

Policy #: 192

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Title

Hematopoietic Stem Cell Transplantation for Autoimmune Diseases

Description

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. Certain hematologic malignancies, aplastic anemia, and inborn errors of metabolism are treated with HSCT. However, its usage in autoimmune diseases has only been performed in approximately 1,000 patients in the last decade.

The rationale for HSCT for autoimmune disease is based on studies in experimental animal models, and on observations of remissions of autoimmune disease in patients who received HSCT for hematologic malignancies.

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).

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. Depending on 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.

When services are covered for commercial products and for Medicare HMO Blue and Medicare PPO Blue¹

There are no covered indications for this procedure as it does not meet the Medical Technology Assessment Guidelines #[350](#).

When services are not covered for commercial products or for Medicare HMO Blue and Medicare PPO Blue¹

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

Individual consideration

All our medical policies are written for the majority of people with a given condition. Each policy is based on medical science. For many of our medical policies, each individual's unique clinical circumstances may be considered in light of current scientific literature. For consideration of an individual patient, physicians may send relevant clinical information to:

For services already billed

Blue Cross Blue Shield of Massachusetts
Provider Appeals
PO Box 986065
Boston, MA 02298

Prior to performance of service

Blue Cross Blue Shield of Massachusetts
Case Creation/Medical Policy
One Enterprise Drive
Quincy, MA 02171
Tel: 1-800-327-6716
Fax: 1-888-282-0780

Authorization Information

This is not a covered service.

Other information

For our Medical Technology Assessment Guidelines, see document #350.

Coding information

This is not a covered service. Any codes submitted to bill for this service will reject leaving no patient balance.

Policy update history

New policy, effective 6/01/10. Reviewed 9/2010 MPG-Hematology and Oncology, no changes in coverage were made. Updated 1/2011 to clarify additional non-covered connective tissue disorders. References updated. Reviewed 7/2011 MPG – Hematology and Oncology, no changes in coverage were made. Updated 5/1/2012 with additional references based on BCBSA national policy reviewed 9/1/2011.

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Footnotes

¹ Based on BCBSA policy # 8.01.25, Hematopoietic Stem Cell Transplantation for Autoimmune Diseases, reviewed September 2011.