MASSACHUSETTS

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Medical Policy

Hematopoietic Cell Transplantation for Autoimmune Diseases

Table of Contents

Policy Number: 192

BCBSA Reference Number: 8.01.25 (For Plan internal use only)
NCD/LCD: NA

Related Policies

Plasma Exchange #466

Policy

Commercial Members: Managed Care (HMO and POS), PPO, and Indemnity Medicare HMO Blue[SM] and Medicare PPO Blue[SM] Members

Autologous or allogeneic hematopoietic cell transplantation is considered **INVESTIGATIONAL** as a treatment of autoimmune diseases, including, but not limited to, the following:

- Multiple sclerosis
- Systemic lupus erythematosus
- Juvenile idiopathic or rheumatoid arthritis
- Chronic inflammatory demyelinating polyneuropathy
- Type 1 diabetes mellitus.

Autologous hematopoietic cell transplantation is considered **MEDICALLY NECESSARY** as a treatment of systemic sclerosis/scleroderma if all of the following conditions are met:

- Adult individuals <60 years of age; **AND**
- Maximum duration of condition of 5 years; **AND**
- Modified skin scores ≥15; **AND**
- Internal organ involvement*; **AND**
- History of < 6 months treatment with cyclophosphamide; **AND**
- No active gastric antral vascular ectasia; **AND**
- Do not have any exclusion criteria. **

*Autologous HCT should be considered for individuals with systemic sclerosis (SSc) only if the condition is rapidly progressing and the prognosis for survival is poor. An important factor influencing the occurrence of treatment-related adverse effects and response to treatment is the level of internal organ

involvement. If organ involvement is severe and irreversible, HCT is not recommended. Below are clinical measurements which can be used to guide the determination of organ involvement.

Individuals with internal organ involvement indicated by the following measurements **may be considered** for autologous HCT:

- Cardiac: abnormal electrocardiogram; **OR**
- Pulmonary: diffusing capacity of carbon monoxide (DLCo) <80% of predicted value; decline of forced vital capacity (FVC) of ≥10% in last 12 months; pulmonary fibrosis; ground glass appearance on high resolution chest CT; **OR**
- Renal: scleroderma-related renal disease.

Individuals with internal organ involvement indicated by the following measurements **should not be considered or autologous HCT:

- Cardiac: left ventricular ejection fraction <50%; tricuspid annular plane systolic excursion <1.8 cm; pulmonary artery systolic pressure >40 mm Hg; mean pulmonary artery pressure >25 mm Hg
- Pulmonary: DLCo <40% of predicted value; FVC <45% of predicted value
- Renal: creatinine clearance <40 ml/minute.

Autologous hematopoietic cell transplantation as a treatment of systemic sclerosis/scleroderma not meeting the above criteria is considered **INVESTIGATIONAL**.

Prior Authorization Information

Inpatient

- For services described in this policy, precertification/preauthorization **IS REQUIRED** for all products if the procedure is performed **inpatient**.

Outpatient

- For services described in this policy, see below for products where prior authorization **might be required** if the procedure is performed **outpatient**.

	Outpatient
Commercial Managed Care (HMO and POS)	Prior authorization is **required**.
Commercial PPO and Indemnity	Prior authorization is **required**.
Medicare HMO Blue℠	Prior authorization is **required**.
Medicare PPO Blue℠	Prior authorization is **required**.

Requesting Prior Authorization Using Authorization Manager

Providers will need to use Authorization Manager to submit initial authorization requests for services. Authorization Manager, available 24/7, is the quickest way to review authorization requirements, request authorizations, submit clinical documentation, check existing case status, and view/print the decision letter. For commercial members, the requests must meet medical policy guidelines.

To ensure the service request is processed accurately and quickly:

- Enter the facility's NPI or provider ID for where services are being performed.
- Enter the appropriate surgeon's NPI or provider ID as the servicing provider, *not* the billing group.

Authorization Manager Resources

Refer to our Authorization Manager page for tips, guides, and video demonstrations.

CPT Codes / HCPCS Codes / ICD Codes

Inclusion or exclusion of a code does not constitute or imply member coverage or provider reimbursement. Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage as it applies to an individual member.

Providers should report all services using the most up-to-date industry-standard procedure, revenue, and diagnosis codes, including modifiers where applicable.

The following codes are included below for informational purposes only; this is not an all-inclusive list.

The above medical necessity criteria MUST be met for the following codes to be covered for Commercial Members: Managed Care (HMO and POS), PPO, Indemnity, Medicare HMO Blue and Medicare PPO Blue:

CPT Codes

CPT codes:	Code Description
38241	Bone marrow or blood-derived peripheral stem-cell transplantation; autologous

HCPCS Codes

HCPCS codes:	Code Description
S2150	Bone marrow or blood-derived peripheral stem-cell harvesting and transplantation, allogeneic or autologous, including pheresis, high-dose chemotherapy, and the number of days of post-transplant care in the global definition (including drugs; hospitalization; medical surgical, diagnostic and emergency services)

ICD-10 Procedure Codes

ICD-10-PCS procedure codes:	Code Description
30233G0	Transfusion of Autologous Bone Marrow into Peripheral Vein, Percutaneous Approach
30233X0	Transfusion of Autologous Cord Blood Stem Cells into Peripheral Vein, Percutaneous Approach
30233Y0	Transfusion of Autologous Hematopoietic Stem Cells into Peripheral Vein, Percutaneous Approach
30243G0	Transfusion of Autologous Bone Marrow into Central Vein, Percutaneous Approach
30243X0	Transfusion of Autologous Cord Blood Stem Cells into Central Vein, Percutaneous Approach
30243Y0	Transfusion of Autologous Hematopoietic Stem Cells into Central Vein, Percutaneous Approach
3E03305	Introduction of Other Antineoplastic into Peripheral Vein, Percutaneous Approach
3E04305	Introduction of Other Antineoplastic into Central Vein, Percutaneous Approach
3E05305	Introduction of Other Antineoplastic into Peripheral Artery, Percutaneous Approach
3E06305	Introduction of Other Antineoplastic into Central Artery, Percutaneous Approach

Description

Autoimmune Disease Treatment

Immune suppression is a common treatment strategy for many autoimmune diseases, particularly rheumatic diseases (eg, rheumatoid arthritis [RA], systemic lupus erythematosus [SLE], scleroderma). Most patients with autoimmune disorders respond to conventional therapies, which consist of anti-inflammatory agents, immunosuppressants, and immunomodulating drugs; however, conventional drug therapies are not curative, and a proportion of patients suffer from autoimmune diseases that range from severe to recalcitrant to rapidly progressive. It is for this group of patients with a severe autoimmune disease that alternative therapies have been sought, including hematopoietic cell transplantation (HCT). The primary concept underlying the use of HCT for these diseases is this: ablating and “resetting” the

immune system can alter the disease process by inducing a sustained remission that possibly leads to cure.[1]

Hematopoietic Cell Transplantation
Hematopoietic cell transplantation is a procedure in which hematopoietic stem cells are intravenously infused to restore bone marrow and immune function in cancer patients who receive bone marrow-toxic doses of cytotoxic drugs with or without whole-body radiotherapy. Hematopoietic stem cells may be obtained from the transplant recipient (autologous HCT) or a donor (allogeneic HCT [allo-HCT]). They can be harvested from bone marrow, peripheral blood, or umbilical cord blood shortly after delivery of neonates.

Immunologic compatibility between infused hematopoietic stem cells and the recipient is not an issue in autologous HCT. In allogeneic stem cell transplantation, immunologic compatibility between donor and patient is a critical factor for achieving a successful outcome. Compatibility is established by typing of human leukocyte antigens (HLA) using cellular, serologic, or molecular techniques. The term HLA refers to the gene complex expressed at the HLA-A, -B, and -DR (antigen-D related) loci on each arm of chromosome 6. An acceptable donor will match the patient at all or most of the HLA loci.

Conditioning for Hematopoietic Cell Transplantation
Conventional Conditioning
The conventional ("classical") practice of allo-HCT involves administration of cytotoxic agents (eg, cyclophosphamide, busulfan) with or without total body irradiation at doses sufficient to cause bone marrow ablation in the recipient. The beneficial treatment effect of this procedure is due to a combination of the initial eradication of malignant cells and subsequent graft-versus-malignancy (GVM) effect mediated by non-self-immunologic effector cells. While the slower GVM effect is considered the potentially curative component, it may be overwhelmed by existing disease in the absence of pretransplant conditioning. Intense conditioning regimens are limited to patients who are sufficiently medically fit to tolerate substantial adverse effects. These include opportunistic infections secondary to loss of endogenous bone marrow function and organ damage or failure caused by cytotoxic drugs. Subsequent to graft infusion in allo-HCT, immunosuppressant drugs are required to minimize graft rejection and graft-versus-host disease (GVHD), which increases susceptibility to opportunistic infections.

The success of autologous HCT is predicated on the potential of cytotoxic chemotherapy, with or without radiotherapy, to eradicate cancerous cells from the blood and bone marrow. This permits subsequent engraftment and repopulation of the bone marrow with presumably normal hematopoietic stem cells obtained from the patient before undergoing bone marrow ablation. Therefore, autologous HCT is typically performed as consolidation therapy when the patient's disease is in complete remission. Patients who undergo autologous HCT are also susceptible to chemotherapy-related toxicities and opportunistic infections before engraftment, but not GVHD.

Reduced-Intensity Conditioning Allogeneic Hematopoietic Cell Transplantation
Reduced-intensity conditioning (RIC) refers to the pretransplant use of lower doses of cytotoxic drugs or less intense regimens of radiotherapy than are used in traditional full-dose myeloablative conditioning treatments. Although the definition of RIC is variable, with numerous versions employed, all regimens seek to balance the competing effects of relapse due to residual disease and non-relapse mortality. The goal of RIC is to reduce disease burden and to minimize associated treatment-related morbidity and non-relapse mortality in the period during which the beneficial GVM effect of allogeneic transplantation develops. Reduced-intensity conditioning regimens range from nearly total myeloablative to minimally myeloablative with lymphoablation, with intensity tailored to specific diseases and patient condition. Patients who undergo RIC with allo-HCT initially demonstrate donor cell engraftment and bone marrow mixed chimerism. Most will subsequently convert to full-donor chimerism. In this review, the term *reduced-intensity conditioning* will refer to all conditioning regimens intended to be nonmyeloablative.

Summary
Description

Most patients with autoimmune disorders respond to conventional drug therapies; however, conventional drug therapies are not curative and a proportion of patients suffer from autoimmune diseases that range from severe to recalcitrant to rapidly progressive. It is in this group of patients with a severe autoimmune disease that alternative therapies have been sought, including hematopoietic cell transplantation (HCT).

Summary of Evidence

For individuals with multiple sclerosis who receive HCT, the evidence includes randomized controlled trials (RCTs), systematic reviews, and several nonrandomized studies. Relevant outcomes are overall survival (OS), health status measures, quality of life (QOL), and treatment-related mortality (TRM) and morbidity. Systematic reviews are primarily comprised of observational data. One RCT compared HCT with mitoxantrone, and the trial reported intermediate outcomes (number of new T2 magnetic resonance imaging lesions); the group randomized to HCT developed significantly fewer lesions than the group receiving conventional therapy. The other RCT compared nonmyeloablative HCT results in patients with continued disease-modifying therapy and found a benefit to HCT in prolonged time to disease progression. The findings of the nonrandomized studies revealed improvements in clinical parameters following HCT compared with baseline. Adverse event rates were high, and most studies reported treatment-related deaths. Controlled trials (with appropriate comparator therapies) reporting on clinical outcomes are needed to demonstrate efficacy. The evidence is insufficient to determine that the technology results in an improvement in the net health outcome.

For individuals with systemic sclerosis/scleroderma who receive HCT, the evidence includes systematic reviews, 3 RCTs, and observational studies. Relevant outcomes are OS, symptoms, health status measures, QOL, and TRM and morbidity. All 3 RCTs compared cyclophosphamide conditioning plus autologous HCT with cyclophosphamide alone. Patients in the RCTs were adults <60 years of age with a maximum duration of disease of 5 years, modified Rodnan skin scores (mRSS) >15, and internal organ involvement. Patients with severe and irreversible organ involvement were excluded from the trials. Short-term results of the RCTs show higher rates of adverse events and TRM among patients receiving autologous HCT compared with patients receiving chemotherapy alone. However, long-term improvements (4 years) in overall mortality and clinical outcomes such as mRSS and forced vital capacity in patients receiving HCT compared with patients receiving cyclophosphamide alone were consistently reported in all RCTs. Due to sample size limitations in 2 of the 3 RCTs, statistical significance was found only in the larger RCT. The evidence is sufficient to determine that the technology results in an improvement in the net health outcome.

For individuals with systemic lupus erythematosus who receive HCT, the evidence includes a systematic review and case series. Relevant outcomes are OS, symptoms, QOL, and TRM and morbidity. Studies were heterogeneous in conditioning regimens and source of cells. The largest series (n=50) reported an overall 5-year survival rate of 84% and the probability of disease-free survival was 50%. Additional data are needed from controlled studies to demonstrate efficacy. The evidence is insufficient to determine that the technology results in an improvement in the net health outcome.

For individuals with juvenile idiopathic or rheumatoid arthritis who receive HCT, the evidence includes registry data and a case series. Relevant outcomes are OS, symptoms, QOL, and TRM and morbidity. The registry included 50 patients with juvenile idiopathic or rheumatoid arthritis. The overall drug-free remission rate was approximately 50% in the registry patients and 69% in the smaller case series. Additional data are needed from controlled studies to demonstrate efficacy. The evidence is insufficient to determine that the technology results in an improvement in the net health outcome.

For individuals with chronic inflammatory demyelinating polyneuropathy who receive HCT, the evidence includes recent observational study and case reports. Relevant outcomes are OS, symptoms, health status measures, QOL, and TRM and morbidity. Additional data are needed from controlled studies to demonstrate efficacy. The evidence is insufficient to determine that the technology results in an improvement in the net health outcome.

For individuals with type 1 diabetes who receive HCT, the evidence includes case series and 2 meta-analyses. Relevant outcomes are OS, symptoms, health status measures, QOL, and TRM and morbidity.

While a substantial proportion of patients tended to become insulin-free after HCT, remission rates were high. The meta-analyses revealed that HCT may improve HbA1 and C-peptide levels compared with baseline values and compared with insulin. One meta-analysis found that HCT is more effective in patients with type 1 diabetes compared with type 2 diabetes, and when the treatment is administered soon after the diagnosis. Certain factors limit the conclusions that can be drawn about the overall effectiveness of HCT in treating diabetes; those factors are heterogeneity in the stem cell types, cell number infused, and infusion methods. The evidence is insufficient to determine that the technology results in an improvement in the net health outcome.

For individuals with other autoimmune diseases (eg, Crohn disease, immune cytopenias, relapsing polychondritis) who receive HCT, the evidence includes 1 RCT and small retrospective studies and case series. Relevant outcomes are OS, symptoms, health status measures, QOL, and TRM and morbidity. The RCT was conducted on patients with Crohn disease. At 1 year follow-up, 1 patient in the control group and 2 patients in the HCT group achieved remission. Data are needed from additional controlled studies to demonstrate efficacy. The evidence is insufficient to determine that the technology results in an improvement in the net health outcome.

Policy History

Date	Action
3/2024	Annual policy review. References updated. Policy statements unchanged.
9/2023	Policy clarified to include prior authorization requests using Authorization Manager.
3/2023	Annual policy review. Minor editorial refinements to policy statements; intent unchanged.
3/2021	Annual policy review. Description, summary, and references updated. Policy statements unchanged.
10/2020	Clarified coding information
4/2020	Bone marrow harvesting codes were removed. Outpatient prior authorization is not required.
3/2020	Annual policy review. Description, summary, and references updated. Policy statements unchanged.
6/2019	Annual policy review. New medically necessary indications described. Policy statement for systemic sclerosis was changed from “investigational” to “medically necessary.” Clarified coding information. Effective 6/2019.
1/2019	Outpatient prior authorization is required for all commercial products including Medicare Advantage. Effective 1/1/2019.
2/2018	Annual policy review. New references added.
9/2017	Annual policy review. “Stem” removed from title and Policy. Policy statement unchanged.
10/2016	Clarified coding information.
3/2016	Annual policy review. New references added.
12/2014	Annual policy review. New references added.
6/2014	Updated Coding section with ICD10 procedure and diagnosis codes. Effective 10/2015.
3/2014	Annual policy review. New investigational indications described. Effective 3/1/2014. Coding information clarified.
12/2012	Updated to add new CPT code 38243.
11/2011-4/2012	Medical policy ICD 10 remediation: Formatting, editing and coding updates. No changes to policy statements.
7/2011	Reviewed - Medical Policy Group – Hematology and Oncology. No changes to policy statements.
9/2010	Reviewed - Medical Policy Group – Hematology and Oncology. No changes to policy statements.
6/01/2010	Medical Policy 192 effective 6/01/2010.

Information Pertaining to All Blue Cross Blue Shield Medical Policies

Click on any of the following terms to access the relevant information:

Medical Policy Terms of Use
Managed Care Guidelines
Indemnity/PPO Guidelines
Clinical Exception Process
Medical Technology Assessment Guidelines

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