APPENDIX A

Request Form for Board of Trustee Consideration of a Change to SHP Benefits

This form is to be used by individuals or groups that would like to propose new benefits coverage or request changes to benefits already covered by the State Health Plan. Please read the Procedure – Requests for Benefits Changes, SHP-PRO-7001-SHPfor more information regarding these types of requests.

Please submit completed forms by email to SHP.Board@nctreasurer.com or mail to NC State Health Plan Board of Trustees, 4901 Glenwood Avenue, Suite 300, Raleigh, NC 27612-3638.

Name of Requestor: Susan Elizabeth Sharf

Contact Information (*phone, email, mailing address):*

919-408-1809 / asharf@triad.rr.com / 3513 Bentridge Drive Mebane, NC 27302

Requested Change in Benefits Coverage: increase in donor search coverage for BMT patients

Reason for Request: current $10,000 maximum does not cover HLA typing costs for this need

Proposed Effective Date of Change: ASAP

Supporting Documentation (*Please provide documents to support your request; examples include research or studies regarding medical services, treatment or procedures, fiscal impact analyses if available, or petitions from members.*):

Would you like to speak with the Board of Trustees about this issue at a Board of Trustees meeting? yes/can arrange for Nat'l Marrow Donor Program personnel to speak as well

The Board of Trustees reviews select requests annually at a regularly scheduled Board of Trustee meeting. For calendar year 2013, requests will be reviewed at the November meeting. For calendar year 2014, requests will be reviewed at the July meeting. Review of requests in no way obligates the State Treasurer to make changes to benefits.

TO: NC Board of Trustees **DATE:** June 8, 2015

RE: Bone Marrow Transplant Donor Search Coverage Benefits

As a subscriber to the State Employee Health Plan, **I would like the Board to consider raising the $10,000 donor search coverage limit for bone marrow transplant recipients**. This is not a sufficient amount to cover the donor search costs for those candidates who are not fortunate enough to find a match within their own family.

To help explain my rationale for this request, I have attached several documents for review. In addition, I will outline my concerns with this benefit coverage as follows:

Being a donor match for a bone marrow transplant (BMT) recipient is different from those who need to receive a solid organ transplant (heart, kidney, lung, etc.). For solid organ transplants, 'matching' requires that both the donor and the recipient must have blood types that are compatible just like a blood transfusion. For BMT, however, there are markers on our white blood cells called Human Leukocyte Antigens (HLA) which must match to a certain degree in order for a donor to be considered. The better the "match grade" is, the better the chance that the BMT will be successful.

We all have only a 30% chance of finding a suitably matched donor amongst our full siblings (same mother and father) based upon the way our DNA is mapped. If a patient is not fortunate enough to find a match within their family or has no full siblings to type, the search turns to finding an unrelated donor within a registry such as The National Marrow Donor Program (NMDP). The success of finding an unrelated donor depends solely upon finding identical ethnicity within the HLA markers that are passed on from generation to generation in someone with whom you are not related. As an example, I have very common HLA markers for Caucasians from northern Europe; however, one HLA marker that has been passed along to me is of American Indian ethnicity. This would mean that the chances of my finding a fully matched unrelated donor decreases significantly unless someone who shares this exact combination of ethnicities on their HLA markers happens to be listed as a donor on a public registry. For those who have multiple ethnicities, the probability of finding a donor decreases which means a donor search may be more difficult, ultimately take longer and cumulatively cost more.

The current donor coverage that the State Health Plan offers *could* be adequate to cover search expenses if one were lucky enough to find a donor within their family. However, if an unrelated donor search needs to occur, this funding will *not* be sufficient - especially when there are multiple family members to type first which would exhaust the $10,000 benefit quickly. HLA typing can cost $2,500-$3,500 per donor to complete which would be enough to test only two to three siblings. My concern is that other insurance companies, such as BCBS, offer their subscribers unlimited donor search coverage and it seems that our North Carolina teachers, firefighters, policemen/women as well as all other state employees deserve similar benefits. Increasing the dollar amount available for this specific part of our coverage will not affect the majority of our State Health Plan members; however, this modification would be significant for those who must undergo a BMT. Families can become financially devastated as a result of undergoing a BMT and an approval to increase these benefits would certainly be helpful for those subscribers directly affected.

Thank you for your consideration and I appreciate your time in reading this proposal.

Very truly yours,

Susan Sharf 919-408-1809

Susan Sharf

NATIONAL MARROW DONOR PROGRAM® BE THE MATCH®

Benefit Design for HCT

Recommendations for designing an effective health insurance benefit set for hematopoietic cell transplantation (HCT)

Benefit Category	Recommendations
Allogeneic Donor Search Process	**Recommendation**: Full coverage of tissue typing of patient, potential related donors, and unrelated donors through Be The Match® or other approved registry. **Rationale**: 70% of patients do not have a fully matched sibling donor. Limiting or excluding search coverage delays transplant and can result in unnecessary and costly complications. Information about average costs and processes can be found at Payor.BeTheMatchClinical.org **Administrative Guidance:** Place search and procurement benefits in separate categories to ensure availability for each stage. Requiring proof of donor insurance policy denial for typing will unnecessarily delay the process; all policies prohibit coverage of costs when a member is acting as a donor. The Medicare claims processing manual indicates that donors should never be billed for transplant costs.
Cell Procurement or Acquisition	**Recommendation**: Full coverage of cell source acquisition and transport, including travel and lodging of related donor for harvest procedure. **Rationale**: Obtaining the cell source is a necessary part of the transplant process. For allogeneic unrelated HCT, cost of procurement is dependent on donor location and type of cells selected for transplant. **Administrative Guidance**: Place search and procurement benefits in separate categories to ensure availability for each stage.
Cell Infusion or Transplant	**Recommendation:** Full coverage of HCT and subsequent therapeutic infusions for all medically necessary indications, including full coverage of all relevant hospital stays. **Rationale**: HCT indications are expanding rapidly and improving the lives of patients with otherwise fatal conditions. Limiting access to HCT as a treatment option may result in increased costs and poor patient outcomes, including death. **Administrative Guidance:** HCT and the associated services fit within the definition of Essential Health Benefits as defined by the Department of Health and Human Services and therefore should not be subject to an annual dollar limitation. For information on transplant indications, visit CIBMTR.org. Limitation of bed days or hospital days on an annual basis is counterproductive to treatment and may be life-threatening. Several inpatient visits are needed for treatment of primary disease, preparation for transplant and recovery. Length of stay varies by disease, condition, cell or graft source success and complications. Utilization of a standard transplant authorization form can streamline requests and reduce processing time. A standard form can be found at Payor.BeTheMatchClinical.org
Medications	**Recommendation**: Full coverage, without co-pay or co-insurance, of all necessary medications throughout the HCT process, including the post-transplant period with access to in-person pharmacies not just mail order pharmacies. **Rationale**: Access to medication is critical for success of HCT. Prohibitive co-payments or co-insurance may result in non-compliance, poor outcomes, graft failure and/or expensive hospitals readmissions due to infection or complications. **Administrative Guidance**: Off-label use of medications is common for the treatment of cancer care of all types, including hematologic malignancies and HCT. Have health plan case management team review list of prescribed medications and work with the patients Pharmacy Benefit Manager (PBM) to issue a test claim prior to discharge.
Clinical Trials	**Recommendation**: Full coverage of routine care in clinical trials appropriate to the patient's disease, treatment stage and clinical condition. **Rationale:** Limiting access to clinical trials slows improvements in standards of care. Paying for identical care outside of a clinical trial has identical cost without gaining future benefit. **Administrative Guidance:** As of 2014, the Affordable Care Act requires coverage of all routine costs associated with clinical trials that meet sponsorship or approval requirements.

The recommendations in this guide were developed by a stakeholder group convened by the National Marrow Donor Program®, including: transplant physicians, representatives from national health insurance companies and transplant networks, and administrators from hospitals with HCT programs.

NATIONAL MARROW DONOR PROGRAM®

BE THE MATCH®

Unrelated Donor: Search Costs

Every year, thousands of people of all ages are diagnosed with leukemia and other life-threatening diseases. Many of them will die unless they get a bone marrow or cord blood transplant from a matching donor. Seventy percent of people do not have a donor in their family and depend on the Be The Match Registry®, operated by the National Marrow Donor Program® (NMDP), to find a match to save their life.

Search process

When a patient requires a transplant from an unrelated donor, a physician can request a free **preliminary search** of the Be The Match Registry to determine if there are potential matches.

To verify that potential donors or cord blood units match the patient, NMDP transplant center physicians can initiate a **formal search** to request further testing. A formal search includes a one-time activation fee plus additional costs for outreach and lab tests of potential donors and/or cord blood units.

Search costs

Performed by	Donor search activity	Typical cost range	Description
NMDP	Preliminary search	No cost	Returns a snapshot of potential matched unrelated donors and umbilical cord blood units
NMDP	Formal search activation fee	$1,100-$2,500	One-time fee that covers the initiation of a patient's formal search profile
NMDP	Donor management	$5,000-15,000	Includes donor outreach, high-resolution HLA testing, health history screening, infectious disease testing and collection of samples for use by transplant centers
Transplant center	HLA typing	Determined by transplant center	Additional HLA typing of donor samples must be completed by transplant centers

Donor management costs include high-resolution Human Leukocyte Antigen (HLA) typing requests, adult donor infectious disease testing and shipment of donor blood samples to a transplant center. These costs, however, **do not cover HLA typing that must be completed at the patient's transplant center.** Each patient's donor search is unique, and depending on the difficulty of the search, a transplant center may need to perform HLA typing on several potential donors, incurring costs for each. These costs vary among transplant centers.

LEARN MORE > Payor.BeTheMatchClinical.org

For information on costs, payor-focused education programs, transplant outcomes data, CPT coding help and much more, visit Payor.BeTheMatchClinical.org or contact NMDPPayorPolicy@nmdp.org

EXAMPLE >
Search Process

This is for illustrative purposes only; each transplant situation is unique.

An adult patient is referred to a transplant center with a life-threatening disease, such as acute lymphoblastic leukemia. Because the patient does not have a sibling match, the transplant center physician requests a free **preliminary search** of the Be The Match Registry, which identifies several potential matched unrelated adult donors and cord blood units.

The transplant physician activates a **formal search** by requesting specific high-resolution typing on a small number of adult donors who have a high likelihood of matching, as well as HLA typing on a few partially matched cord blood units. The transplant center is charged a one-time **formal search activation fee** and typing costs for those donors willing and medically eligible to proceed to donation.

After receiving high-resolution typing results, the patient's physician requests a few select donors to have fresh blood samples drawn for infectious disease testing and additional HLA testing at the transplant center. The transplant center is charged a **donor management fee** for donor health screening, drawing and shipping fresh blood samples, and performing infectious disease testing.

e p

Transplant Benefits & Coverage

Blood and marrow transplant (BMT) has become a standard of care for many blood cancer and genetic diseases as well as a newer treatment option for others. For many patients, BMT represents the best or only option for a cure. Timely transplant has led to significantly improved outcomes, so patients who need a transplant also need appropriate coverage in place to ensure there are not delays to treatment. Learn about key aspects of transplant and the benefits that will support patients through the process.

For more information, please see our Recommended Benefit Plan Design (PDF).

- See our recommended transplant benefits featured in the NCCN and NBGH's "An Employer's Guide to Cancer Treatment & Prevention"

Steps in a Search for an Unrelated Donor

Only 30% of patients will have a sibling who matches and is able to donate. The other 70%, or approximately 10,000 people per year, need an unrelated donor to donate their healthy marrow or to use a previously donated umbilical cord blood unit. The Be The Match Registry® can be searched for those patients who do not have a related donor. The search process includes:

- **Preliminary Search**: When a patient requires a transplant from an unrelated donor, a physician can request a free preliminary search of our Be The Match Registry to determine if there are potential matches. This search returns a snapshot of potential matched unrelated donors and umbilical cord blood units (CBUs).
- **Formal Search**: To verify that potential donors or cord blood units match the patient, transplant center physicians in our network can initiate a formal search to request further testing of potential donors or CBUs listed on our registry. A formal search includes a one-time activation fee plus additional costs for outreach and lab tests of potential donors and/or CBUs.
- **Donor Management and HLA Typing**: Our donor centers contact potential donors, set up appointments, and perform high-resolution HLA testing, health history screening, infectious disease testing and collect samples for use by transplant centers. Costs vary because of the number of donors that need to be tested to find an actual match. To understand more about this process and the associated costs, please see our Search Costs document (PDF).
- **Cell Procurement/Infusion**: The cost of procuring unrelated donor cells varies greatly depending on the cell type and transplant protocol. These costs may be as low as $30,000 or

higher than $60,000 in cases where a patient requires two simultaneous infusions of cells, such as a double cord blood transplant.

Costs also vary based on the location of the donor or cord blood unit that is the best match for the patient. We work with a number of registries across the world to have access to international donors. Each registry sets its own price for donor products. Cord blood unit prices vary by cord blood bank, as each sets its own fees. To learn more about this process, please see our Procurement Costs document (PDF).

Key Benefits for Supporting the Transplant Process:

There are several components to transplant that require specialized benefit support. Providing these benefits will greatly assist in achieving the best possible outcome for the patient.

- **Donor Search and Cell Acquisition**: The process for identifying a donor and acquiring the cells used for BMT is substantially different than the process used in solid organ transplantation. Patients need full coverage for HLA typing of themselves, their potential related donors and the potential donors on the Be The Match registry. They also need coverage for the cell source that is identified based on their particular clinical situation—marrow, PBSC or cord blood.
- **Inpatient Stays and Clinic Visits**: Patients receiving an unrelated donor transplant may stay in the hospital up to 100 days after cell infusion. They will also need a number of follow-up clinic visits and many of these may need to be at the hospital where they received their transplant, due to the specialization and training of the clinical teams.
- **Medications**: Access to medications is critical for success of BMT. Prohibitive co-payments or co-insurance on medications may result in non-compliance, poor outcomes, graft failure and/or expensive hospital readmissions due to infection or complications.
- **Clinical Trials**: The remarkable improvement in outcomes of HCT has been made possible because of clinical trials. Many patients who receive an HCT will be asked to join a clinical trial. The trials used in HCT do not mean that the medication or treatment is unproven or never before tested. Often the trial will test two standard options to determine which yield better results. Results of clinical trials improve care for all patients. Identical care outside of a trial has identical cost without gaining future benefit from trial outcomes.
- **Travel/Lodging**: Patients may need to travel during the transplant process for a variety of reasons—access to an in-network transplant center, access to a center that specializes in their disease condition, and/or follow-up care post-transplant with their original treatment team. The typical travel and lodging allows for up to $10,000 in travel related costs and follows IRS specifications in how the benefit can be provided.

Our Websites

National Marrow Donor Program —
Entrusted to operate the C.W. Bill Young Cell Transplantation Program, including Be The Match Registry®.

Biology of Blood and Marrow Transplantation

journal homepage: www.bbmt.org

ASBMT American Society for Blood and Marrow Transplantation

Opinion

Optimal Benefits for Hematopoietic Stem Cell Transplantation: A Consensus Opinion

Richard T. Maziarz [1,*,†], Stephanie Farnia [2], Patricia Martin [3], Krishna V. Komanduri [4]

[1] *Adult Blood and Marrow Transplant Program, Knight Cancer Institute, Oregon Health and Science University, Portland, Oregon*
[2] *National Marrow Donor Program, Minneapolis, Minnesota*
[3] *Anthem Blue Cross and Blue Shield*
[4] *Adult Stem Cell Transplant Program, University of Miami Sylvester Cancer Center, Miami, Florida*

Article history:
Received 18 April 2014
Accepted 7 July 2014

Key Words:
Hematopoietic stem cell transplantation
Consensus

A B S T R A C T

Variability in transplantation benefits may directly affect outcomes of individuals undergoing autologous or allogeneic hematopoietic stem cell transplantation procedures. The Financial Working Group of the National Marrow Donor Program–sponsored System Capacity Initiative addressed the issue of variable benefits and reviewed multiple transplantation benefit packages from both public and private payer organizations. On completion of the review, a consensus was obtained on defining a recipient benefit package that avoids major coverage gaps that could negatively influence patient outcomes. The recommendation was to encourage adoption of these benefits at a national level by payers, benefit brokers/consultants, and sales teams.

INTRODUCTION

Hematopoietic stem cell transplantation (HCT) remains the standard of care and often the only curative treatment option for a wide range of diseases, including high-risk and relapsed hematologic malignancies [1]. Currently, approximately 20,000 HCT procedures are performed in the United States each year [2–5]. HCT can be performed with either autologous (ie, the patient's own) or allogeneic (from a full or partially HLA-matched family member or unrelated donor) hematopoietic stem cells (HSC). The choice of the optimal HSC source is influenced by the nature of the underlying disorder, its responsiveness to chemotherapy, and its sensitivity to the immunologic effects mediated by an allogeneic donor graft. Medical considerations that may influence the decision to proceed to transplantation and the choice of HSC donor include disease stage and risk of relapse, patient age, and the presence of medical comorbidities. In addition, nonmedical reasons, including socioeconomic factors, such as the availability of a support network and access to financial resources, including payer availability, may influence the decision to perform HCT.

A recognized but understudied issue has been the impact of payer source on transplantation outcomes. In the United States, a multipayer system that includes state and federal governmental payers, as well as commercial ('third party') sources, exists. As the safety and efficacy of transplantation have improved over time for most diseases in which autologous and allogeneic HCT are used, transplantation has dramatically increased. Given the inevitable increases in costs associated with providing care for an increased number of transplantation patients, some payers have placed limitations on transplantation benefits, which may have unintended consequences for key clinical outcomes, including overall survival and quality of life. Studies have documented that HCT outcomes can be influenced by race and financial status, and analyses have suggested that the composition of a payer benefits package can positively or negatively affect outcomes [6]. As an example, it has been recognized that patients who are in need of allogeneic HCT often have benefit policies with inadequate "donor search" benefits—meaning coverage for the costs of finding and typing potential allogeneic donors. Clinical trial coverage varies by payer and may improve somewhat under the new requirements of the Affordable Care Act (ACA) implemented in 2014, but it is often a significant financial barrier, particularly in the case of emerging disease indications for HCT [7]. Finally, coverage for obtaining outpatient post-transplantation medications can be problematic for patients; substantial monthly expenses may be encountered because of high copays and coinsurance for specialized medications, with vast differences in coverage observed between individual self-funded

Financial disclosure: See Acknowledgments on page 1676.

* Correspondence and reprint requests: Richard T. Maziarz, MD, Knight Cancer Institute, OR Health & Science University, 3181 SW Sam Jackson Park Road, Portland, OR 97239-3098.
E-mail address: maziarzr@ohsu.edu (R.T. Maziarz).

† On behalf of the Financial Working Group of the National Marrow Donor Program System Capacity Initiative.

1083-8791/$ – see front matter © 2014 American Society for Blood and Marrow Transplantation.
http://dx.doi.org/10.1016/j.bbmt.2014.07.007

private payer plans and in benefits offered by governmental payers (eg, Medicare and state Medicaid plans).

THE NATIONAL MARROW DONOR PROGRAM SYSTEM CAPACITY INITIATIVE FINANCIAL WORKING GROUP

In September 2009, the National Marrow Donor Program (NMDP) organized the System Capacity Initiative (SCI), a 3-year project to assess the current health care system's ability to accommodate the predicted growth in the number of patients who will need an HCT by 2020. The SCI initiative addressed, through the formation of individual working groups, a wide range of HCT-related issues, including workforce availability, care delivery systems, education, access, and reimbursement [8,9]. As part of this initiative, a Financial Working Group (FWG) was assembled to identify and address financial barriers to transplantation. The FWG members represented a cross-section of the transplantation community, including transplantation medical directors, representatives of leading commercial payers, including medical and program directors responsible for payment for complex medical services, transplantation center administrators, and transplantation-specific risk management and contracting organizations leaders.

The initial efforts of the FWG were focused on identifying the scope of its activities, and, ultimately, in defining areas which the multidisciplinary FWG could provide guidance to the transplantation and payer communities. Under the auspices of the US Health Resources and Services Administration, an initiative to define a modern list of diseases appropriately treated with HCT, and for which coverage should be provided, was already underway and continues at present; therefore, it was felt that the group should support and not duplicate its efforts. Endorsement was provided for the need to create a catalogue of individual state Medicaid benefits, and this effort was individually pursued by the health services research division of the NMDP [10]. Ultimately, the entire committee decided to focus on 4 major issues, with the recognition that the effort could be completed within the 36-month period and yield working products that reflected a consensus opinion of the members of the diverse group. These projects included the following: (1) the creation of consensus guidelines that would define the appropriate benefit package for the HCT recipient, (2) the development of tools to enhance the efficiency of the preauthorization process for private payers, (3) the creation of materials and tools to educate transplantation centers on the complexity of coding in reimbursement, and (4) the generation of a plan to communicate these consensus opinions and tools for the broader HCT community, including transplantation medical directors, center administrators, leadership within groups of public and commercial payers, and the greater health care purchaser industry involved in transplantation benefits formulation and administration, including plan managers, benefit consultants, and reinsurers.

METHODS

Process of Benefits Analysis and Development of a Consensus Benefits Package

An FWG subcommittee was formed to define the key elements of a consensus benefit package. The first step was the confirmation and ascertainment of the need for a clear set of recipient benefits for patients undergoing allogeneic and autologous HCT, based on available clinical and administrative best practices. This deliverable was identified as a priority effort because of the readily discernible, wide variation in benefits packages known to the subcommittee members. The group acquired, and reviewed in detail, information regarding individual benefit packages from a wide range of commercial payers and the available benefits provided by various state Medicaid agencies and Medicare coverage standards. There was a consensus that many governmental payers, particularly state Medicaid plans, provided limited and often inadequate HCT benefits, an observation that led to an independent NMDP policy team analysis, which confirmed this view [9]. The group also recognized that there has been extensive growth in the number of self-funded plans that, although often administered by major commercial payers, were the ultimate arbiters of benefits provided to their own employees. There was also recognition that HCT-associated benefits may not be entirely defined by the primary payer, but that reinsurer groups can also be responsible for transplantation and other complex services carved out of the primary benefits package. Specifically, there was a focused effort to examine both benefits provided by entities that provide reinsurance coverage to an employer's self-insured benefit plan (the circumstance where the reinsurer does not define benefits under the employer's plan but rather establishes which benefits are covered under the reinsurance coverage) and a second group of payer entities that provide insurance (not reinsurance) coverage for transplantation benefits that have truly been carved out of the medical benefit set. In this latter circumstance, the entity is providing fully insured (not self-insured) coverage for a defined set of transplantation services that has been carved out—ie, excluded—under the employer's self-insured benefit plan, thus protecting the employer from the financial risk associated with variability in delivery of transplantation services.

As a next step, the working group documented benefits that were universally included within multiple plans. The group then generated a process map required by the transplant recipient, recognizing the high variability of clinical course, based upon the type of transplantation that was to be undertaken. With these steps completed, the group assessed frequent incongruities between benefit plans and also identified common gaps in coverage. The potential clinical consequences of coverage gaps were then discussed and evaluated, with consideration of the costs associated with coverage and the potential unintended consequences (clinical and financial) of benefit limitations. The final steps of the process were to create a document defining a recommended set of insurance benefits derived from clear consensus of all stakeholders and of sufficiently high visibility to encourage near-universal adoption by all payers, benefit brokers/consultants, and account sales teams.

RESULTS

Recommended Benefits for HCT

Benefits described are those that the committee felt provided appropriate support to a patient and his/her care team to maximize the likelihood of achieving optimal HCT outcomes (Table 1). Coverage for HCT and all subsequent therapeutic interventions, and support for travel and lodging, as well as for outpatient care and caregiver requirements, should be provided for any patient with a medically necessary indication and adequate physiologic reserve such that acceptable long-term outcomes could be achieved. Transplantation indications are expanding rapidly and it is recognized that HCT may be either a curative option or life-extending procedure for many patients. Limiting or delaying access to transplantation may result in increased costs and poor patient outcomes, including death. Financial limits for reimbursement of HCT costs, either for the procedure or for medical costs over a patient's lifetime, should not have predetermined restrictive ceilings. Determination of the diagnostic indications for HCT procedures was not felt to be the purview of the subcommittee, but rather, deferred to national organizations or payer bodies performing evidence-based assessments of the value of HCT compared with alternate strategies that are continually evolving.

Donor Search

In the case of an allogeneic HCT, coverage should be provided for HLA typing of the patient and potential donors to identify the best possible "match" or best available cellular product. Related donors will primarily include fully HLA-matched siblings but may also be extended to other family members, while recognizing that less than fully HLA-matched donors are acceptable in selected situations. Unrelated donor HCT procedures have been increasing dramatically over the

Table 1
Benefit Design for Hematopoietic Cell Transplantation: Recommendations for Designing an Effective Health Insurance Benefit Set

Benefit Category	Recommendations
Allogeneic donor search process	Recommendation: Full coverage of tissue typing of patient, potential related donors, and unrelated donors through Be The Match or other approved registry. Rationale: Seventy percent of patients do not have a fully matched sibling donor. Limiting or excluding search coverage delays transplantation and can result in unnecessary and costly complications. Information about average costs and processes can be found at http://payor.bethematchclinical.org. Administrative guidance: Place search and procurement benefits in separate categories to ensure availability for each stage. Requiring proof of donor insurance policy denial for typing will unnecessarily delay the process; all policies prohibit coverage of costs when a member is acting as a donor. The Medicare claims processing manual indicates that donors should never be billed for transplantation costs.
Cell procurement or acquisition	Recommendation: Full coverage of cell source acquisition and transport, including travel and lodging of related donor, for harvest procedure. Rationale: Obtaining the cell source is a necessary part of the transplantation process. For allogeneic unrelated HCT, cost of procurement is dependent on donor location and type of cells selected for transplantation. Administrative guidance: Place search and procurement benefits in separate categories to ensure availability for each stage.
Cell infusion or transplantation; hospital length of stay	Recommendation: Full coverage of HCT and subsequent therapeutic infusions for all medically necessary indications, including full coverage of all relevant hospital stays. Rationale: Transplantation indications are expanding rapidly and improving the lives of patients with otherwise fatal conditions. Limiting access to HCT as a treatment option may result in increased costs and poor patient outcomes, including death. Administrative guidance: HCT and the associated services fit within the definition of Essential Health Benefits as defined by the Department of Health and Human Services and, therefore, should not be subject to an annual dollar limitation. For information on transplantation indications, please visit www.CIBMTR.org. Limitation of bed days or hospital days on an annual basis is counterproductive to treatment and may be life-threatening. Several inpatient visits are needed for treatment of primary disease, preparation for transplant and recovery. Length of stay varies by disease, condition, cell or graft source success and complications. Utilization of a standard transplantation authorization form can streamline requests and reduce processing time. A standard form can be found at www.payor.bethematchclinical.org
Travel and lodging	Recommendation: Full coverage of travel and lodging costs for member and caregiver(s) for the transplantation visit, in addition to necessary pre- and post-transplantation evaluations. Cover costs for additional caregiver travel, if patient is under 18 years of age. Rationale: Patient will likely have to travel to a transplantation center able to treat their condition and/or within their insurance network. Allogeneic HCT programs may require patient to stay near center for up to 100 days after transplantation. Limiting travel/lodging benefits may result in complications caused by delayed care and/or patient seeking care from nonspecialist care teams. Administrative guidance: Encourage member to use discounted housing options if available through the transplantation program. Adopt IRS reimbursement guidelines for taxable amounts allowed for health-related travel or allow flexible spending of plan-determined patient allocation. Patient will need to report to IRS on 1099 form. Consider use of reusable debit card.
Medications	Recommendation: Full coverage, without copay or coinsurance, of all necessary medications throughout the HCT process, including the post-transplantation period. Rationale: Access to medication is critical for success of HCT. Prohibitive copayments or coinsurance may result in noncompliance, poor outcomes, graft failure, and/or expensive hospitals readmissions due to infection or complications. Administrative guidance: Off-label use of medications is common for the treatment of cancer care of all types, including hematologic malignancies and HCT. Have health plan case management team review list of prescribed medications and work with the patients pharmacy benefit manager to issue a test claim before discharge.
Clinical trials	Recommendation: Full coverage of routine care in clinical trials appropriate to the patient's disease, treatment stage, and clinical condition. Rationale: Limiting access to clinical trials slows improvements in standards of care. Paying for identical care outside of a clinical trial has identical cost without potential of future benefit. Administrative guidance: As of 2014, the ACA requires coverage of all routine costs associated with clinical trials that meet sponsorship or approval requirements.

IRS indicates Internal Revenue Service.

past decade [3,4]. Molecular HLA typing of identified potential unrelated adult donors and/or cord blood units should be covered when facilitated through Be The Match or another payer-approved donor registry, such as the Anthony Nolan Registry or the Delete Blood Cancer Deutsche Knochenmarkspenderdatei gGmbH (Translation: German Bone Marrow Donor Center). Potential unrelated donors have preliminary typing results available through the Be The Match registry but need additional and more detailed confirmatory testing before selection of the best donor. Limiting or excluding coverage for donor typing can result in a suboptimal donor choice, which may lead to increased rates of complications associated with increased morbidity and mortality, including graft-versus-host disease and graft rejection. As donation timelines may vary between individual donors, limitation of search services may negatively affect transplantation timing, possibly increasing the risk of disease progression before HCT or treatment failure after transplantation. Increases in complication rates and the corresponding consequences of these complications may increase overall costs. Coverage should be provided for the medical evaluation of the donor as well as the requisite laboratory screening needed to identify potential transmissible hematologic, autoimmune, or infectious diseases. Administrative recommendations for payer consideration were to place search and procurement funding into a separate benefits compartment to ensure funds would be available.

Cell Acquisition and Procurement

Coverage recommendations for cell acquisition vary by transplant and donor type. Autologous HCT patients need full coverage for preparation/mobilization, collection, cryopreservation, and storage of cells. Clarification of the onset of autologous product mobilization and collection is needed, recognizing the different approaches (and associated costs) resulting from strategies that commonly include mobilization after the administration of cytotoxic chemotherapy followed by growth factors, compared with the use of growth factors alone for mobilization of peripheral blood stem cells. Allogeneic HCT recipients need full coverage for donor clearance, preparation, mobilization, and cell collection, transportation, and delivery costs. This includes costs associated with

unrelated donor products, which may include single or double umbilical cord blood products, bone marrow products collected by operative harvesting, or peripheral blood stem cells products collected by apheresis after administration of growth factors to healthy donors. In some circumstances, there may need to be allowances for variable practice, including need for cryopreservation, thawing, and preparation of HSC, including enrichment and/or depletion of graft subsets, depending on the situation and donor source. When a fully or partially HLA-matched related donor is utilized, coverage for donor travel to and lodging at the patient's transplantation center should be provided, when necessary, in addition to the actual procurement. Administrative recommendations for payer consideration were to place search and procurement funding into a separate benefits compartment to ensure funds would be available.

Cell Infusion ("Transplantation") Procedures

Full coverage of the actual cell infusion procedure should be provided. Financial support for management of the primary hospitalization and long-term medical complications should be planned. Administrative guidance recommendations include placing all transplantation benefits under general medical benefits spending and/or not to implement a separate transplantation-only benefit and spending limit. This recommendation has been further clarified by the ACA, as transplantation procedures are within the scope of the Essential Health Benefit set and cannot be restricted by qualified health plans. There has been the steady adoption of transplantation benefits to cover a variably defined episode of care (ie, preparation, infusion, and a number of recovery days, usually 100), rather than what is the tightly temporally defined procedure. There is an emerging understanding that the primary transplantation HSC infusion is distinct from subsequent infusion episodes (eg, performed to treat graft failure and/or relapse) and that consistent terminology regarding associated practices is needed. Recent efforts by professional societies and payers have led to the development of consensus statements [11], and the FWG expressed support for further efforts to develop and maintain consistency of terminology used by various stakeholders in the HCT community.

Travel and Lodging

Full coverage is recommended for travel and lodging costs for a patient and his/her caregiver(s) for transplantation candidacy evaluation, preparation, and the procedure itself, in addition to post-transplantation follow-up visits. In the case of a pediatric or adolescent/young adult patient, coverage for a second caregiver and/or allowance for alternating caregivers is often needed and should be covered. Patients may be required to stay within close range of a transplantation center for several months after HCT, with longer intervals (up to several months) typically required in the setting of allogeneic transplantation. Limiting travel and lodging benefits may create financial barriers for patients pursuing transplantation as a treatment option and reduces their ability to seek appropriate follow-up care with their primary transplantation team, which may lead to suboptimal management of complications and increased risk and cost of complications. Payers can promote the use of discounted housing options offered by transplantation centers, particularly when relocating patients to an identified center of excellence within the transplantation network. Payers may choose to either adopt Internal Revenue Service guidelines for these benefits or allow flexible spending of an allowed amount and later issuing an Internal Revenue Service form 1099 to the patient. Consideration has been recommended for providing reloadable debit cards and for extension of travel and lodging benefits to support daily expenditures, such as food and local travel.

Hospital Care/Length of Stay

There should not be a limit placed on the number of inpatient days covered for an HCT patient during the course of a calendar year or subsequent years, as arbitrary limits could result in suboptimal management of early or late transplantation complications. The hospital stay for the HCT conditioning, infusion, and recovery periods can vary based on a variety of factors that govern transplantation risk (eg, patient clinical status, disease, graft type) and also the variable incidence of complications even within defined risk groups. Patients may also face inpatient stays for control of their malignancy before the transplantation process and multiple readmissions after transplantation for treatment of complications. The practice of setting arbitrary limits on hospital days was considered counterproductive to optimal treatment and may increase the risk of adverse outcomes with ultimately increased cost.

Clinical Trials

Coverage of clinical trial participation should be provided for trials appropriate to the patient's disease, stage, and clinical condition. Routine costs associated with clinical trials that are federally approved or sponsored (eg, HCT trials supported by the National Heart Lung Blood Institute (NHLBI) and National Cancer Institutes (NCI), including multicenter or single center studies performed at NCI-designated cancer centers) are required of most health insurance policies as of January 1, 2014, under the provisions of the ACA. However, coverage for well-designed clinical trials that have not secured federal funding should also be considered when recommended by a patient's care team, particularly for emerging transplantation indications. Well-designed, statistically sound, single institution, scientifically innovative trials, such as the recently published studies of chimeric antigen receptor-T cells in relapsed acute lymphoid leukemia from the University of Pennsylvania Abramson Cancer Center have played an important role in furthering the HCT field [12]. Limiting patient access only to multicenter, well-designed, nationally supported clinical trials has the risk of slowing improvement in standards of care that otherwise would continue to evolve at a high rate, given the rapid pace of scientific and clinical developments relevant to HCT. Paying for identical care outside of a clinical trial has identical cost without the collective societal benefit gained via clinical trials. HCT is an area of medicine with a high proportion of patients treated on clinical trials because of the complexity of the treatment, the variety of diseases treated, and the rapid evolution of best practices, including those efforts spearheaded by research consortiums that include the NHLBI- and NCI-sponsored Blood and Marrow Transplant Clinical Trials Network [13,14].

Prescription Medication

The HCT process is dependent on prescription medications, often required for years, that include antimicrobials agents, for prophylaxis and therapy, and immunosuppressive medications critical to the safety and success of allogeneic transplantation. Coverage of all necessary medications, particularly post-transplantation medications, should be provided, ideally with waived coinsurance or copay responsibilities. There was strong consensus that cost-sharing provisions intended to limit

unnecessary medication costs may be more likely, in the HCT setting, to result in noncompliance, leading to significant complications, including higher rates of graft-versus-host disease and/or infections, both of which are important causes of morbidity and mortality after HCT. Thus, noncompliance related to the financial burden of coinsurance or copay costs may result in poor outcomes and, ultimately, in expensive hospital readmissions. Off-label use of medications is commonplace in cancer treatment protocols and in supportive care of HCT patients, supported by a strong evidence base for multiple off-label medications used in HCT patients. A review of patients' medications between all stakeholders is recommended before discharge, as is a test claim of the medications to identify cost and/or coverage problems. A test claim is the "dummy" submission of the prescription claim from the hospital to the payer, which results in detailed information as to any potential copays, formulary issues, and denied medications.

DISCUSSION

The management of the HCT recipient, whether the patient has undergone an autologous or allogeneic procedure, is a complex process requiring extensive medical evaluation, the complex delivery of ambulatory and inpatient services, and a need for ongoing diagnostic clinical and laboratory evaluations. All of these efforts must be performed with ongoing awareness and attention to the underlying disease and associated medical comorbidities, with contextual clinical decision-making considering a variety of socioeconomic factors, such as patient education, caregiver support, and access to health care systems; all of these factors ultimately influence individual patient outcomes. Not surprisingly, the total costs of HCT will be significant and may be accrued over an extended period of time [15,16]. Total HCT episode costs are likely to continue to rise because of expanded utilization of HCT and improved survival after transplantation. The increasing costs of HCT must be considered in the context of rising general costs for the diseases most often indicated for transplantation, as leukemia and lymphoma have already been identified by the NCI within the top 6 cancer disease categories that result in the greatest annual cancer expenditure [17]. To maximize the possibility of achieving optimal outcomes, the workforce must be intact [18] and the financial support and clinical infrastructure needed to provide care to individuals undergoing intensive cancer therapies must be assured. These goals motivated the establishment of the NMDP SCI and its subcommittees, including the FWG, which identified a high-priority need to define the key elements of an effective financial benefits package for the transplantation patient and to subsequently facilitate understanding and adoption of these recommendations.

This manuscript has described the details of the recommended transplantation recipient benefit package, outlining the importance of subcategories that need to be considered. Historically, there has been a tendency to fragment transplantation benefits packages, with independent allocations for individual elements of care (eg, search, transplantation medical benefits, and general medical care). This compartmentalization may contribute to disjointed and often suboptimal care of the HCT patient. Dramatic variations in payer benefits packages may also limit the ability of transplantation centers to practice consistent and evidenced-based care or develop clear patient medical pathways, resulting in a need to deviate from uniform care standards as a result of restraints imposed by divergent benefits packages. The effort of the FWG to define a transplant recipient benefit package is an important first step toward improving the consistency of care and an iterative process, wherein outcomes are optimized while minimizing the costs of care to the individual patient and to the health care system. We recognize that adopting any perceived expansion of benefits requires a detailed cost analysis of the total episode of care to determine if additional costs to the system have been incurred but ideally, an optimal benefit package could contribute to outcome improvement in diminished complications through additional supportive care. We also recognize that there already exists significant variation in inpatient costs among HCT transplantation centers, as recently documented by the analysis of Thao et al. [19]. We also anticipate that there will be ongoing analysis during the expected evolution of care delivery as a product of expansion of the transplantation-eligible patient population that will be the result of the ACA; the 2014 implementation of key provisions of the ACA impact access to HCT in numerous ways and a separate and specific analysis has recently been published by the NMDP health policy team [20].

We expect these guidelines to be reviewed by transplantation centers and payers, yielding further discussion and action, and immediate consequences of this consensus effort are already evident. Using the recipient benefit package as a model for care, a review of Oregon Health and Science University's institutional requests for transplantation benefits was performed and in a 4-month time line, 50% of the requests for preauthorization failed to meet SCI benefit guidelines (Maziarz, unpublished data: Oral presentation—NMDP Blood and Marrow Transplant: A Forum on Quality, Transparency, Cost, and Value [July 2013]). Preussler et al. have reviewed the US Medicaid programs and have demonstrated that no state provides coverage in all benefit categories [10]. Three states had adequate benefits for 4 of the categories; 21 states had adequate coverage for 3 categories; 15 states had adequate coverage for only 2 of the categories, and 8 states, including 2 of the most populous states of the country, met the proposed benefits in only 1 transplantation benefit category. These data suggest that education and advocacy will be necessary to ensure improvement of benefits packages at the state level.

On a more positive note, as a result of the generation of the SCI recommendation for covered transplantation benefits, the National Comprehensive Cancer Network and the National Business Group on Health have integrated these benefit recommendations into their Employer's Guide to Cancer Treatment and Prevention [21]. The National Business Group on Health/National Comprehensive Cancer Network series provides reference tools specific to cancer care and treatment for employers who are purchasing health care benefits. They recommend that coverage include pre-transplantation, transplantation, and post-transplantation care recommended by the transplantation center and that the benefit plan also include donor search and typing costs including: "full cost of biological sibling typing; full cost of unrelated donor search, including typing and testing of potential donors, through the NMDP or other approved registry; full cost of related donor procurement, including travel and lodging of the selected related donor for the donation process; and full cost of donor cell product procurement for the unrelated donor" [21]. Ongoing outreach activities are planned, through the NMDP and affiliated organizations, to extend education about and adoption of these consensus recommendations.

CONCLUSION

HCT is an important but complex treatment modality and continues to be utilized in an expanding fashion because of improved safety and efficacy for a broad range of indications. Although expensive, HCT has also been demonstrated to be cost effective for many indications, and it is often the treatment modality most likely to be curative or extend life in transplantation candidates. For underinsured or uninsured transplantation patients, facing the complex process of care with limited or no health insurance benefits is daunting and is very likely to undermine the likelihood of success. Because the major component of payer cost is for the transplantation procedure and hospitalization, attempts to control costs for ancillary processes or procedures, supportive care of the patient, or medications may paradoxically increase care because of an unintended increased risk of complications. It is the hope of the working group that all patients undergoing HCT will be able to concentrate on their compliance, recovery, healing, and quality of life rather than the long-term financial implications of their treatment.

ACKNOWLEDGMENTS

The authors acknowledge the thoughtful contributions of the SCI Financial Working Group members: Bonnie Anderson, Peggy Appel, Michael Boo, Ruth Brentari, Richard Champlin, Allen Chernov, James Coates, Mary Foote, Dennis Irwin, Navneet Majhail, Philip McCarthy, Michael Rabin, Deborah Rodriguez, and Debbie Stenhjem, as well as current and former members of the NMDP staff: Debbie Jacobson, Joan Jarosh and Aaron Schnell. The authors are also grateful for the vision of Drs. Edward Snyder and Jeffrey Chell for the establishment and maintenance of the NMDP System Capacity Initiative.

Financial disclosure: The authors have nothing to disclose.

REFERENCES

1. Copelan E. Hematopoietic stem-cell transplantation. *N Engl J Med.* 2006;354:1813-1826.
2. Pasquini MC, Wang Z, Horowitz MM, Gale RP. 2010 eport from the Center for International Blood and Marrow Transplant Research (CIBMTR): current uses and outcomes of hematopoietic cell transplants for blood and marrow disorders. *Clin Transpl.* 2010:87-105.
3. Pasquini MC, Wang Z. Current use and outcome of hematopoietic stem cell transplantation: CIBMTR summary slides. 2013. Available at, http://www.cibmtr.org.
4. Hahn T, McCarthy PL, Hassebroek A, et al. Significant improvement in survival after allogeneic hematopoietic cell transplantation in North America. *J Clin Oncol.* 2013;31:2437-2449.
5. McCarthy PL, Hahn T, Hassebroek AM, et al. Significant improvement in survival after autologous hematopoietic cell transplantation in North America. *Biol Blood Marrow Transplant.* 2013;19:1116-1123.
6. McHenry M, Hayes-Lattin B, Maziarz RT. Impact of health insurance on time to approval and time to transplant among patients receiving hematopoietic stem cell transplantation. *Biol Blood Marrow Transplant.* 2006;14(Suppl. 2):146.
7. Kircher SM, Benson AB 3rd, Farber M, Nimeiri HS. Effect of the accountable care act of 2010 on clinical trial insurance coverage. *J Clin Oncol.* 2012;30:548-553.
8. Majhail NS, Murphy EA, Denzen E, et al. The National Marrow Donor Program's symposium on hematopoietic cell transplantation in 2020: a health care resource and infrastructure assessment. *Biol Blood Marrow Transplant.* 2012;18:172-182.
9. Denzen EM, Majhail NS, Ferguson SS, et al. Hematopoietic cell transplantation in 2020: summary of year 2 recommendations of the National Marrow Donor Program's system capacity initiative. *Biol Blood Marrow Transplant.* 2013;9:4-11.
10. Preussler JM, Farnia SH, Denzen EM, Majhail N. Variation in Medicaid coverage for hematopoietic cell transplantation. *J Oncol Pract.* 2014;10:e196-200.
11. LeMaistre CF, Farnia S, Crawford S, et al. Standardization of terminology for episodes of hematopoietic stem cell patient transplant care. *Biol Blood Marrow Transplant.* 2013;19:851-857.
12. Grupp SA, Kalos M, Barrett D, et al. Chimeric antigen receptor-modified T cells for acute lymphoid leukemia. *N Engl J Med.* 2013;368:1509-1518.
13. Weisdorf D, Carter S, Confer D, et al. Blood and marrow transplant clinical trials network (BMT CTN): Addressing unanswered questions. *Biol Blood Marrow Transplant.* 2007;1393:257-262.
14. Ferrara J. Blood and Marrow Transplant Clinical Trials Network: progress since the state of the science symposium. *Biol Blood Marrow Transplant.* 2007;20:149-153.
15. Stranges E, Russo CA, Friedman B. *Procedures with the most rapidly increasing hospital costs, 2004-2007. HCUP Statistical Brief #82.* Rockville, MD: Agency for Healthcare Research and Quality. Available at, http://www.hcup-us.ahrq.gov/reports/statbriefs/sb82.pdf; December 2009. Accessed: May 7, 2013.
16. Bentley TS, Hanson SG. 2011 U.S. organ and tissue transplant cost estimates and discussion. Milliman Research Report 2011:1–20.
17. Mariotto AB, Yabroff KR, Shao Y, et al. Projections of the cost of cancer care in the United States: 2010-2020. *J Natl Cancer Inst.* 2011;103:117-128.
18. Gajewski JL, LeMaistre CF, Silver SM, et al. Impending challenges in the hematopoietic stem cell transplant physician workforce. *Biol Blood Marrow Transplant.* 2009;15:1493-1501.
19. Thao V, Kozhimannil KB, Thomas W, Golberstein E. Variation in inpatient costs of hematopoietic cell transplantation among transplant centers in the United States. *JAMA Intern Med.* 2014 Aug 1;174(8):1409-1412.
20. Farnia S, Gedan A, Boo M. Impact of the Affordable Care Act on stem cell transplantation. *Curr Hematol Malig Rep.* 2014;9(1):66-72.
21. Danielson E, Finch R, Kendall D, et al. *Tool 2: plan design and assessment tool.* National Business Group on Health; National Comprehensive Cancer Network. Available at, http://www.businessgrouphealth.org/resources/topics/cancer.cfm; 2013.