

## Corporate Medical Policy

### Hematopoietic Stem-Cell Transplantation for Autoimmune Diseases

<b>File Name:</b>	hematopoietic_stem-cell_transplantation_for_autoimmune_diseases
<b>Origination:</b>	2/2001
<b>Last CAP Review:</b>	11/2011
<b>Next CAP Review:</b>	11/2012
<b>Last Review:</b>	11/2011

#### Description of Procedure or Service

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##### Autoimmune Diseases

Autoimmune diseases represent a heterogeneous group of immune-mediated disorders, with some of the most common types being multiple sclerosis (MS), rheumatoid arthritis (RA), systemic lupus erythematosus (SLE), and systemic sclerosis/scleroderma. The National Institutes of Health estimate that 5%–8% of Americans have an autoimmune disorder.

The pathogenesis of autoimmune diseases is not well understood but appears to involve underlying genetic susceptibility and environmental factors that lead to loss of self-tolerance, culminating in tissue damage by the patient's own immune system (T cells).

Immune suppression is a common treatment strategy for many of these diseases, particularly the rheumatic diseases (e.g., RA, SLE, and scleroderma). Most patients with autoimmune disorders respond to conventional therapies, which consist of anti-inflammatory agents, immunosuppressants, and immunomodulating drugs. However, these drugs are not curative, and a proportion of patients will have severe, recalcitrant, or rapidly progressive disease. It is in this group of patients with severe autoimmune disease that alternative therapies have been sought, including hematopoietic stem-cell transplantation (HSCT).

HSCT in autoimmune disorders raises the question of whether ablating and “resetting” the immune system can alter the disease process and sustain remission and possibly lead to cure.

##### Hematopoietic Stem-Cell Transplantation

Hematopoietic stem cell transplantation (HSCT) refers to a procedure in which hematopoietic stem cells are infused to restore bone marrow function in patients who receive bone-marrow-toxic doses of cytotoxic drugs with or without whole body radiation therapy. Hematopoietic stem cells may be obtained from the transplant recipient (autologous HCT) or from a donor (allogeneic HCT). They can be harvested from bone marrow, peripheral blood, or umbilical cord blood shortly after delivery of neonates. Although cord blood is an allogeneic source, the stem cells in it are antigenically “naïve” and thus are associated with a lower incidence of rejection or graft-versus-host disease (GVHD). Cord blood is discussed in greater detail in policy, Cord Blood as a Source of Stem Cells.

Immunologic compatibility between infused hematopoietic stem cells and the recipient is not an issue in autologous HSCT. However, immunologic compatibility between donor and patient is a critical factor for achieving a good outcome of allogeneic HSCT. Compatibility is established by typing of human leukocyte antigens (HLA) using cellular, serologic, or molecular techniques. HLA refers to the tissue type expressed at the Class I and Class II loci on chromosome 6. Depending on

# Hematopoietic Stem-Cell Transplantation for Autoimmune Diseases

the disease being treated, an acceptable donor will match the patient at all or most of the HLA loci (with the exception of umbilical cord blood).

## **Autologous Stem-Cell Transplantation for Autoimmune Diseases**

The goal of autologous HSCT in patients with autoimmune diseases is to eliminate self-reactive lymphocytes (lymphoablative) and generate new self-tolerant lymphocytes. This approach is in contrast to destroying the entire hematopoietic bone marrow (myeloablative), as is often performed in autologous HSCT for hematologic malignancies. However, there is currently no standard conditioning regimen for autoimmune diseases and both lymphoablative and myeloablative regimens are used. The efficacy of the different conditioning regimens has not been compared in clinical trials.

Currently, for autoimmune diseases, autologous transplant is preferred over allogeneic, in part because of the lower toxicity of autotransplant relative to allogeneic, the GVHD associated with allogeneic transplant, and the need to administer post-transplant immunosuppression after an allogeneic transplant.

## **Allogeneic Stem-Cell Transplantation for Autoimmune Diseases**

The experience of using allogeneic HSCT for autoimmune diseases is currently limited, but has two potential advantages over autologous transplant. First, the use of donor cells from a genetically different individual could possibly eliminate genetic susceptibility to the autoimmune disease and potentially result in a cure. Second, there exists a possible graft-versus-autoimmune effect, in which the donor T cells attack the transplant recipient's autoreactive immune cells.

***\*\*\*Note: This Medical Policy is complex and technical. For questions concerning the technical language and/or specific clinical indications for its use, please consult your physician.***

## **Policy**

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**Hematopoietic stem-cell transplantation for Autoimmune Diseases is considered investigational for all applications. BCBSNC does not cover investigational services or procedures.**

**Some patients may be eligible for coverage under Clinical Trials. Refer to the policy on Clinical Trial Services for Life-Threatening Conditions.**

## **Benefits Application**

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This medical policy relates only to the services or supplies described herein. Please refer to the Member's Benefit Booklet for availability of benefits. Member's benefits may vary according to benefit design; therefore member benefit language should be reviewed before applying the terms of this medical policy.

**Some health benefit plans may exclude benefits for transplantation.**

## **When Hematopoietic Stem-Cell Transplantation for Autoimmune Diseases is covered**

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Not applicable.

# Hematopoietic Stem-Cell Transplantation for Autoimmune Diseases

## When Hematopoietic Stem-Cell Transplantation for Autoimmune Diseases is not covered

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Autologous or allogeneic hematopoietic stem-cell transplantation is considered investigational as a treatment of autoimmune diseases, including, but not limited to multiple sclerosis (MS), juvenile idiopathic and rheumatoid arthritis (RA), systemic lupus erythematosus (SLE), systemic sclerosis/scleroderma, and type 1 diabetes mellitus.

## Policy Guidelines

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Initial studies focused on using HSCT as salvage therapy for end-stage treatment of refractory autoimmune diseases. More recent experience has better helped to define which patients are most likely to benefit from HSCT, and the field has shifted to the use of HSCT earlier in the disease course before irreversible organ damage and to safer and less intense nonmyeloablative conditioning regimens.

The experience with HSCT and autoimmune disorders has been predominantly with autologous transplants. A number of published clinical reports with follow-up have demonstrated the safety and in some patients (particularly those with systemic sclerosis, SLE, and MS) the impact of HSCT in selected autoimmune diseases.

Although some of the initial results have been promising, this field continues to evolve. Many trials (randomized and nonrandomized) are currently recruiting or ongoing comparing the use of HSCT to conventional therapy for most of the diseases addressed in this policy; the results of these trials will further define the role of HSCT in the management of these diseases.

HSCT in the treatment of juvenile idiopathic arthritis and type 1 diabetes mellitus are added as additional investigational indications due to the evolving nature of HSCT for these conditions.

## Billing/Coding/Physician Documentation Information

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This policy may apply to the following codes. Inclusion of a code in this section does not guarantee that it will be reimbursed. For further information on reimbursement guidelines, please see Administrative Policies on the Blue Cross Blue Shield of North Carolina web site at [www.bcbsnc.com](http://www.bcbsnc.com). They are listed in the Category Search on the Medical Policy search page.

*Applicable service codes: 38205, 38206, 38230, 38232, 38240, 38241, 38242, S2150*

BCBSNC may request medical records for determination of medical necessity. When medical records are requested, letters of support and/or explanation are often useful, but are not sufficient documentation unless all specific information needed to make a medical necessity determination is included.

## Scientific Background and Reference Sources

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### Bone Marrow Transplant for Autoimmune Disease

BCBSA Medical Policy Reference Manual, 12/1/1999

# Hematopoietic Stem-Cell Transplantation for Autoimmune Diseases

BCBSA TEC Evaluation, Tab 1, June 2000

BCBSA Medical Policy Reference Manual, 8/18/2000

ECRI Health Technology Assessment; Executive Briefings, Sept. 2000; No. 93

BCBSA TEC Evaluation 2001

BCBSA Medical Policy Reference Manual, 2/15/2002; 8.01.25

Specialty Matched Consultant Advisory Panel - 11/2002

BCBSA Medical Policy Reference Manual [Electronic Version]. 8.01.25, 7/15/2004

Specialty Matched Consultant Advisory Panel - 11/2004

BCBSA Medical Policy Reference Manual [Electronic Version]. 8.01.25, 4/25/06

Specialty Matched Consultant Advisory Panel - 11/2006

BCBSA Medical Policy Reference Manual [Electronic Version]. 8.01.25, 9/11/08

Specialty Matched Consultant Advisory Panel - 11/2008

## **Hematopoietic Stem-Cell Transplantation for Autoimmune Diseases**

BCBSA Medical Policy Reference Manual [Electronic Version]. 8.01.25, 9/10/2009

Specialty Matched Consultant Advisory Panel - 11/2010

BCBSA Medical Policy Reference Manual [Electronic Version]. 8.01.25, 9/16/2010

BCBSA Medical Policy Reference Manual [Electronic Version]. 8.01.25, 9/1/2011

Specialty Matched Consultant Advisory Panel – 11/2011

## **Policy Implementation/Update Information**

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### **Bone Marrow Transplant for Autoimmune Disease**

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| 1/01 | Specialty Matched Consultant Advisory Group.   |
| 2/01 | Original policy issued.  |
| 5/02 | Policy statement reaffirmed and reference sources added. Codes 38220 and 38221 added to Billing and Coding section.  |
| 2/03 | Specialty Matched Consultant Advisory Panel review 11/2002. No change in criteria. Codes 86812-86822 removed; codes 38231 and 86915 deleted and codes 38242, 38205 |

# Hematopoietic Stem-Cell Transplantation for Autoimmune Diseases

and 38206 added to the Billing/Coding section. System coding changes.

- 1/04 Benefits Application and Billing/Coding sections updated for consistency.
- 2/04 Individual CPT codes listed for CPT code ranges 38240-38242 under Billing/Coding section.
- 7/29/04 HCPCS code S2150 added to Billing/Coding section.
- 12/9/04 Specialty Matched Consultant Advisory Panel review 11/29/2004. No change to criteria. Description of Procedure or Service revised. Rationale added in Policy Guidelines section. Policy number added to Policy Key Words section. Hematopoietic and Opportunistic added to Definitions. References added.
- 12/11/06 Specialty Matched Consultant Advisory Panel review 11/6/06. No changes to policy statement. Added the following statement to the "Policy" section; Some patients may be eligible for coverage under Clinical Trials. Refer to the policy on Clinical Trial Services for Life-Threatening Conditions. Updated rationale in "Policy Guidelines" section. References added.
- 12/22/08 Specialty Matched Consultant Advisory Panel review 11/13/2008. No change to policy statement. "Policy Guidelines" section updated. References added. (btw)
- 6/22/10 Policy Number(s) removed. (amw)

## **Hematopoietic Stem-Cell Transplantation for Autoimmune Diseases**

- 1/4/11 Policy name changed from "Bone Marrow Transplant for Autoimmune Diseases" to "Hematopoietic Stem-Cell Transplantation for Autoimmune Diseases". Specialty Matched Consultant Advisory Panel review 11/29/10. No change to policy statement. (btw)
- 3/1/11 "Description" section revised. Added indications of juvenile idiopathic arthritis and diabetes mellitus to the "When Not Covered" section as investigational. No change to intent of policy. "Policy Guidelines" updated. References added. Medical Director review 2/9/2011. (btw)
- 1/10/12 Specialty Matched Consultant Advisory Panel review 11/30/11. No change to policy statement. References added. (btw)
- 2/21/12 New 2012 CPT code 38232 added to Billing/Coding section. (btw)

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Medical policy is not an authorization, certification, explanation of benefits or a contract. Benefits and eligibility are determined before medical guidelines and payment guidelines are applied. Benefits are determined by the group contract and subscriber certificate that is in effect at the time services are rendered. This document is solely provided for informational purposes only and is based on research of current medical literature and review of common medical practices in the treatment and diagnosis of disease. Medical practices and knowledge are constantly changing and BCBSNC reserves the right to review and revise its medical policies periodically.