Molina Clinical Policy
Hematopoietic Stem Cell Transplantation for Non – Cancer Diseases
Policy No. 454

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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

This policy covers hematopoietic stem cell transplantation for the following diseases:

Inborn Errors of Immunity	Inborn Errors of Metabolism

Hematopoietic Stem Cell Transplantation (HSCT) refers to the infusion of multipotent hematopoietic stem cells into a recipient, using cells from either a donor or the patient's own body, to restore hematopoietic function. Hematopoietic stem cells are immature cells that can differentiate into erythrocytes, leukocytes, or platelets, and are typically harvested from bone barrow, peripheral blood, or umbilical cord blood. HSCT can be autologous (using the patient's own stem cells) or allogeneic (using donor-derived stem cells). In allogeneic HSCT, optimal outcomes are achieved when the donor is human leukocyte antigen (HLA)-identical, usually a sibling. HLA mismatched or haploidentical transplants increase the risk of graft rejection and non-malignant hematological conditions. HSCT can be utilized in the treatment of a variety of diseases from blood cancers to solid tumors (Chao 2024; Khaddour et al. 2023; [1-2]Negrin 2025).

Haploidentical Allogeneic HSCT is becoming a viable alternative for patients in need of a bone marrow transplant but lack a fully matched donor of stem cells. In general, allogeneic hematopoietic cell transplantation may cure a broad variety of malignant and non-malignant disorders. The hematopoietic stem cells required are obtained from a related or unrelated donor's bone marrow or peripheral blood. For best outcomes, the stem cell donor is a human leukocyte antigen (HLA)-matched sibling; however, there is only a 25% chance a sibling will be a full match for a patient in need of a stem cell transplant. When there is not an HLA-matched sibling, alternative sources of donor grafts may be used, including suitably HLA-matched adult unrelated donors, umbilical cord blood stem cells, and partially HLA-mismatched, or HLA-haploidentical donors are options. Haploidentical related donors have a 50% match for important HLA markers. There are advantages and disadvantages of HLA-haploidentical HSCT. Advantages include the rapid availability of donor stem cell sources from family and graft versus leukemic effect which may improve overall survival. Haploidentical family members are available much faster than the many months it takes to conduct a nationwide search for unrelated donors, which means this alternative donor source may be especially relevant for minority ethnic groups for which well-matched unrelated donors are less common. The disadvantage of haploidentical allogeneic HSCT is alloreactivity, which can lead to graft rejection and graft versus host disease (Fuchs & Luznik 2024; NCCN 2026).

Inborn Errors of Immunity (IEIs), historically called primary immunodeficiency disorders, are congenital defects of the immune system that result in the impaired ability to fight pathogens and manifest in patients as failure to thrive, autoimmune disease, and/or recurrent/chronic infections. IEIs are a vast and growing cohort of hundreds of genetic mutations that can produce disease states that range from severe and life-threatening beginning in the neonatal period to mild disorders diagnosed in adulthood. IEIs may be the primary diagnosis, i.e., Severe Combined Immunodeficiency Disorder (SCID), or a part of an overarching genetic syndrome, and are treated based on the specific phenotype and severity of disease (Keller 2025). Treatment can include immunoglobulin therapy, antimicrobial therapies, enzyme replacement therapy, gene therapy, HSCT, and more. HSCT is a critical therapy in the treatment of SCID, as allogeneic

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HSCT allows immune reconstitution. While HSCT is the primary curative treatment of choice for SCID, it may also be a treatment option for many other types of IEIs. However, as there are many life-threatening complications of allogeneic HSCT, risks and benefits must be weighed, especially for indications that are less clear ([1-2]Dvorak 2025).

Inborn Errors of Metabolism (IEMs) are rare congenital metabolic disorders that lead to the accumulation of metabolites in the body due to enzyme or enzyme cofactor absence or dysfunction in various metabolic pathways. As IEMs encompass a large and complex group of diseases, clinical manifestations occur across all body systems and commonly involve neurologic, musculoskeletal, gastrointestinal, and integumentary dysfunction. Many IEMs are tested during standard newborn screens with the aim of early detection. With early detection, ideally prior to symptom manifestation, treatment involving enzyme replacement therapy and diet modification can slow or prevent disease progression all together. Some IEMs, such as lysosomal storage diseases and peroxisomal metabolic disorders, can be treated with HSCT. The best example of HSCT in the treatment for IEMs is in children under 2 years of age diagnosed with mucopolysaccharidoses type IH (Hurler syndrome). HSCT is also a treatment option for metachromatic leukodystrophy, X-Linked adrenoleukodystrophy, and Krabbe disease. HSCT is less established as a standard of care in other IEMs, and the risks and benefits must always be weighed for each patient and their unique clinical case (Kaplan 2024; Kaplan 2026; Jain & De Jesus 2023; Sutton 2025; Tan et al. 2019)**.**

RELATED POLICIES

MCP-459: Pre-Transplant and Transplant Evaluations
MCP-472: Allogeneic Processed Thymus Tissue–agdc (Rethymic)

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.

Please see *MCP-459 Pre-Transplant and Transplant Evaluations* for pre-transplant criteria and transplant evaluation criteria that must be met prior to hematopoietic stem cell transplantation (HSCT).

Inborn Errors of Immunity

Medically Necessary

1. *Allogenic HSCT (*ablative or non-myeloablative) from an appropriate human leukocyte antigen (HLA) matched sibling or well-matched donor may be **considered medically necessary** when ALL *MCP 459 Pre-Transplant and Transplant Evaluations* criteria are met, AND treatment is intended for ONE of the following:
    a. Absent, severely diminished, or dysregulated T-cell function (e.g., SCID, non-SCID combined immunodeficiencies)
    b. Absent, severely diminished, or dysregulated natural killer cell function (e.g., Chediak-Higashi syndrome)
    c. Absent, severely diminished, or dysregulated neutrophil function (e.g., Leukocyte adhesion deficiency, Kostmann syndrome)
    Note: Refer to supplemental information section for more examples of additional applicable inborn errors of immunity, as those listed as examples above are not an exhaustive list of disease states eligible for HSCT.

2. *Repeat Allogenic HSCT* may be **considered medically necessary** only once in the case of primary graft failure OR failure to engraft

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Investigational, Unproven, and/or Not Medically Necessary

1. Thymus transplantation is the preferred definitive treatment for inborn errors of immunity due to athymia (e.g., congenital athymia, DiGeorge syndrome). HSCT may be considered medically necessary ONLY when thymus transplantation is not available or when urgent immune reconstitution is required due to life-threatening infection, particularly from an HLA-matched sibling donor.
2. Autologous HSCT
3. A planned tandem allogeneic HSCT
4. Hematopoietic stem cell collection, storage, and freezing for a future unplanned transplant, including in utero collection

Continuation of Therapy criteria may be found in MCP 459 Pre-Transplant and Transplant Evaluations

Inborn Errors of Metabolism

Medically Necessary

1. *Allogenic HSCT* (ablative or non-myeloablative) from an appropriate HLA matched sibling or well-matched donor may be **considered medically necessary** for the treatment of inborn errors of metabolism when ALL *MCP 459 Pre-Transplant and Transplant Evaluations* criteria are met AND treatment is intended for ONE of the following:
    a. X-linked adrenoleukodystrophy (ALD) when ALL the following criteria are met:
        i. Childhood cerebral ALD (CCALD) phenotype is present
        ii. Absence of advanced disease
    b. ANY of the following Lysosomal Storage Diseases:
        i. Metachromatic leukodystrophy (MLD) in the absence of advanced disease
        ii. Krabbe disease/Globoid cell leukodystrophy (GLD) in the absence of advanced disease
        iii. Mucopolysaccharidoses (MPS) when ALL the following criteria are met:
            a) Age < 2 years for type IH (Hurler)
            b) Neurologically intact or mild developmental delay, as evidence by a Developmental Quotient > 70
            c) Failed conventional therapy (e.g., diet modification and/or enzyme replacement therapy), if applicable, for type I (Hurler-Scheie, Scheie), type II (Hunter), type III (SanFilippo), type IV (Morquio), type VI (Maroteaux-Lamy), and type VII (Sly)

2. *Repeat Allogenic HSCT* may be **considered medically necessary** only once in the case of primary graft failure OR failure to engraft

Investigational, Unproven, and/or Not Medically Necessary

1. A second or repeat allogeneic (ablative or non-myeloablative) transplant due to persistent, progressive, or relapsed disease
2. Autologous HSCT
3. A planned tandem allogeneic HSCT
4. Hematopoietic stem cell collection, storage, and freezing for a future unplanned transplant, including in utero collection

Haploidentical Allogenic HSCT

Medically Necessary

1. *Haploidentical Allogenic HSCT* may be **considered medically necessary** when there are no HLA matched sibling or well-matched donors, and ALL the following criteria are met:
    a. Member receiving transplant has met all disease specific criteria for HSCT transplantation
    b. Donor must be medically, socially, and psychologically fit to donate
    c. Donor age <40 years preferred over donor age ≥40 years
    d. Recipient does not have anti-donor HLA antibodies of sufficient strength to result in a positive crossmatch result by flow cytometry or by complement-dependent cytotoxicity assay (i.e., a favorable immunological compatibility is established by the recipient lacking sufficient anti-donor antibodies that would result in donor cell death)

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e. No major ABO incompatibility between donor and recipient; major ABO incompatibilities include:
   i. Recipient blood type O: Donor type A, B, or AB
   ii. Recipient blood type A: Donor blood type B or AB
   iii. Recipient blood type B: Donor blood type A or AB
   iv. Recipient blood type AB: No major ABO incompatibilities
f. Matched CMV IgG serologic status between donor and recipient include:
   i. For a recipient who is CMV IgG negative, use a CMV IgG negative donor
   ii. For a recipient who is CMV IgG positive, use a CMV IgG positive donor
g. Use an ABO compatible donor over a minor ABO incompatible donor (ABO compatible transplants are O→O, A→A, B→B, or AB→AB)

Continuation of Therapy criteria may be found in MCP 459 Pre-Transplant and Transplant Evaluations

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

Inborn Errors of Immunity (IEIs)

Non-Randomized Studies, Retrospective Reviews, and Other Evidence

AlQahtani et al. (2025) conducted a retrospective review of 11 patients diagnosed with zeta-chain-associated protein kinase 70 (ZAP70) deficiency treated with HSCT. The median age of treatment with HSCT was 7 months, and eight patients did not receive conditioning prior to HSCT due to active infections. Primary outcome measured was immune reconstitution, defined as a composite of clinical, cellular, functional, and humoral recovery. The median follow up was 7 years (1 – 15), and 8 (73%) of the 11 patients remain alive, while three died due to transplant related complications. The eight surviving patients showed evidence of immune reconstitution after HSCT, as demonstrated by resolution of recurrent infections, clearance of viral reactivations, and they were able to discontinue antimicrobial prophylaxis. One patient needed a stem cell boost post-transplant and another required a second HSCT to achieve immune reconstitution. Seven patients discontinued IVIG replacement and were able to maintain normal serum IgG, IgA, IgM. One patient demonstrated partial functional reconstitution, but achieved adequate CD8+ T-cell counts and remained clinically well without infection. The authors concluded that HSCT remains the only curative treatment for ZAP70 deficiency, and early transplantation is best.

Cheminant et al. (2023) conducted a retrospective analysis of outcomes in adults with IEIs who received allogeneic HSCT versus those who were not transplanted and received conservative management. A total of 281 patients were included (79 transplanted, 202 non-transplanted) with a median age of 21 years at the time of transplantation and median follow up of 4.8 years (interquartile range, 2.5-7.2). The 79 transplanted patients underwent allogeneic HSCT between 2008 and 2018 for IEIs such as chronic granulomatous disease (n = 20) and various combined immune deficiencies (n = 59). To control for variables as much as possible the non-transplanted patients were selected from the French Centre de Référence Déficits Immunitaires Héréditaires registry and matched for birth decade, age at last review greater than index patient age at allogeneic HSCT, chronic granulomatous disease or combined immune deficiencies, and autoimmune/lymphoproliferative complications. One-year transplant-related mortality rate was 13%, and the estimated 5 – year disease free survival rate of 58% and 33% in transplanted vs non-transplanted patients, respectively. Non-transplanted patients had increased recurrent events compared to transplanted patients, leading the authors to conclude that allogeneic HSCT prevents progressive morbidity associated with IEIs in adults, which may outweigh the negative impact of transplant-related mortality.

Miyamoto et al. (2022) conducted a retrospective analysis on HSCT for non-SCID inborn errors of immunity between the years 1985 – 2016 utilizing data from the Japanese national database, Transplant Registry Unified Management Program. A total of 566 underwent allogeneic HSCT during the 31-year analysis period. The 10-year OS and event-free survival (EFS) were 74% and 64%, respectively. Donor source of all HSCTs were 39% unrelated bone marrow

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donor, 33% sibling matched bone marrow donor, and 28% unrelated cord blood donor, with 10-year OS rates being 79%, 81%, and 69% respectively. The analysis revealed that the intensity of conditioning was not associated with OS or neutrophil recovery; however, myeloablative conditioning was more frequently associated with infection-related death. The authors did note that OS has increased in the most recent decade.

Marsh et al. (2018) conducted an analysis of the indications, practice changes, and survival of patients with inborn errors of immunity treated with allogeneic HSCT between 1974–2016 using data from the Center for International Blood and Marrow Transplant Research (CIBMTR). Due to the passage of the Stem Cell Therapeutic and Research Act of 2005 all allogeneic transplants in the United States are reported to the CIBMTR, thus the authors categorized transplantations by broad disease category (SCID vs non-SCID) and transplant periods: 1974–1989, 1990–1999, 2000–2009, and 2010–2016. A total of 981 SCID and 1902 non-SCID patients underwent allogeneic HCT between 1974–2016. The number of transplantations increased over time, culminating in 352 SCID and 816 non-SCID transplants performed between 2010-2016. The median age for transplantation was 0.5 years for SCID and 2 years for non-SCID patients, with both indications and pre-transplant regimens evolving over the decades. Overall survival rates increased across all diagnoses and ages resulting with 3 – year overall survival (OS) rates prior to 2000 being 64% for SCID (95% confidence interval [CI] 58–69%) and 62% for non-SCID (CI 57–67%), versus from 2000–2009 being 73% for SCID (CI 68–78%) and 70% for non-SCID , and from being 2010–2016 80% for SCID (CI 76–85%) and 75% for non-SCID (CI 72–79%) (p<0.001). SCID was added to newborn screening in 2010, and likely accounts for the superior increase in SCID survival after 2010, as those diagnosed by newborn screening experienced a 94% 3-year survival probability (CI 89–98%).

Inborn Errors of Metabolism

Systematic Reviews and Meta-Analyses

Abbasi et al. (2025) conducted a systematic review to evaluate the impact of HSCT, and especially bone marrow transplantation, on growth outcomes in pediatric patients with **mucopolysaccharidosis (MPS)**. Outcomes included height, weight, body mass index (BMI), head circumference, and pubertal development following HSCT. The review excluded single patient case reports. Quality assessment was performed using the Newcastle-Ottawa Scale for cohort studies and the Joanna Briggs Institute for case series. The review included patients across various MPS subtypes, but most notably MPS I, II, and VI, with sample sizes ranging from small case series to a multicenter cohort of 217 patients. Overall findings indicate that HSCT generally leads to improvements in height, weight, and BMI, particularly when performed at a younger age (ideally before 2 years of age). However, despite improvements, many patients continued to experience declining height z-scores over time and did not achieve normal adult stature. A decrease in height z-scores was typically more pronounced in patients treated at an older age, and some studies found associations between short stature and conditioning regimens such as total body irradiation. Sitting heigh was often disproportionately affected, contributing to short stature due to poor spinal growth. While growth hormone (rhGH) therapy in HSCT patients sometimes led to temporary improvements, it generally did not result in significant long-term gains in height z-scores or normalization of adult height. For BMI, HSCT was associated with initial improvement, and children with MPS I showed normalization of weight and BMI after HSCT particularly when treated early. However, some patients, especially those entering adolescence, developed elevated BMI levels. Macrocephaly was common prior to HSCT, particularly in MPS IH patients. HSCT helped to normalize head growth over time, and while some patients retained disproportionately large head circumferences, the degree of macrocephaly was generally reduced post-transplant. Most patients proceeded through puberty at normal ages, with bone age generally consistent with chronological age. Overall, the authors concluded that HSCT shows promise for improving growth outcomes, including height, weight, and BMI for patients with MPS; however, long-term growth challenges such as short stature, disproportionate weight gain, and incomplete resolution of macrocephaly persist. Long-term post-HSCT monitoring of growth and pubertal progression in patients with MPS is critical.

Non-Randomized Studies, Retrospective Reviews, and Other Evidence

Anna et al. (2026) conducted a retrospective review of six patients treated with HSCT for juvenile **globoid cell leukodystrophy (GLD)**. All six patients had a biochemical and genetically confirmed diagnosis of GLD. Baseline neurological status, disability (assessed via the modified Rankin Scale), cognitive status, galactocerebrosidase activity, MRIs and nerve conduction studies were collected as available, and then evaluated post-HSCT through last follow up. At diagnosis, two patients were asymptomatic and four were symptomatic. Two of the symptomatic patients had extensive pre-HSCT white matter disease and significant clinical manifestations (epilepsy and cognitive impairments). These two patients had sub-optimal outcomes post-HSCT despite galactocerebrosidase enzyme activity normalization,

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highlighting that appropriate standardized assessments of pre-HSCT disease progression are important for prognostic indicators. The four remaining patients achieved near-normal cognitive, motor, and functional status. Additionally, their Loes scores stabilized or improved, galactocerebrosidase enzyme activity normalized, and their electrophysiological measures (via evoked potentials and nerve conduction studies) remained stable. The authors concluded that HSCT in the absence of advanced disease results in optimal long-term outcomes for patients with juvenile GLD.

Li et al. (2026) conducted a retrospective review of thirty-one male children diagnosed with **X-linked adrenoleukodystrophy (ALD)** at a single institution between 2015 – 2023. Clinical features, adrenal function, brain MRI findings and associated Loes scores, and *ABCD1* mutations were analyzed for all patients. Based on MRI findings, 24 patients were diagnosed with cerebral ALD (cALD) phenotype. Those with a Loes score < 9 were classified as early-stage cALD and underwent HSCT. Their outcomes post-HSCT were compared to patients with more advanced cALD clinical manifestations (e.g., Loes score > 9, seizures, cognitive decline, visual impairment) who did not undergo HSCT due to disease progression. Though statistical significance was unable to be evaluated due to small samples sizes, those classified as early-stage cALD demonstrated a clinically meaningful trend toward improved 5-year overall survival (78%) compared to non-transplanted patients (29%). The authors concluded that early treatment HSCT prior to disease progression displays survival advantages.

Xu et al. (2025) conducted a longitudinal assessment of 57 children with **MPS** who received HSCT between the years 2019-2024. The follow up period was 24 months post HSCT, and the MPS subtypes included were type I (n=20), type II (n=26), type III (n=1), type IV (n=3), type VI (n=5), and type VII (n=2). All patients were evaluated utilizing the Griffiths Development Scales-Chinese (GDS-C) to assess neurologic function at baseline and at 3 months, 12 months, and 24 months post – HSCT. All patients were enzyme replacement therapy naïve and received a standard conditioning regime prior to HSCT. Overall significant post-HSCT improvements in locomotor ($F = 111.57$, $p < 0.001$), language ($F = 12.27$, $p = 0.001$), and eye-hand coordination ($F = 11.77$, $p = 0.001$) were observed across all subtypes. However, all domains exhibited gradual decline over 24 months post-HSCT, which suggests that a single HSCT intervention may not fully arrest secondary CNS degeneration in MPS patients. Age at transplantation was a critical determinant of positive outcomes, as younger recipients (≤ 4 years) exhibited preserved locomotor (32.7% higher scores, $p = 0.003$) and personal-social functions compared to their older counterparts who faced accelerated functional decline, possibly reflecting irreversible oligodendrocyte maturation deficits. The authors concluded HSCT provides meaningful clinical improvement in patients with MPS. The study was limited by small sample sizes [MPS III (n = 1), IV (n = 3), and VII (n = 2) subtypes] which limits generalizability, cerebrospinal fluid GAG levels were not quantified to correlate with functional scores, and the exclusion of enzyme replacement therapy -treated patients limits generalizability to combined-modality cohorts.

Qu et al. (2022) completed a single-center study of 42 children with **MPS** who received HSCT and a follow-up of ≥ 1 year following transplant. Children included in the study had MPS type I (n=9), type II (n=14), type IV (n=15), or type VI (n=4) and received either peripheral blood stem cells (n=24) or umbilical cord stem cells (n=18). Those that received umbilical cord stem cells received the blood from a matched family fresh cord blood donor (n=1), a mismatched unrelated cord blood donor (7/10 to 9/10 HLA-matched [n=15]), or from a double-mismatched unrelated cord blood donor (1 from a 6/10 to 8/10 HLA-matched donor and 1 from a 6/10 to 7/10 HLA-matched donor). Those that received peripheral blood stem cells received the blood from a matched family donor (n=4), a matched unrelated donor (n=10), a mismatched unrelated donor (7/10 to 9/10 HLA-matched [n=4]), or from a haploid donor (n=6). Children receiving peripheral blood stem cell transplants were condition with intravenous busulfan every 6 hours for 16 doses, intravenous cyclophosphamide, and antihuman thymocyte globulin. Specific lysosomal enzyme levels returned to normal in all recipients and 95.2% achieved full chimerism. The estimated OS at 1-year was 92.9% with no significant difference between peripheral or umbilical cord blood transplants. There was also no significant difference in acute or chronic GVHD between either group. High rates of pneumonia were noted in both groups (45.8% for peripheral blood and 33.3% for umbilical blood). A total of 3 deaths were reported following peripheral blood HSCT, 1 each due to grade III and IV GVHD, thrombotic microangiopathy, and combined grade III and IV GVHD and thrombotic microangiopathy. No patients with MPS type IV or VI died following transplantation. Researchers noted improvement in respiratory and CNS functions following HSCT. Valvular heart disease improved in some patients but progressed in others.
Gentner et al. (2021) reported interim results on an ongoing phase 1-2, non-randomized, single-center study involving 8 children diagnosed with **MPS** type I who lacked a suitable allogeneic donor for HSCT. The participants received autologous HSCT, and progenitor cells transduced ex vivo with an alpha-L-iduronidase (IDUA) encoding lentiviral vector after myeloablative conditioning. Median age at time of autologous HSCT was 1.9±0.5 years. Primary safety end points

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of the study include overall survival, hematologic engraftment by day 45, short- and long-term safety of drug-product infusion, and adverse event monitoring. The primary efficacy end point is blood IDUA activity at 1-year post-treatment. Secondary efficacy end points include anti-IDUA antibody immune response and engraftment of transduced cells at levels of 30% or more, normalization of GAGs, and growth velocity at 1- and 3-years post-treatment. The study has an expected 5-year duration and is currently at a median follow-up period of 2.1 years. Patients will continue to be followed for at least 15 years. Interim results reported showed hematologic engraftment that was rapid and consistent in all patients. Neutrophil recovery occurred at a median of 20 days and early spontaneous platelet recovery occurred at a median of 14 days. There were no reports of graft-versus-host disease due to the autologous nature of transplantation. A total of 19 serious adverse events were reported with only 1 of those (an acute allergic reaction) potentially related to the treatment. Previously undetectable levels of IDUA in the cerebrospinal fluid at baseline were detectable starting at 3-months post-treatment and persisting through each subsequent follow-up. GAG levels in the cerebrospinal fluid were also noted to decline. The IDUA and GAG results suggest a rapid and profound metabolic correction of the central nervous system. All patients also progressively acquired motor skills with 6 patients having a total motor performance within normal range or in the low average of normal. Other typical clinical manifestations, such as coarse facial features, upper airway obstruction, hearing loss, and corneal opacity, which were evident at the time of treatment showed improvement or stabilization at 1- and 2-year follow-ups.

Raymond et al. (2019) conducted an international, multicenter, retrospective chart review of 137 patients with cerebral **X-linked adrenoleukodystrophy** (cALD). Sixty-five patients underwent HSCT and their outcomes were compared to 72 patients who did not receive HSCT treatment. For the untreated cohort, the Kaplan Meier-estimated probabilities of overall survival at 2 and 5years from diagnosis were 74% (95% CI, 62.5%–83.0%) and 55% (95% CI, 42.2%–65.7%), respectively; versus the HSCT treated cohort with a Kaplan Meier-estimated probabilities of overall survival at 2 and 5 years post-HSCT (n = 65) were 82% (95% CI, 69.8% to 89.1%) and 74% (95% CI, 59.3% to 83.6%). Due to the short time between diagnosis and HSCT treatment, the ages of patients were the same at diagnosis as they were at the time of HSCT treatment, making the overall survival comparison possible. The Kaplan Meier-estimated major functional disabilities-free survival rate at 2 years from diagnosis was 48% (95% CI, 36.3%–59.6%) for the untreated cohort versus 56% (95% CI, 41.8% to 67.3%) at 2 years for the HSCT treated cohort. The disease progression stage at the time of diagnosis impacted the major functional disabilities-free survival rate in the HSCT treated cohort, with those classified as early-stage having a major functional disabilities-free survival rate at 2 years of 91% (95% CI, 73.5% to 96.9%). Early-stage disease was defined as a Neurologic Function Score ≤1 and a Loes score < 9. The authors highlighted the greatest advantage of HSCT is in early stage cALD, which emphasizes the importance of early diagnosis.

Groeschel et al. (2016) conducted a retrospective case control study to evaluate the long-term outcome for 24 patients with juvenile **metachromatic leukodystrophy (MLD)** after allogeneic HSCT compared to 41 patients untreated with HSCT. Primary outcome measurements were overall survival, loss of gross motor function assessed via the Gross Motor Function Classification in MLD (GMFC-MLD), loss of any language function, and MRI severity score for cerebral changes. Due to infection and graft rejection, the 5-year survival rate was 79% (5 of 24) in the HSCT treated group versus the untreated cohort 5-year survival post-diagnosis rate of 100% (41 of 41). However, 11 (27%) died of MLD progression, so while the overall mortality did not differ between the transplanted and nontransplanted patients, fatal disease progression occurred significantly less after HSCT (8% [2 of 24] vs 27% [11 of 41]) in a comparable observation period. Ten years after disease onset, 40% (8 of 20) of the HSCT treated patients versus 68% (28 of 41) of the untreated cohort had progressed to GMFC-MLD level 5, and language loss was observed in 40% (8 of 20) of the HSCT treated patients versus 68% (28 of 41) of the untreated cohort. The HSCT treated patients had lower MRI severity scores than the untreated cohort at the last MRI session post-transplant. Additionally, the untreated cohort had a significant increase in their MRI severity scores from their early to late disease stage ($P < .001$), whereas no significant increase was found in the HSCT treated patients ($P = .12$). Factors that correlated to better prognosis were patients who underwent HSCT at GMFC-MLD levels 0 and 1 ($P = .02$), patients with an IQ of at least 85 ($P = .02$), and patients with an age at onset older than 4 years ($P = .01$).

Aldenhoven et al. (2015) evaluated the survival and graft outcomes of **MPS** patients receiving HSCT. Two consecutive conditioning regimens were used, busulfan/cyclophosphamide or fludarabine/busulfan-based, both with exposure-targeted intravenous busulfan. A noncarrier matched sibling donor (MSD), matched unrelated cord blood (UCB), or matched unrelated donor (MUD) were preferred donors; however, if none were available, a mismatched UCB donor was used. A total of 62 MPS patients (56 MPS type I-Hurler, 2 MPS type II, 2 MPS type III, and 2 MPS type VI) received HSCT (41 UCB, 17 MSD, 4 MUD) at median age of 13.5 months. High overall survival (95.2%) and event-free survival

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(90.3%) were achieved with only low toxicity: 13.3% acute graft-versus-host disease aGVHD) grades II to IV and 14.8% chronic GVHD (1.9% extensive). A mismatched donor predicted a lower event-free survival (P = .04). A higher age at HSCT was a predictor for both aGVHD (P = .001) and chronic GVHD (P = .01). The use of a mismatched donor was a predictor for aGVHD (P = .01). Higher rates of full donor chimerism were achieved in successfully transplanted UCB recipients compared with MSD/MUD (P = .002). HSCT in MPS patients results in high safety and efficacy when international HSCT guidelines are followed, which allows extension of HSCT to more attenuated MPS types. Due to a reduction in HSCT related toxicity being associated with younger age at transplantation, newborn screening may further increase safety.

Martin et al. (2013) conducted a retrospective chart review to evaluate the neurologic outcomes post-HSCT of twenty-seven patients diagnosed with **metachromatic leukodystrophy (MLD).** The HSCT was conducted utilizing cord blood from public banks in the United States. Ten patients were diagnosed with late-infantile-onset MLD, with four being asymptomatic; and 17 were diagnosed with juvenile-onset MLD, with four being asymptomatic. The long-term (5-year) survival probability was 74.1% (95% CI, 53.2% to 86.7%) and varied by diagnosis group. The late-infantile group had a 5-year survival probability of 60.0% (95% CI, 25.3% to 82.7%), versus the juvenile group with a 5-year survival probability of 82.4% (95% CI, 54.7% to 93.9%). Four late-infantile patients died post-transplant, with significant disease progression noted in the remaining six patients. Four juvenile onset patients died post-transplant, with significant disease progression noted in three patients. MRI scans from 24 patients pre-transplant and 21 patients post-transplant were analyzed to reveal the pre-transplant modified Loes scores demonstrated baseline MRI abnormalities for all children except two asymptomatic patients diagnosed based on family history. Median Loes scores were 14.5 for late-infantile MLD and 5.5 for juvenile MLD, indicating more severe central nervous system impairment for patients with late-infantile onset. Of the 19 children who had both pre- and post-transplant modified Loes scores, 16 showed improvement post-transplant. The lower the pre-transplant Loes score, the greater developmental gains in both cognitive skills (late infantile, r = −.72; P = .10; juvenile, r = −.68; P = .022) and gross motor skills (late infantile, r = −.84; P = .037; juvenile, r = −.88; P = <.001).

Two case reports support an increased quality of life following HSCT in patients with **MPS** VII Sly Syndrome. Yamada (1998) reported on a 12-year-old female who underwent successful HSCT and had almost normal levels of beta-glucuronidase activity in her lymphocytes and excretion of glycosaminoglycans in the urine was greatly diminished in the 31 months post HSCT. In addition to ultrastructural findings demonstrating no abnormal vacuoles and inclusion bodies in the cytoplasm of her rectal mucosal cells, the patient was able to walk alone for a long time without aid, ride a bicycle, and take a bath independently. The patient's recurrent upper respiratory and middle ear infections decreased in frequency and severity, as well as a decrease in the patient's dyspnea on exertion, severe snoring, and vertigo. Orii (2020) reported the 22 year follow up of the patient and revealed that her β-glucuronidase activity in leukocytes remained at normal levels, and urinary glycosaminoglycan excretion was reduced and kept within normal levels. Presently the patient remains able to walk independently and climb stairs, as well as sustain simple conversation. Due to the long-term stabilization of this patient's clinical picture, HSCT for MPS VII is a viable therapeutic option.

National and Specialty Organizations

The **American Academy of Allergy, Asthma & Immunology (AAAAI), the American College of Allergy, Asthma & Immunology (ACAAI), and the Joint Council of Allergy, Asthma & Immunology** published the *Practice Parameter on Diagnosis and Management of Primary Immunodeficiency* to provide consultant allergists/immunologists or other practitioners 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 PID diseases. The guidelines also note that patients with SCID are fragile and extremely susceptible to infection. Definitive HSCT should be sought as quickly as possible, as early HSCT is associated with better outcomes, whereas complications before HSCT indicate a poorer prognosis. HSCT may be a therapeutic indication for combined immunodeficiencies (e.g., SCID [IL2RG, ADA, other forms], CD40L), immunodeficiency syndromes (e.g., Wiskott-Aldrich Syndrome), immune dysregulation disorders (e.g., familial hemophagocytic lymphohistiocytosis, autoimmune lymphoproliferative syndrome, IPEX syndrome), phagocytic cell defects (e.g., neutropenia, chronic granulomatous disease, leukocyte adhesion deficiency), innate immune defects, (e.g., NEMO deficiency and other NF-kB defects). HSCT is not indicated for autoinflammatory disorders, complement deficiencies, antibody deficiencies, or cytokine autoantibody-mediated disorders (Bonilla et al. 2015).

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The **American Society for Transplantation and Cellular Therapy (ASTCT**) published *Indications for Hematopoietic Cell Transplantation and Immune Effector Cell Therapy: Guidelines from the American Society for Transplantation and Cellular Therapy* which stipulates the various indications and uses for autologous and allogenic HSCT for malignant and non-malignant diseases (Kanate et al. 2020).

For pediatric patients, allogeneic HSCT is classified as standard of care for severe aplastic anemia, thalassemia, and hemophagocytic disorders, supported by high-quality evidence. Additionally, allogeneic HSCT is considered standard of care for Fanconi anemia, congenital amegakaryocytic thrombocytopenia, SCID and SCID, variants, Wiskott-Aldrich syndrome, IPEX syndrome, severe congenital neutropenia, chronic granulomatous disease, other phagocytic cell disorders, and other autoimmune and immune dysregulation disorders (e.g., mucopolysaccharidosis I, severe recessive osteopetrosis, globoid cell leukodystrophy, metachromatic leukodystrophy, and cerebral X-linked adrenoleukodystrophy); however, as these disease are rare, supporting high-quality evidence is not feasible and therefore treatment must be evaluated on a case-by-case basis with careful evaluation of risks and benefits. Other mucopolysaccharidoses (II, IV, VI) are considered developmental indications where preclinical and/or early-phase clinical studies show HSCT to be a promising treatment option and may be reclassified as standard of care as more evidence comes available (Kanate et al. 2020).

For adults, allogeneic HSCT is classified as standard of care for severe aplastic anemia (both newly diagnosed and refractory), sickle cell disease, and refractory hemophagocytic syndromes, supported by high-quality evidence. For Wiskott-Aldrich syndrome, HSCT is classified as standard of care with sufficient clinical evidence available to support its use after careful evaluation of risks and benefits. Additionally, allogeneic HSCT is considered standard of care for Fanconi anemia, dyskeratosis congenita, common variable immunodeficiency, chronic granulomatous disease, cerebral X-linked adrenoleukodystrophy, and other inherited marrow failure syndromes; however, supporting high-quality evidence is not feasible and therefore treatment must be evaluated on a case-by-case basis with careful evaluation of risks and benefits. Several autoimmune and metabolic disorders, such as multiple sclerosis, systemic sclerosis, rheumatoid arthritis, lupus, Crohn's disease, and osteopetrosis are classified as developmental indications (Kanate et al. 2020).

The ASTCT guidelines also emphasize that for many rare, nonmalignant diseases, the appropriateness of allogeneic HSCT depends on disease severity, progression, and patient-specific factors. Since prospective trials are lacking in such cases, recommendations are based on expert opinion and small studies. Individualized risk-benefit discussions, referral to experienced centers, and consideration for clinical trial enrollment are encouraged. Donor and graft source selection is also critical. Although HLA-matched sibling donors are preferred, excellent outcomes have been achieved with matched unrelated donors, umbilical cord blood, and haploidentical donors (Kanate et al. 2020).

The **Center for International Blood and Marrow Transplant Research (CIBMTR)** (2024), in the *Forms Instruction Manual*, states the following:

- *Comprehensive Baseline & Follow-up Manuals: 2100: Post-Infusion Follow-Up (Q10-16):*
    - Engraftment is defined as an absolute neutrophil count (ANC) ≥ 0.5 x 109/L (or ≥ 500/mm3) for three consecutive days or three consecutive laboratory values obtained on different days.
    - Absolute neutrophil recovery (ANC) recovery is defined as an ANC of ≥ 500/mm^3 (or ≥ 0.5 × 109/L) for three consecutive laboratory values obtained on different days. Date of ANC recovery is the date of the first of three consecutive laboratory values where the ANC is ≥ 500/mm^3. At some institutions, the laboratory reports display the ANC value once there are sufficient white blood cells to perform a differential count. At other institutions, the laboratory reports do not display the ANC, and it must be calculated from the white blood cell count and the percent of segmented and band neutrophils. If the laboratory report displays an automated ANC value of exactly 500/mm^3, the actual ANC value should be calculated from the manual differential if available. The calculated value from the manual differential will determine ANC recovery.
    - Traditionally, the definition of ANC recovery required selecting the first date of three consecutive days in which the recipient's ANC was ≥ 0.5×10^9/L (500/mm^3). For various reasons it may not be possible to obtain daily laboratory values. Under those circumstances, report ANC recovery based upon three consecutive laboratory values (drawn more than a day apart) as long as the ANC remains ≥ 0.5×10^9/L (500/mm^3).

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The **CIBMTR** is a research collaboration between the **Medical College of Wisconsin** and the **National Marrow Donor Program** (NMDP). In an NMDP *IND Annual Report (BB-IND #7555-0136)* for *A Centralized Cord Blood Registry to Facilitate Allogeneic, Unrelated Donor Umbilical Cord Blood Transplantation,* neutrophil engraftment is defined as achievement of an ANC of ≥ 500 neutrophils/mm^3 sustained for three consecutive laboratory measurements on different days (NMDP 2022).

The **European Society for Blood and Marrow Transplantation (EBMT)** published *Indications for haematopoietic cell transplantation and CAR-T for haematological diseases, solid tumours and immune disorders: 2025 EBMT practice recommendations* (Greco et al. 2025) outlining current indications for HSCT. The guidelines state that allogenic HSCT is the standard of care in children and adolescents diagnosed with an inborn error of immunity, except for those with an absent thymus, as HSCT will not replace thymic tissue. HSCT is indicated as a standard of care in MPS-IH (Hurler Syndrome), metachromatic leukodystrophy, and X-linked adrenoleukodystrophy. HSCT is listed as a clinical option in other inborn errors of metabolism such as MPS II-VII and Wolman disease.

The **National Marrow Donor Program (NMDP)** provides evidence-based pre- and post-HSCT guidelines to patients and providers, including: *Hematopoietic cell transplant (HCT) consultation guidelines & outcomes*, *Understanding engraftment*, *Post-transplant care and early HCT complications* ([1-3]NMDP date unknown).

SUPPLEMENTAL INFORMATION

Type of Inborn Error of Immunity	Examples
Absent, severely diminished, or dysregulated T-cell function	• Severe combined immunodeficiency disease (SCID) • Leaky SCID • Other combined immunodeficiencies [e.g., CD40L deficiency, DOCK8 deficiency, MHC class II deficiency, ZAP-70 deficiency] • Immune dysregulation, polyendocrinopathy, enteropathy, X-linked (IPEX) • Wiskott-Aldrich Syndrome • Omenn Syndrome • CTLA4 pathway defects including LRBA deficiency
Absent, severely diminished, or dysregulated natural killer cell function	• Hemophagocytic Lymphohistiocytosis (HLH) • Chediak-Higashi syndrome • Griscelli syndrome • GATA2 deficiency • X-linked lymphoproliferative disease (XLP) type 2 • Classical Natural Killer Cell Deficiency (cNKD) • Functional Natural Killer Cell Deficiency (fNKD)
Absent, severely diminished, or dysregulated neutrophil function	• Primary granulocyte dysfunction • Chronic granulomatous disease • Omenn Syndrome • Leukocyte adhesion deficiency • Severe congenital neutropenia (Kostmann syndrome) • GATA2 deficiency

CODING & BILLING INFORMATION

CPT (Current Procedural Terminology)

Code	Description
	Collection Codes
38205	Blood-derived hematopoietic progenitor cell harvesting for transplantation, per collection; allogeneic

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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
	Histocompatibility codes
86812	HLA typing; A, B, or C (e.g., A10, B7, B27), single antigen
86813	HLA typing; A, B, or C, multiple antigens
86816	HLA typing; DR/DQ, single antigen
86817	HLA typing; DR/DQ, multiple antigens

HCPCS (Healthcare Common Procedure Coding System)

Codes	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

06/10/2026	Policy revised. Expanded criteria to include more inborn error of metabolism diseases (XLD, MLD, GLD). IRO peer reviewed on June 6, 2026 by a practicing physician board certified in pediatric hematology/oncology.
06/11/2025	Policy revised. Removed numerical HLA matching criteria. Changed definition of engraftment and ANC criteria in coverage policy to align with current guidelines.
06/12/2024	New policy comprised of retired MCPs 265 (Immunodeficiency) and 256 (MPSs) into condensed HSCT for Non-Cancerous Diseases. Haploidentical transplant added to coverage criteria. IRO Peer Reviewed on May 24, 2024, by a practicing physician board certified in Pediatrics and Pediatric Hematology/Oncology.

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