Allogeneic Hematopoietic Cell Transplantation for Genetic Diseases and Acquired Anemia

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Products:	Medicaid
Application:	All participating hospitals and providers
Page Number(s):	1 of 6

Disclaimer

Highmark Health Options medical policy is intended to serve only as a general reference resource regarding coverage for the services described. This policy does not constitute medical advice and is not intended to govern or otherwise influence medical decisions.

POLICY STATEMENT

Highmark Health Options may provide coverage under medical surgical benefits of the Company's Medicaid products for medically necessary allogeneic hematopoietic cell transplantation for genetic diseases and acquired anemia.

This policy is designed to address medical necessity guidelines that are appropriate for the majority of individuals with a particular disease, illness or condition. Each person's unique clinical circumstances warrant individual consideration, based upon review of applicable medical records.

The qualifications of the policy will meet the standards of the National Committee for Quality Assurance (NCQA) and the Delaware Department of Health and Social Services (DHSS) and all applicable state and federal regulations.

DEFINITIONS

Highmark Health Options (HHO) – Managed care organization serving vulnerable populations that have complex needs and qualify for Medicaid. Highmark Health Options members include individuals and families with low income, expecting mothers, children, and people with disabilities. Members pay nothing to very little for their health coverage. Highmark Health Options currently services Delaware Medicaid: Delaware Healthy Children Program (DHCP) and Diamond State Health Plan Plus members.

PROCEDURES

Prior authorization is not required.

Several inherited and acquired conditions have the potential for severe and/or progressive disease. For some conditions, allogeneic hematopoietic cell transplantation (HCT) has been used to alter the natural history of the disease or potentially offer a cure.

Allogeneic HCT involves the intravenous (IV) infusion of allogeneic (donor) stem cells to reestablish hematopoietic function in individuals whose bone marrow or immune system is damaged or defective. They can be harvested from bone marrow, peripheral blood, or umbilical cord blood and placenta shortly after delivery of neonates.

Allogeneic HCT may be considered medically necessary for select individuals with the following disorders:

- Hemoglobinopathies
  - Sickle cell anemia for children or young adults with either a history of prior stroke or at increased risk of stroke or end-organ damage; or
  - Homozygous beta-thalassemia (i.e., thalassemia major); or
- Bone marrow failure syndromes
  - Aplastic anemia including hereditary (including Fanconi anemia, dyskeratosis congenita, Shwachman-Diamond, Diamond-Blackfan); or
  - Acquired (e.g., secondary to drug or toxin exposure) forms; or
- Primary immunodeficiencies
  - Absent or defective T-cell function (e.g., severe combined immunodeficiency, Wiskott-Aldrich syndrome, X-linked lymphoproliferative syndrome); or
  - Absent or defective natural killer function (e.g., Chediak-Higashi syndrome); or
  - Absent or defective neutrophil function (e.g., Kostmann syndrome, chronic granulomatous disease, leukocyte adhesion defect); or
- Inherited metabolic disease
  - Lysosomal and peroxisomal storage disorders except Hunter, Sanfilippo, and Morquio syndromes; or
- Genetic disorders affecting skeletal tissue
  - Infantile malignant osteopetrosis (Albers-Schonberg disease or marble bone disease).

Allogeneic HCT that does not meet the criteria of this policy is considered not medically necessary.

The following guidelines list the immunodeficiencies that have been successfully treated by allo-HCT:

- Lymphocyte immunodeficiencies
  - Adenosine deaminase deficiency
  - Artemis deficiency
  - Calcium channel deficiency
  - CD 40 ligand deficiency
  - Cernunnos/X-linked lymphoproliferative disease deficiency
  - CHARGE syndrome with immune deficiency
  - Common gamma chain deficiency
  - Deficiencies in CD45, CD3, CD8
  - DiGeorge syndrome
  - DNA ligase IV deficiency syndrome
  - Interleukin-7 receptor alpha deficiency
  - Janus-associated kinase 3 (JAK3) deficiency
  - Major histocompatibility class II deficiency
  - Omenn syndrome
  - Purine nucleoside phosphorylase deficiency
  - Recombinase-activating gene (RAG) 1/2 deficiency
  - Reticular dysgenesis
  - Winged helix deficiency
  - Wiskott-Aldrich syndrome
  - X-linked lymphoproliferative disease
  - Zeta-chain-associated protein-70 (ZAP-70) deficiency

- Phagocytic deficiencies
  - Chediak-Higashi syndrome
  - Chronic granulomatous disease
  - Hemophagocytic lymphohistiocytosis
  - Griscelli syndrome, type 2
  - Interferon-gamma receptor deficiencies
  - Leukocyte adhesion deficiency
  - Severe congenital neutropenias
  - Shwachman-Diamond syndrome
- Other immunodeficiencies
  - Autoimmune lymphoproliferative syndrome
  - Cartilage hair hypoplasia
  - CD25 deficiency
  - Hyper IgD and IgE syndromes
  - Immunodeficiency, centromeric instability, and facial dysmorphism syndrome (ICF syndrome)
  - Immunodysregulation polyendocrinopathy enteropathy X-linked syndrome (IPEX syndrome)
  - Nuclear factor-κ B (NF-κB) essential modulator deficiency
  - NF-κB inhibitor, NF-KB-a deficiency
  - Nijmegen breakage syndrome

For inherited metabolic disorders, allogeneic HCT has been proven effective in some cases of Hurler, Maroteaux-Lamy, and Sly syndromes, childhood onset cerebral X-linked adrenoleukodystrophy, globoid-cell leukodystrophy, metachromatic leukodystrophy, alpha-mannosidosis, and aspartylglucosaminuria. Allogeneic HCT is possibly effective for fucosidosis, Gaucher types 1 and 3, Farber lipogranulomatosis, galactosialidosis, GM1, gangliosidosis, mucolipidosis II (I-cell disease), multiple sulfatase deficiency, Niemann-Pick, neuronal ceroid lipofuscinosis, sialidosis, and Wolman disease. Allogeneic HCT has not been effective in Hunter, Sanfilippo, or Morquio syndromes.

The experience with reduced-intensity conditioning (RIC) and allogeneic HCT for the diseases listed in this policy has been limited to small numbers of individuals and have yielded mixed results, depending upon the disease category. In general, the results have been most promising in the bone marrow failure syndromes and primary immunodeficiencies. In the hemoglobinopathies, success has been hampered by difficulties with high rates of graft rejection, and in adult individuals, severe graft-versus-host-disease (GVHD).

POST-PAYMENT AUDIT STATEMENT

The medical record must include documentation that reflects the medical necessity criteria and is subject to audit by Highmark Health Options at any time pursuant to the terms of your provider agreement.

PLACE OF SERVICE: INPATIENT/OUTPATIENT

Allogeneic hematopoietic cell transplantation for genetic diseases and acquired anemia is typically an outpatient procedure which is only eligible for coverage as an inpatient procedure in special circumstances, including, but not limited to, the presence of a comorbid condition that would require monitoring in a more controlled environment such as the inpatient setting.

CODING REQUIREMENTS

CPT code	Description
38230	Bone marrow harvesting for transplantation.
38240	Hematopoietic progenitor cell (HPC); allogeneic transplantation per donor.

DIAGNOSIS CODES

Codes						
D56.0	D56.1	D56.2	D56.3	D56.5	D56.8	D56.9
D57.00	D57.01	D57.02	D57.03	D57.09	D57.1	D57.20
D57.211	D57.212	D57.213	D57.218	D57.219	D57.40	D57.411
D57.412	D57.413	D57.418	D57.419	D57.42	D57.431	D57.432
D57.433	D57.438	D57.439	D57.44	D57.451	D57.452	D57.453
D57.438	D57.439	D57.80	D57.811	D57.812	D57.813	D57.818
D57.819	D60.0	D60.1	D60.8	D60.9	D61.01	D61.09
D61.1	D61.2	D61.3	D61.810	D61.811	D61.818	D61.82
D61.89	D61.9	D70.0	D81.0	D81.1	D81.2	D81.30
D81.31	D81.32	D81.39	D81.6	D81.7	D81.89	D81.9
D82.0	E75.21	E75.22	E75.240	E75.241	E75.242	E75.243
E75.248	E75.249	E75.3	E76.01	E76.02	E76.03	E76.1
E76.210	E76.211	E76.219	E76.22	E76.29	E76.3	E76.8
E76.9	E77.0	E77.1	E77.8	E77.9	Q78.2	

REIMBURSEMENT

Participating facilities will be reimbursed per their Highmark Health Options contract.

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POLICY UPDATE HISTORY

10/08/2021	Approved in medical policy committee
08/24/2022	Annual review; approved by medical policy committee
09/13/2022	QI/UM approval