo-Junior P, Ruiz M, Rullo VE, Seber A, Takano OA, Tavares FS, Toledo E, Vilela MM, Costa-Carvalho BT. Severe combined immunodeficiency in Brazil: management, prognosis, and BCG-associated complications. J Investig Allergol Clin Immunol. 2014;24(3):184–91.

5. Haddad E, Hoenig M. Hematopoietic stem cell transplantation for severe combined immunodeficiency (SCID). Front Pediatr. 2019;7:481. https://doi.org/10.3389/fped.2019.00481

6. Fernandes JF, Rocha V, Labopin M, Neven B, Moshous D, Gennery AR, Friedrich W, Porta F, Diaz de Heredia C, Wall D, Bertrand Y, Veys P, Slatter M, Schulz A, Chan KW, Grimley M, Ayas M, Gungor T, Ebell W, Bonfim C, Kalwak K, Taupin P, Blanche S, Gaspar HB, Landais P, Fischer A, Gluckman E, Cavazzana-Calvo M; Eurocord and Inborn Errors Working Party of European Group for Blood and Marrow Transplantation. Transplantation in patients with SCID: mismatched related stem cells or unrelated cord blood? Blood. 2012;119(12):2949–55. https://doi.org/10.1182/blood-2011-06-363572

7. Lum SH, Albert MH, Gilbert P, Sirait T, Algeri M, Muratori R, Fournier B, Laberko A, Karakukcu M, Unal E, Ayas M, Yadav SP, Fisgin T, Elfeky R, Fernandes J, Faraci M, Cole T, Schulz A, Meisel R, Zecca M, Ifversen M, Biffi A, Diana JS, Vallée T, Giardino S, Ersoy GZ, Moshous D, Gennery AR, Balashov D, Bonfim C, Locatelli F, Lankester A, Neven B, Slatter M. Outcomes of HLA-mismatched HCT with TCRαβ/CD19 depletion or post-HCT cyclophosphamide for inborn errors of immunity. Blood. 2024;144(5):565–80. https://doi.org/10.1182/blood.2024024038

8. The 48th Annual Meeting of the European Society for Blood and Marrow Transplantation: Physicians - Oral Sessions (O009-O155). Bone Marrow Transplant. 2022;57(Suppl. 1):16–99. https://doi.org/10.1038/s41409-022-01815-2

9. Richardson PG, Riches ML, Kernan NA, Brochstein JA, Mineishi S, Termuhlen AM, Arai S, Grupp SA, Guinan EC, Martin PL, Steinbach G, Krishnan A, Nemecek ER, Giralt S, Rodriguez T, Duerst R, Doyle J, Antin JH, Smith A, Lehmann L, Champlin R, Gillio A, Bajwa R, D'Agostino RB Sr, Massaro J, Warren D, Miloslavsky M, Hume RL, Iacobelli M, Nejadnik B, Hannah AL, Soiffer RJ. Phase 3 trial of defibrotide for the treatment of severe veno-occlusive disease and multi-organ failure. Blood. 2016;127(13):1656–65. https://doi.org/10.1182/blood-2015-10-676924

10. Lankester AC, Albert MH, Booth C, Gennery AR, Güngör T, Hönig M, Morris EC, Moshous D, Neven B, Schulz A, Slatter M, Veys P; Inborn Errors Working Party of the European Society for Blood and Marrow Transplantation and the European Society for Immune Deficiencies, and European Reference Network on Rare Primary Immunodeficiency Autoinflammatory Autoimmune diseases (RITA). EBMT/ESID inborn errors working party guidelines for hematopoietic stem cell transplantation for inborn errors of immunity. Bone Marrow Transplant. 2021;56(9):2052–62. https://doi.org/10.1038/s41409-021-01378-8

11. Lankester AC, Neven B, Mahlaoui N, von Asmuth EGJ, Courteille V, Alligon M, Albert MH, Serra IB, Bader P, Balashov D, Beier R, Bertrand Y, Blanche S, Bordon V, Bredius RG, Cant A, Cavazzana M, Diaz-de-Heredia C, Dogu F, Ehlert K, Entz-Werle N, Fasth A, Ferrua F, Ferster A, Formankova R, Friedrich W, Gonzalez-Vicent M, Gozdzik J, Güngör T, Hoenig M, Ikinciogullari A, Kalwak K, Kansoy S, Kupesiz A, Lanfranchi A, Lindemans CA, Meisel R, Michel G, Miranda NAA, Moraleda J, Moshous D, Pichler H, Rao K, Sedlacek P, Slatter M, Soncini E, Speckmann C, Sundin M, Toren A, Vettenranta K, Worth A, Yeşilipek MA, Zecca M, Porta F, Schulz A, Veys P, Fischer A, Gennery AR. Hematopoietic cell transplantation in severe combined immunodeficiency: The SCETIDE 2006-2014 European cohort. J Allergy Clin Immunol. 2022;149(5):1744–54.e8. https://doi.org/10.1016/j.jaci.2021.10.017

12. Petit A, Neven B, Min V, Mahlaoui N, Moshous D, Castelle M, Allouche M, Stérin A, Visentin S, Saultier P, Boucekine M, Shawket AM, Picard C, Auquier P, Michel G, Fischer A, Barlogis V. Impact of graft function on health status and quality of life in very long-term survivors who received an HSCT for inborn errors of immunity, a prospective study of the CEREDIH. Transplant Cell Ther. 2023;29(9):582.e1–e6. https://doi.org/10.1016/j.jtct.2023.06.006

13. Riller Q, Fourgeaud J, Bruneau J, De Ravin SS, Smith G, Fusaro M, Meriem S, Magerus A, Luka M, Abdessalem G, Lhermitte L, Jamet A, Six E, Magnani A, Castelle M, Lévy R, Lecuit MM, Fournier B, Winter S, Semeraro M, Pinto G, Abid H, Mahlaoui N, Cheikh N, Florkin B, Frange P, Jeziorski E, Suarez F, Sarrot-Reynauld F, Nouar D, Debray D, Lacaille F, Picard C, Pérot P, Regnault B, Da Rocha N, de Cevins C, Delage L, Pérot BP, Vinit A, Carbone F, Brunaud C, Marchais M, Stolzenberg MC, Asnafi V, Molina T, Rieux-Laucat F, Notarangelo LD, Pittaluga S, Jais JP, Moshous D, Blanche S, Malech H, Eloit M, Cavazzana M, Fischer A, Ménager MM, Neven B. Late-onset enteric virus infection associated with hepatitis (EVAH) in transplanted SCID patients. J Allergy Clin Immunol. 2023;151(6):1634–45. https://doi.org/10.1016/j.jaci.2022.12.822

14. Aranda CS, Pimentel MG, Guimaraes RR, Fernandes JF, Rizzo MCFV, Ishizuka E, de Oliveira Junior EB, Hadachi SM, Hayashi GY, de Andrade CEF, de Marco Mauro AM, Sole D, Condino-Neto A. Newborn screening for inborn errors of immunity in Brazil. Pediatr Allergy Immunol. 2025;36(2):e70047. https://doi.org/10.1111/pai.70047

15. Kohn DB, Hershfield MS, Puck JM, Aiuti A, Blincoe A, Gaspar HB, Notarangelo LD, Grunebaum E. Consensus approach for the management of severe combined immune deficiency caused by adenosine deaminase deficiency. J Allergy Clin Immunol. 2019;143(3):852–63. https://doi.org/10.1016/j.jaci.2018.08.024

16. Eissa H, Thakar MS, Shah AJ, Logan BR, Griffith LM, Dong H, Parrott RE, O'Reilly RJ, Dara J, Kapoor N, Forbes Satter L, Chandra S, Kapadia M, Chandrakasan S, Knutsen A, Jyonouchi SC, Molinari L, Rayes A, Ebens CL, Teira P, Dávila Saldaña BJ, Burroughs LM, Chaudhury S, Chellapandian D, Gillio AP, Goldman F, Malech HL, DeSantes K, Cuvelier GDE, Rozmus J, Quinones R, Yu LC, Broglie L, Aquino V, Shereck E, Moore TB, Vander Lugt MT, Mousallem TI, Oved JH, Dorsey M, Abdel-Azim H, Martinez C, Bleesing JH, Prockop S, Kohn DB, Bednarski JJ, Leiding J, Marsh RA, Torgerson T, Notarangelo LD, Pai SY, Pulsipher MA, Puck JM, Dvorak CC, Haddad E, Buckley RH, Cowan MJ, Heimall J. Posttransplantation late complications increase over time for patients with SCID: A Primary Immune Deficiency Treatment Consortium (PIDTC) landmark study. J Allergy Clin Immunol. 2024;153(1):287–96. https://doi.org/10.1016/j.jaci.2023.09.027

17. Hardin O, Lokhnygina Y, Buckley RH. Long-term clinical outcomes of severe combined immunodeficiency patients given nonablative marrow transplants. J Allergy Clin Immunol Pract. 2022;10(4):1077–83. https://doi.org/10.1016/j.jaip.2021.11.032