Molina Clinical Policy
Hematopoietic Stem Cell Transplantation for Immunodeficiency Disorders: Policy No. 265

Last Approval: 10/12/2022
Next Review Due By: October 2023

DISCLAIMER

This Molina Clinical Policy (MCP) is intended to facilitate the Utilization Management process. Policies are not a supplementation or recommendation for treatment; Providers are solely responsible for the diagnosis, treatment and clinical recommendations for the Member. It expresses Molina's determination as to whether certain services or supplies are medically necessary, experimental, investigational, or cosmetic for purposes of determining appropriateness of payment. The conclusion that a particular service or supply is medically necessary does not constitute a representation or warranty that this service or supply is covered (e.g., will be paid for by Molina) for a particular Member. The Member's benefit plan determines coverage – each benefit plan defines which services are covered, which are excluded, and which are subject to dollar caps or other limits. Members and their Providers will need to consult the Member's benefit plan to determine if there are any exclusion(s) or other benefit limitations applicable to this service or supply. If there is a discrepancy between this policy and a Member's plan of benefits, the benefits plan will govern. In addition, coverage may be mandated by applicable legal requirements of a State, the Federal government or CMS for Medicare and Medicaid Members. CMS's Coverage Database can be found on the CMS website. The coverage directive(s) and criteria from an existing National Coverage Determination (NCD) or Local Coverage Determination (LCD) will supersede the contents of this MCP and provide the directive for all Medicare members. References included were accurate at the time of policy approval and publication.

OVERVIEW

The primary immunodeficiencies are a genetically heterogeneous group of diseases that affect distinct components of the immune system. The most severe defects (collectively known as severe combined immunodeficiency or SCID) represents a group of rare, sometimes fatal, congenital disorders characterized by little or no immune response. The defining feature of SCID, commonly known as "bubble boy" disease, is a defect in the specialized white blood cells (B- and T-lymphocytes) that defend us from infection by viruses, bacteria and fungi. Without a functional immune system, SCID patients are susceptible to recurrent infections such as pneumonia, meningitis and chicken pox, and can die before the first year of life. Though invasive, new treatments such as bone marrow and stem-cell transplantation save as many as 80% of SCID patients. (DynaMed, 2022; National Center for Biotechnology Information, 1998).

Stem-cell transplantation refers to transplantation of hematopoietic stem cells (HSCs) from a donor into a patient. HSCs are immature cells that can develop into any of the three types of blood cells (red cells, white cells or platelets). HSCs are created in the bone marrow and are found in the bone marrow and peripheral blood. There is also a high concentration of HSCs in umbilical-cord blood. Hematopoietic stem-cell transplantation (HSCT) can be either autologous (using the person's own stem cells) or allogeneic (using stem cells from a donor). In allogeneic HSCT, it is preferable for donors to have a human leukocyte antigen (HLA) type that is identical to the recipient. Matching is performed on the basis of variability at three of more loci of the HLA gene (e.g., HLA-A, HLA-B, HLA-DRB1). As HLA variability increases, transplant-related morbidity and mortality, including graft rejection and graft-versus-host disease, also increase. (Heimall, 2022; Holmberg & Sandmaier, 2022; Notarangelo, 2022).

COVERAGE POLICY

All transplants require prior authorization from the Corporate Transplant Department. Solid organ transplant requests will be reviewed by the Corporate Senior Medical Director or qualified clinical designee. All other transplants will be reviewed by the Corporate Senior Medical Director or covering Medical Director. If the criteria are met using appropriate NCD and/or LCD guidelines, State regulations, and/or MCP policies the Corporate Senior Medical Director's designee can approve the requested transplant.

Office visits with participating Providers do NOT require prior authorization. Providers should see the Member in office visits as soon as possible and without delay. Failure to see the Member in office visits may be considered a serious quality of care concern.

Pre-Transplant Evaluation

(AMR, 2019; Bonilla et al., 2015; ECOG, n.d.; [1-5] NMDP, n.d.)

Please see *MCP-323 Pre-Transplant Evaluation* for additional criteria and information.

Criteria for transplant evaluation include:

1. History and physical examination; **AND**

2. Psychosocial evaluation and clearance:
   a. No behavioral health disorder by history or psychosocial issues:
      - If history of behavioral health disorder, no severe psychosis or personality disorder;
      - Mood/anxiety disorder must be excluded or treated;
      - Member has understanding of surgical risk and post procedure compliance and follow-up required.

   AND
   b. Adequate family and social support.

AND

3. EKG; **AND**

4. Chest x-ray; **AND**

5. Cardiac clearance in the presence of any of the following:
   a. Chronic smokers; **OR**
   b. Members > 50 years age; **OR**
   c. Those with a clinical or family history of heart disease or diabetes.

AND

6. Pulmonary clearance if evidence of pulmonary artery hypertension (PAH) or chronic pulmonary disease; **AND**

7. Neurological exam and clearance for transplant including **ONE** of the following:
   a. Normal exam by H&P; **OR**
   b. Abnormal neurological exam with positive findings including **ONE** of the following:
      - Lumbar puncture normal cytology; **OR**
      - Lumbar puncture with cytological exam abnormal: CNS disease treated prior to clearance.

AND

8. A Performance Status that includes **ONE** of the following:
   a. Karnofsky score 70-100%; **OR**
   b. Eastern Cooperative Oncology Group (ECOG) Grade 0-2.

AND

9. Lab studies that include:
   a. Complete blood count; kidney profile (blood urea nitrogen, creatinine); electrolytes; calcium; phosphorous; albumin; liver function tests; and coagulation profile (prothrombin time, and partial thromboplastin time);*
   b. Serologic screening for: HIV; Epstein Barr virus (EBV); Hepatitis virus B (HBV); Hepatitis C (HCV); cytomegalovirus (CMV); RPR and/or FTA:*
      - If HIV positive **ALL** of the following must be met:
        i. CD4 count >200 cells/mm-3 for >6 months; **AND**
        ii. HIV-1 RNA undetectable; **AND**
        iii. On stable anti-retroviral therapy >3 months; **AND**
        iv. No other complications from AIDS (e.g., opportunistic infection, including aspergillus, tuberculosis, coccidioides mycosis, resistant fungal infections, Kaposi's sarcoma, or other neoplasm).

- If abnormal serology, need physician plan to address and/or treatment as indicated.
  i. Antinuclear antibody, smooth muscle antibody, antimitochondrial antibody
  ii. Ceruloplasmin, α1-antitrypsin phenotype
  iii. Alpha-fetoprotein

c. Urine drug screen (UDS) if Member is current or gives a history of past drug abuse.

AND

10. Colonoscopy (if indicated or if Member is age ≥ 50) with complete workup and treatment of abnormal results as indicated; an initial screening colonoscopy after initial negative screening requires a follow-up colonoscopy every 10 years).*

AND

11. Gynecological examination with Pap smear for women ages ≥ 21 to ≤ 65 years of age or if indicated (not indicated in women who have had a total abdominal hysterectomy [TAH] or a total vaginal hysterectomy [TVH]) within the last three years with complete workup and treatment of abnormal results as indicated.

Within the last 12 months:
1. Dental examination or oral exam showing good dentition and oral care or no abnormality on panorex or plan for treatment of problems pre- or post-transplant; **AND**
2. Mammogram (if indicated or > age 40) with complete workup and treatment of abnormal results as indicated;* **AND**
3. PSA if history of prostate cancer or previously elevated PSA with complete workup and treatment of abnormal results as indicated.*

* Participating Centers of Excellence may waive these criteria.

Criteria for Hematopoietic Allogenic Stem Cell Transplantation (HSCT)

(Notarangelo, 2022; AMR, 2019; Bonilla et al., 2015; [1-5] NMDP, n.d.)

Hematopoietic Allogeneic Stem Cell Transplantation (HSCT) ablative or non-myeloablative from a human leukocyte antigen (HLA)-matched donor (e.g., at least six out of eight match of the HLA-A, HLA-B, HLA-C and HLA-DRB1 markers) or from cord blood when there are no matched sibling or unrelated donors (e.g., at least four out of six match of the HLA-A, HLA-B and HLA-DRB-1 markers) **is considered medically necessary** and may be authorized for the treatment of immunodeficiency disorders when **ALL** of the following criteria are met:

1. All pre-transplant criteria are met; **AND**

2. Diagnosis of **ONE** of the following immunodeficiency disorders (including, but not limited to):

   a. Absent T-cell function as evidenced by a diagnosis of **ONE** of the following:
   - Hemophagocytic Lymphohistiocytosis (HLH); **OR**
   - Severe Combined Immunodeficiency (SCID); **OR**
   - Wiskott-Aldrich Syndrome (WAS); **OR**
   - X-linked lymphoproliferative syndrome.

   OR

   b. Absent or defective natural killer function as evidenced by a diagnosis of Chediak-Higashi syndrome; **OR**

   c. Absent or defective neutrophil function as evidenced by a diagnosis of **ONE** of the following:
   - Primary granulocyte dysfunction; **OR**
   - Chronic granulomatous disease; **OR**
   - Omenn Syndrome; **OR**
   - Leukocyte adhesion deficiency; **OR**
   - DiGeorge Syndrome; **OR**
   - Kostmann Syndrome.

AND

3. The requesting transplant recipient should not have any of the following absolute contraindications:
   a. Cardiac, pulmonary, and nervous system disease that cannot be corrected and is a prohibitive risk for surgery; **OR**
   b. Malignant neoplasm with a high risk for reoccurrence, non-curable malignancy (excluding localized skin cancer); **OR**
   c. Systemic and/or uncontrolled infection; **OR**
   d. AIDS (CD4 count < 200cells/mm3); **OR**
   e. Unwilling or unable to follow post-transplant regimen:
      - Documented history of non-compliance
      - Inability to follow through with medication adherence or office follow-up

   OR
   f. Chronic illness with one year or less life expectancy; **OR**
   g. Limited, irreversible rehabilitation potential; **OR**
   h. Active untreated substance abuse issues, requires documentation supporting free from addiction for minimally 6 months if previous addiction was present; **OR**
   i. No adequate social/family support.

AND

4. The requesting transplant recipient should be evaluated carefully and potentially treated if any of the relative contraindications below are present. (Irreversible lung disease patients require consultation and clearance by a Pulmonologist prior to consideration of transplantation).
   a. Smoking, documentation supporting free from smoking for 6 months; **OR**
   b. Active peptic ulcer disease; **OR**
   c. Active gastroesophageal reflux disease; **OR**
   d. CVA with long term impairment that is not amendable to rehabilitation or a patient with CVA/transient ischemic attack within past 6 months; **OR**
   e. Obesity with body mass index of >30 kg/m^2 may increase surgical risk; **OR**
   f. Chronic liver disease such as Hepatitis B/C/D, or cirrhosis which increases the risk of death from sepsis and hepatic failure requires consultation by a gastroenterologist or hepatologist; **OR**
   g. Gall bladder disease requires ultrasound of the gall bladder with treatment prior to transplantation.

Criteria for Subsequent Hematopoietic Allogenic Stem Cell Transplantation (HSCT)

Hematopoietic Allogeneic Stem Cell Transplantation (HSCT) (ablative or non-myeloablative) **may be authorized after the first prior stem cell transplantation has occurred** only one time for members with immunodeficiency disorders who meet all of the above criteria for transplant and have **ANY** of the following:

1. Primary graft failure indicated by no signs of engraftment* by 42 days after the transplant; **OR**
2. Failure to engraft*; **AND**
3. A suitable allogeneic donor has been identified if applicable

*NOTE: Engraftment is defined as the first 3 consecutive days on which the absolute neutrophil count (ANC) exceeds 5 x 10^9/L or > ANC500 at any time after transplantation.

For Members with Significant or Daily Marijuana Use

1. Documentation of compliance with a physician prescribed and managed program of abstinence, and a reasonable expectation that the Member will be abstinent from marijuana use during the transplant and immediate post-transplant time period. Daily marijuana use is an absolute contraindication for both transplant and pre-transplant evaluation unless there is a state mandate applicable for medical marijuana use and transplants, and there is documentation of Member compliance with a physician prescribed plan of care for prescribed marijuana use.

2. If the Member's marijuana use is in compliance with a formal, State-based program for managed medical marijuana, the request should include:
   - Documentation of the Plan of Care for medical marijuana (including the medical decision making that supports the use of medical marijuana); **AND**
   - Transplant Provider agreement with the Plan of Care (including agreement to be accountable for managing the Member's use of medical marijuana).

Continuation of Therapy

When extension of a previously approved transplant authorization is requested, review using updated clinical information is appropriate.

1. If Molina Healthcare has authorized prior requests for transplantation **ALL** of the following information is required for medical review:
   a. Presence of no absolute contraindication as listed above; **AND**
   b. History and physical within the last 12 months; **AND**
   c. Kidney profile within the last 12 months; **AND**
   d. Cardiac update if history of cardiac disease within two years (≥ 50 years of age); **AND**
   e. Psychosocial evaluation or update within the last 12 months; **AND**
   f. Per initial and updated history and physical, any other clinically indicated tests and/or scans as determined by transplant center physician or Molina Medical Director.

2. If authorized prior requests for transplantation were obtained from another insurer, **ALL** of the following information is required for medical review:
   a. Authorization letter/documentation from previous insurer; **AND**
   b. Presence of no absolute contraindication as listed above; **AND**
   c. History and physical within the last 12 months; **AND**
   d. Cardiac update if history of cardiac disease within two years (≥ 50 years of age); **AND**
   e. Psychosocial evaluation or update within the last 12 months; **AND**
   f. Per initial and updated history and physical, any other clinically indicated tests and/or scans as determined by transplant center physician or Molina Medical Director.

Limitations and Exclusions

1. Allogeneic (ablative or non-myeloablative) stem cell transplantation when the above criteria are not met.
2. A second or repeat allogeneic (ablative or non-myeloablative) transplant due to persistent, progressive or relapsed disease.
3. Autologous stem cell transplantation.
4. A planned tandem allogeneic hematopoietic stem cell transplantation.
5. Hematopoietic stem cell collection, storage and freezing for a future unplanned transplant.

DOCUMENTATION REQUIREMENTS. Molina Healthcare reserves the right to require that additional documentation be made available as part of its coverage determination; quality improvement; and fraud; waste and abuse prevention processes. Documentation required may include, but is not limited to, patient records, test results and credentials of the provider ordering or performing a drug or service. Molina Healthcare may deny reimbursement or take additional appropriate action if the documentation provided does not support the initial determination that the drugs or services were medically necessary, not investigational or experimental, and otherwise within the scope of benefits afforded to the member, and/or the documentation demonstrates a pattern of billing or other practice that is inappropriate or excessive.

SUMMARY OF MEDICAL EVIDENCE

The published medical evidence and outcomes for hematopoietic stem cell transplantation for immunodeficiency disorders in the United States consists of registry data obtained from transplant centers that perform adult and pediatric transplantation and is available from the United Network for Organ Sharing (UNOS) database. Registry data demonstrates graft survival rates and outcomes for stem cell transplantation based on demographic and clinical information. ([1] NMDP, n.d.). There is a large amount of published literature regarding transplant outcomes in immunodeficiency disorders. Two studies showing graft survival rates and outcomes for stem cell transplantation are outlined below:

A retrospective analysis by Rousso et al. (2015) was conducted of HSCT in children with PID in a tertiary medical center over the period of 1983 to 2012. Participants included 93 children with PID with a median follow-up of 3.6 years (range, 29 d to 21.2 y) after HSCT. The 2-year survival rates after HSCT for children with severe combined immune deficiency, hemophagocytic lymphohistiocytosis/lymphoproliferative disease, Wiskott-Aldrich syndrome, granulocyte defect, and undefined PID were 65.7%±6.8%, 80%±10.3%, 83.3%±15.2%, 75%±12.5%, and 25%±21.7%, respectively. Survival was associated with year of HSCT and matching. The hazard ratio (HR) (95% CI) for HSCT done in 1983 to 1999 compared with 2000 to 2012 and for matched (related and unrelated) compared with mismatched donor were 2.14 (0.99 to 4.653) and 3.07 (1.46 to 6.4), respectively. Survival was not associated with age, sex of the recipient, underlying PID, conditioning regimen, and presence of acute graft-versus-host disease. After adjustment to the underlying PID, donor and use of fludarabine-based conditioning, the HR (95% CI) for HSCT from the year 2000 was 4.69 (range, 1.4 to 15.45). Advances in HSCT over time have improved the survival of children with PID.

Gungor et al. (2014) performed a prospective study in 16 centers in 10 countries worldwide enrolled patients aged 0 to 40 years with CGD treated with RIC HSCT consisting of high-dose Flu, serotherapy or low-dose alemtuzumab, and low-dose (50% to 72% of myeloablative dose) or targeted busulfan administration. Unmanipulated bone marrow or peripheral blood stem cells from HLA-matched related-donors or HLA-9/10 or HLA-10/10 matched unrelated-donors were infused. The primary end points were OS and EFS, probabilities of OS and EFS at 2 years, incidence of acute and chronic GVHD, achievement of at least 90% myeloid donor chimerism, and incidence of graft failure after at least 6 months of follow-up. A total 56 patients (median age 12.7 years) with chronic granulomatous disease were enrolled; 42 patients (75%) had high-risk features (i.e., intractable infections and autoinflammation), 25 (45%) were adolescents and young adults (age 14-39 years). Median time to engraftment was 19 days for neutrophils and 21 days for platelets. At median follow-up of 21 months, OS was 93% (52/56) and EFS was 89% (50/56). The 2-year probability of OS was 96% (95% confidence interval [CI], 86.46 to 99.09) and of EFS was 91% (79.78 to 96.17). Graft-failure occurred in 5% (3/56) of patients. The cumulative incidence of acute GVHD of grade III to IV was 4% (2/56) and of chronic GVHD was 7% (4/56). Stable (>/=90%) myeloid donor chimerism was documented in 52 (93%) surviving patients.

National and Specialty Organizations

The **National Marrow Donor Program (NMDP)** recommends HCT at time of diagnosis or if detected on newborn screening for immunodeficiency disorders. The NMDP has published guidance on: *Disease-Specific HCT Indications and Outcomes Data; Engraftment; HLA Matching; Patient Eligibility for HCT; Transplant Consultation Timing; and Treatment Before Transplant.* ([1-6] NMDP, n.d.).

The **American Academy of Allergy, Asthma & Immunology; the American College of Allergy, Asthma & Immunology; and the Joint Council of Allergy, Asthma & Immunology** published the *Practice Parameter on Diagnosis and Management of Primary Immunodeficiency.* The purpose of the practice parameter is to provide consultant allergist/immunologist or other practitioner with a practical guide for the clinical recognition and diagnosis of immunodeficiency, along with the general principles that guide management of these disorders. In addition, the practice parameter organizes current knowledge and practice in the diagnosis and management of primary immunodeficiency diseases (PIDDs). (Bonilla et al., 2015).

SUPPLEMENTAL INFORMATION

None.

CODING & BILLING INFORMATION

CPT Codes

CPT	Description
	Collection Codes
38205	Blood-derived hematopoietic progenitor cell harvesting for transplantation, per collection; allogeneic
38206	Blood-derived hematopoietic progenitor cell harvesting for transplantation, per collection; autologous
38230	Bone marrow harvesting for transplantation; allogeneic
38232	Bone marrow harvesting for transplantation; autologous

	Cell Processing Services
38207	Transplant preparation of hematopoietic progenitor cells; cryopreservation and storage
38208	Transplant preparation of hematopoietic progenitor cells; thawing of previously frozen harvest, without washing, per donor
38209	Transplant preparation of hematopoietic progenitor cells; thawing of previously frozen harvest, with washing, per donor
38210	Transplant preparation of hematopoietic progenitor cells; specific cell depletion within harvest, T-cell depletion
38211	Transplant preparation of hematopoietic progenitor cells; tumor cell depletion
38212	Transplant preparation of hematopoietic progenitor cells; red blood cell removal
38213	Transplant preparation of hematopoietic progenitor cells; platelet depletion
38214	Transplant preparation of hematopoietic progenitor cells; plasma (volume) depletion
38215	Transplant preparation of hematopoietic progenitor cells; cell concentration in plasma, mononuclear, or buffy coat layer
	Cell infusion codes
38240	Hematopoietic progenitor cell (HPC); allogeneic transplantation per donor
38241	Hematopoietic progenitor cell (HPC); autologous transplantation
38242	Allogeneic lymphocyte infusions
38243	Hematopoietic progenitor cell (HPC); HPC boost

HCPCS Codes

HCPCS	Description
S2140	Cord blood harvesting for transplantation, allogeneic
S2142	Cord blood derived stem-cell transplantation, allogeneic
S2150	Bone marrow or blood-derived stem cells (peripheral or umbilical), allogeneic or autologous, harvesting, transplantation, and related complications; including pheresis and cell preparation/storage; marrow ablative therapy; drugs; supplies; hospitalization with outpatient follow-up; medical/surgical, diagnostic, emergency, and rehabilitative services; and the number of days of pre-and post-transplant care in the global definition

CODING DISCLAIMER. Codes listed in this policy are for reference purposes only and may not be all-inclusive. Deleted codes and codes which are not effective at the time the service is rendered may not be eligible for reimbursement. Listing of a service or device code in this policy does not guarantee coverage. Coverage is determined by the benefit document. Molina adheres to Current Procedural Terminology (CPT®), a registered trademark of the American Medical Association (AMA). All CPT codes and descriptions are copyrighted by the AMA; this information is included for informational purposes only. Providers and facilities are expected to utilize industry standard coding practices for all submissions. When improper billing and coding is not followed, Molina has the right to reject/deny the claim and recover claim payment(s). Due to changing industry practices, Molina reserves the right to revise this policy as needed.

APPROVAL HISTORY

10/12/2022	Policy reviewed, no changes to criteria, included section on marijuana use.
10/13/2021	Policy reviewed, no update to criteria, updated references.
9/16/2020	Policy reviewed, no updates to criteria, updated references.
9/18/2019	Policy reviewed, no update to clinical criteria, updated references and professional guidelines. In the Diagnosis section, added definitions for: absent T-cell function, absent or defective natural killer function, and absent or defective neutrophil function.
6/22/2017	Policy reviewed, no updates.
3/8/2018	Policy reviewed, no updates.
2/10/2016	New policy.

REFERENCES

Government Agencies

1. Centers for Medicare and Medicaid Services (CMS). Medicare coverage database. National coverage determination (NCD) – Stem cell transplantation 110.23. Available from CMS. Effective Date January 27, 2016. Accessed August 17, 2022.
2. National Center for Biotechnology Information (US). Genes and disease. Bethesda (MD): National Center for Biotechnology Information (US); 1998-. Diseases of the immune system. Available from NIH. Accessed August 17, 2022.

Evidence Based Reviews and Publications

1. AMR Peer Review. Policy reviewed April 17, 2019 by an Advanced Medical Reviews (AMR) practicing, board-certified physician in the areas of Oncology, Hematology.
2. DynaMed. Severe combined immunodeficiency (SCID) – record no. T116174. Available from DynaMed. Updated November 30, 2018. Accessed August 17, 2022. Registration and login required.
3. Holmberg HJ, Sandmaier BM. Determining eligibility for autologous/allogenic hematopoietic cell transplantation. Available from UpToDate. Updated February 21, 2022. Accessed August 17, 2022. Registration and login required.
4. Heimall J. Severe combined immunodeficiency (SCID): An overview. Available from UpToDate. Updated December 12, 2019. Accessed August 17, 2022. Registration and login required.
5. Notarangelo LD. Combined immunodeficiencies. Available from UpToDate. Updated March 6, 2019. Accessed August 17, 2022. Registration and login required.

National and Specialty Organizations

1. Bonilla FA, Khan DA, Ballas ZK, Chinen J, Frank MM, Hsu JT, et al. Practice parameter on diagnosis and management of primary immunodeficiency. J Allergy Clin Immunol. 2015 Nov;136(5):1186-205.e1-78. doi: 10.1016/j.jaci.2015.04.049. Accessed August 17, 2022.
2. Eastern Cooperative Oncology Group (ECOG) Performance Status. Available from ECOG. Accessed August 17, 2022.
3. [1] National Marrow Donor Program (NMDP). Disease-specific HCT indications and outcomes data. Available from NMDP. Accessed August 17, 2022.
4. [2] National Marrow Donor Program (NMDP). Engraftment. Available from NMDP. Accessed August 17, 2022.
5. [3] National Marrow Donor Program (NMDP). HLA matching. Available from NMDP. Accessed August 17, 2022.
6. [4] National Marrow Donor Program (NMDP). Patient eligibility for HCT. Available from NMDP. Accessed August 17, 2022.
7. [5] National Marrow Donor Program (NMDP). Transplant consultation timing. Available from NMDP. Accessed August 17, 2022.
8. [6] National Bone Marrow Donor Program (NMDP). Treatment before transplant. Available from NMDP. Accessed August 17, 2022.

Peer Reviewed Publications

1. Gungor T, Teira P, Slatter M, Stussi G, Stepensky P, Moshous D, et al. Reduced-intensity conditioning and HLA-matched haemopoietic stem-cell transplantation in patients with chronic granulomatous disease: A prospective multicentre study. Lancet. 2014 Feb 1;383(9915):436-48. doi: 10.1016/S0140-6736(13)62069-3. Accessed August 17, 2022.
2. Rousso SZ, Shamriz O, Zilkha A, Braun J, Averbuch D, Or R, et al. Hematopoietic stem cell transplantations for primary immune deficiencies: 3 decades of experience from a tertiary medical center. J Pediatr Hematol Oncol. 2015 Jul;37(5):e295-300. doi: 10.1097/MPH.0000000000000352. Accessed August 17, 2022.

APPENDIX

Reserved for State specific information. *Information includes, but is not limited to, State contract language, Medicaid criteria and other mandated criteria.*