

■ Article

Patient and Family Resources for Living With Myelodysplastic Syndromes

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Primarily a disease affecting older adults, myelodysplastic syndromes (MDS) are a class of incurable myeloid malignancies with variable clinical presentation, treatment recommendations, and prognoses. Although effective communication between healthcare professionals and patients and their caregivers is a significant part of optimizing clinical outcomes, studies have shown that all three frequently have an incomplete understanding of MDS, its therapeutic options, and the fact that MDS is a malignancy. In addition, the advanced age of the patient population, high frequency of comorbidities, and variability of disease outcomes based on risk status require consistent communication across a wide number and type of healthcare providers as well as an individualized approach to patient and caregiver education. This article discusses these challenges and provides a number of resources designed to help educate healthcare professionals, patients, and caregivers.

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Myelodysplastic syndromes (MDS) represent a heterogeneous group of myeloid malignancies with a peak incidence in the seventh and eighth decades of life. The disease is characterized by ineffective hematopoiesis with variability in clinical presentation, treatments, disease trajectory, and prognosis (Kurtin & Demakos, 2010). Although scientific discoveries have been robust, MDS remains largely an incurable disease. A number of studies have indicated that the leading cause of death in patients with MDS is related to the disease itself in more than 75% of patients (Dayyani et al., 2010). However, with the majority of patients being considered “elderly,” healthcare providers (HCPs), patients, and their caregivers often hesitate to pursue disease-modifying therapies based on chronological age alone, despite evidence showing positive effects on overall survival and quality of life (Kurtin, 2010; Life Beyond Limits, 2011). Additional factors noted to limit treatment options offered to the older adult population include fear

of toxicity, limited expectation of benefit, or ageism (Carreca & Balducci, 2009; Kurtin, 2010). Patients are reluctant to pursue active treatment for similar reasons, as well as concern for the cost of treatment and the strain on caregivers (Kurtin, 2010).

In addition to confirming the low threshold for tolerating moderate adverse events in a predominantly older adult population, several surveys of patients and providers have underscored the ambiguity in describing MDS as a myeloid malignancy and a reluctance to offer disease-modifying treatments based on risk analysis (Kurtin & Demakos, 2010; Sekeres, 2011; Sekeres et al., 2011). As a result, patients often are unable to describe the characteristics of their disease, including their International Prognostic Scoring System risk category, blasts percentage, cytogenetic abnormalities, and how these attributes correlate with their treatment options and prognosis (Demakos & Kurtin, 2011; Sekeres et al., 2011). A minority of patients in these surveys had been told that MDS is a malignant disorder. Many oncology practitioners today may see fewer than 10 patients with MDS per

MDS-Specific Organizations

- ▶ Life Beyond Limits
www.mdslifebeyondlimits.org
Brings together an independent group of MDS experts to raise awareness of ageism in access to care for patients with MDS
- ▶ MDS Beacon
www.mdsbeacon.com
Objective and unbiased news and other information related to MDS; mission is to be a key Internet resource and online community for patients with MDS, their families, and others interested in MDS
- ▶ MDS Foundation
www.mds-foundation.org
Multidisciplinary, international, nonprofit organization dedicated to the education of professionals, patients, and caregivers; facilitation and support of clinical trials; and development and support of patient advocacy groups
- ▶ United Kingdom MDS Patient Support Group
www.mdspatientsupport.org.uk
Offers support, information, referral advice, and patient information in the United Kingdom

Organizations That Include MDS Within the Scope of Hematologic Malignancies

- ▶ Aplastic Anemia and MDS Foundation
www.aamds.org
Nonprofit health organization dedicated to supporting patients and families living with aplastic anemia, MDS, paroxysmal nocturnal hemoglobinuria, and related bone marrow failure disease
- ▶ Leukaemia and Lymphoma Research Foundation
www.leukaemialymphomaresearch.org
Programs for support of all of the different blood cancers for patients and their families
- ▶ Leukaemia Care
www.leukaemiacare.org.uk
Resources for people affected by Hodgkin, non-Hodgkin, and other lymphomas; myeloma; MDS; aplastic anemia; and myeloproliferative disorders
- ▶ Leukemia and Lymphoma Society
www.lls.org
Mission is to cure leukemia, lymphoma, Hodgkin disease, and myeloma and improve the quality of life of patients and their families

General Resources

- ▶ American Cancer Society
www.cancer.org
- ▶ American Society of Clinical Oncology
www.asco.org and www.cancer.net
- ▶ American Society of Hematology
www.hematology.org
- ▶ CancerCare
www.cancercare.org
- ▶ Medline Plus®
www.nlm.nih.gov/medlineplus/medlineplus.html
- ▶ Merck Manual Home Edition for Patients and Caregivers
www.merckmanuals.com/home/index.html
- ▶ National Anemia Action Council
www.anemia.org
- ▶ National Heart, Lung and Blood Institute
www.nhlbi.nih.gov
- ▶ National Marrow Donor Registry
www.marrow.org

Clinical Trials and International Drug Approval Information

- ▶ European Medicines Agency
www.ema.europa.eu
Decentralized agency of the European Union, located in London; responsible for the scientific evaluation of medicines developed by pharmaceutical companies for use in the European Union
- ▶ Health Canada
www.hc-sc.gc.ca
Provides a notice of compliance (NOC) for full approval of a new drug or an NOC with conditions in Canada
- ▶ National Cancer Institute, National Institutes of Health
www.clinicaltrials.gov
Registry and results database of federally and privately supported clinical trials conducted in the United States and around the world
- ▶ National Institute of Health and Clinical Excellence
www.nice.org.uk
Guidance for cost effectiveness of treatments for England and Wales
- ▶ Nordic MDS Group
www.nmds.org
Provides Nordic guidelines for MDS management online and patient information in all Nordic languages
- ▶ Pharmaceuticals and Medical Devices Agency
www.pmda.go.jp
Regulation of drug availability in Japan
- ▶ Therapeutic Goods Administration
www.tga.gov.au
Division of the Australian government's Department of Health and Aging; responsible for regulating therapeutic goods including medicines, medical devices, blood, and blood products
- ▶ U.S. Food and Drug Administration
www.fda.gov
Approval required for commercial availability of therapy in the United States

Financial Assistance Programs

- ▶ American Cancer Society
www.cancer.org
- ▶ Anthony Nolan Trust
www.anthonynolan.org
Dedicated to bone marrow transplantation and running a database of donors
- ▶ CancerCare Co-Payment Assistance Foundation
www.cancercarecopay.org
- ▶ Cancer Financial Assistance Coalition
www.cancerfac.org
- ▶ Chronic Disease Fund
www.cdfund.org
- ▶ HealthWell Foundation
www.healthwellfoundation.org
- ▶ Lance Armstrong Foundation
www.livestrong.org
- ▶ Leukemia and Lymphoma Society
www.lls.org/copay
- ▶ MacMillan Cancer Support
www.macmillan.org.uk/Home.aspx
General information, assistance, and financial advice
- ▶ Patient Advocate Foundation Program Co-Pay Relief Program
www.copays.org
- ▶ Patient Handbook: Insurance and Reimbursement Resources for MDS Patients: A Guide to Assistance Programs in the U.S.
www.mds-foundation.org/for-patients-visitors

FIGURE 1. Patient and Caregiver Resource Catalog for Myelodysplastic Syndromes (MDS)

Aplastic Anemia and MDS Foundation (www.aamds.org)			
► <i>Be Transfusion Smart. Be Iron Smart</i>	—	—	●
► <i>Bone Marrow and Stem Cell Transplantation</i>	●	—	●
► <i>How to Evaluate Health Information on the Internet</i>	●	—	●
► <i>Iron Overload in Patients With Bone Marrow Failure Disease</i>	●	—	●
► <i>Understanding Clinical Trials</i>	—	—	●
► <i>What to Expect From Treatment: A Guide to Understanding FDA-Approved Drug Therapies for MDS</i>	—	—	●
► <i>Your Guide to Understanding MDS</i>	—	—	●
MDS Beacon (www.mdsbeacon.com)			
► <i>100 Questions and Answers on MDS</i>	—	—	—
MDS Foundation (www.mds-foundation.org)			
► <i>Anemia, Blood Transfusions, Iron Overload, and Myelodysplastic Syndromes</i>	●	●	●
► <i>Building Blocks of Hope™: Patient and Caregiver Strategies for LIVING with MDS</i>	●	●	●
► <i>Iron Overload DVD</i>	●	—	●
► <i>MDS Foundation Patient Diary</i>	●	—	●
► <i>Newsletter: MDS Foundation</i>	●	—	●
► <i>Insurance and Reimbursement Resources for MDS: A Guide to Assistance Programs in the United States</i>	—	—	●
► <i>Understanding MDS: A Patient Handbook</i>	●	●	●
► <i>What Does My Bone Marrow Do?</i>	●	—	●
MDS-UK Patient Support Group (www.mdspatientsupport.org.uk)			
► <i>Newsletter: MDS UK Patient Support Group</i>	—	—	●
<div> <div>● International focus</div> <div>● Multiple languages</div> <div>● Free</div> </div>			
FDA—U.S. Food and Drug Administration; MDS—myelodysplastic syndromes			

FIGURE 2. MDS-Specific Patient Publications

year, and most locally available support groups for patients with cancer will not include a single patient with MDS.

Together, the heterogeneity of the disease, heterogeneity of older adults, limited provider familiarity with MDS, recent changes in therapeutic and supportive care strategies, and paucity of resources for patient and family support at a local level emphasize the need to identify currently available resources for patients with MDS and their caregivers. Perhaps the most important prerequisite to assist patients and their caregivers in facing the challenge of a cancer diagnosis is to understand the disease itself and what each patient may experience based on an individualized risk analysis and then to have a basic familiarity with appropriate resources for

Patient Outreach and Advocacy Program

The MDS Foundation offers a patient advocacy and outreach program. Patients or caregivers may contact the patient liaison directly by calling (toll-free) 800-637-0839 or via e-mail to ahasan@mds-foundation.org.

patient and caregiver support. Tools and strategies for clinical management of patients with MDS and considerations for quality of life in these patients are reviewed elsewhere in this supplemental publication.

Patient Resources on Myelodysplastic Syndromes

Several surveys of both patients and HCPs have provided some insight into the characteristics of patients with MDS, what patients with MDS understand about their disease, what they perceive as unmet needs, and what they feel are the optimal characteristics of HCPs. The epidemiologic characteristics of the disease in all surveys confirmed the prevalence of MDS in patients older than age 65 years (Demakos & Kurtin, 2011; Ma, Does, Raza, & Mayne, 2007; Sekeres, 2011). That fact is important when considering the best approach to patient education and support given the trend toward online resources.

The MDS Foundation provides a patient advocacy and outreach program, which includes a full-time patient liaison available via telephone and e-mail. Patient phone calls to the MDS Foundation, together with patient surveys conducted as a part of patient and caregiver support programs supported by the MDS Foundation and the United Kingdom MDS Patient Support Group, have provided insight into the educational and supportive care needs of patients with MDS. Members of the International Nursing Leadership Board for the MDS Foundation serve as facilitators for the sessions. The most commonly asked questions in the sessions have been used as a template for the development of patient and caregiver education and support materials (see Appendix A).

Expectations of Patients and Providers

Working with patients facing an incurable disease requires time, compassion, clarity of message, and resilience on the part of HCPs. The shift toward survivorship care planning and personalized medicine places additional responsibilities on the oncology HCP to maintain a current working knowledge of a variety of conditions and treatment standards (Litton et al., 2010). Given the age of most patients with MDS, comorbid conditions are common, often requiring involvement of a number of specialists in addition to a primary care physician (Kurtin & Demakos, 2010). With the limited number of patients with MDS seen in a general oncology practice, the older age of most patients with MDS, and the provision of the majority of care in the outpatient setting where contact with the provider may be limited to 15–20 minutes per visit, clarity and consistency of information provided to patients and caregivers across all HCPs is an imperative and, yet, a daunting task. In addition, patients with cancer identify knowledgeable HCPs and adequate time for questions as desirable attributes of HCPs, emphasizing the need to employ a variety of educational strategies across disciplines.

Most patients facing a cancer diagnosis want to understand their disease, prognosis, available treatment options, potential adverse events, and expected duration of therapy (Protiere,

Implications for Practice

- ▶ Myelodysplastic syndromes are a class of incurable diseases requiring compassionate, clear, and consistent communication among healthcare providers (HCPs), patients, and caregivers.
- ▶ The majority of patients and caregivers want to understand their disease, prognosis, available treatment options, expected duration of therapy, potential adverse events, and strategies for taking an active role in their care.
- ▶ Effective patient, caregiver, and HCP communication will promote patient and caregiver participation in the decision-making process and self-care.

Moumjid, Bouhnik, Le Corroller Soriano, & Moatti, 2011; Rodin et al., 2009). A majority prefer to play a collaborative role in decision making (Brown, Parker, Furber, & Thomas, 2011). Effective patient-caregiver-HCP communication has been shown to improve patient and caregiver participation in decision making and self-care (Brown et al., 2011; Rodin et al., 2009). However, discordant expectations between patients and providers with regard to primary responsibility for survivorship care remain a challenge (Aubin et al., 2011; Cheung, Neville, Cameron, Cook, & Earle, 2009). Current literature supports an individualized approach to patient and caregiver education, with consideration of learning styles, cultural diversity, age, gender, treatment options, and prognosis (Cheung et al., 2009; Fujimori & Uchitomi, 2009; Rodin et al., 2009). Empowering the patient and caregiver to play an active role in patient care using a multidisciplinary approach with a consistent message used in an honest and empathetic way is perhaps one of the greatest tools for positive patient-caregiver-HCP communication.

Preparing the Patient and Family

Development of a consistent evidence-based description of MDS as a myeloid malignancy, defining risk-adapted treatment options including supportive care, and identification of available resources and programs for patient and caregiver support are critical to ensuring optimal outcomes. Adapting strategies to incorporate international variances in treatment approaches based on available therapies and resources is necessary. Individualizing support for each patient based on available resources including social support, availability of caregivers, financial resources, lifestyle, and personal choices for care and learning styles will promote the best outcome. Incorporating a multidisciplinary team approach including HCPs, social services, financial assistance counselors, support groups, and patient navigators is recommended.

Local, regional, national, and international programs and organizations focused on MDS, hematologic malignancies, and general cancer resources

(including financial assistance programs) provide important patient and caregiver resources (see Figure 1). Publications specific to the patient with MDS provide additional patient and caregiver support for those patients without access to or not comfortable with online resources (see Figure 2). As with many diseases in older adult populations, reliance on family members or friends to maintain the prescribed treatments, including travel to appointments, may place additional stressors on the patient and their support network. Careful evaluation of functional status, ability to tolerate treatments, effect of disease progression, and general overall health and family dynamics can provide the best opportunity for support of these patients. Assessment of activities of daily living may allow detection of deficiencies or deficits that require early intervention before they become problematic (Kurtin & Demakos, 2010).

Based on risk status, general health, and performance status, experimental therapeutic options should be presented as appropriate. Important points when considering patients for clinical trials also include the ability of the patient to understand the risks of experimental trials and the ability to give consent. Clinical trials also may provide the only opportunity for interventional therapy for patients who have failed currently approved therapies. Table 1 provides a summary of only a few of the ongoing active clinical trials in MDS.

Conclusion

The majority of clinical management of patients with MDS is provided in the outpatient setting and requires active participation of patients and caregivers for monitoring adverse events and adherence to treatment. Empowering patients

TABLE 1. Active Clinical Trials in MDS

Agent	Mechanism of Action	Phase	Indication
Arry-614	P38MAPK inhibitor	I	Lower risk
Gimatecan	Topoisomerase inhibitor	I	Lower and higher risk that have failed prior treatment
Alemtuzumab	Anti-CD52 immune modulation	II	Lower risk, hypoplastic
Oral azacitidine	Hypomethylating agent	II	Lower risk
Clofarabine	Nucleoside analog	II	Intermediate and higher risk
Vatalanib	Oral VEGF tyrosine kinase inhibitor	II	Primary or secondary MDS, any FAB subtype
Sapacitabine	Nucleoside analog	II/III	Intermediate and higher risk
Lenalidomide	IMiD® immunomodulatory agent	III	Lower risk, non-del(5q)
ON190	Unknown	III	Hypomethylating failure

FAB—French-American-British; MAPK—mitogen-activated protein kinase; MDS—myelodysplastic syndromes; VEGF—vascular endothelial growth factor
Note. Based on information from ClinicalTrials.gov, 2012.

and caregivers by providing clear, consistent, individualized information about their MDS, treatment plan, prognosis, and strategies for support is critical to effective management of patients. Despite the incurable nature of MDS, patients may live many months or years with this disease and will benefit from the numerous available resources. A compassionate, well-informed, and realistically optimistic oncology professional can make a world of difference to patients with MDS and their caregivers.

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For Exploration on the Go



Access information about patient resources and support organizations in the United States and internationally from Life Beyond Limits by opening a barcode scanner on your smartphone. Point your phone at the code and take a photo. Your phone will link to the content automatically.
Retrieve this content at www.mdslifebeyondlimits.org/about-mds/resources.

What is MDS? (MDS Foundation, 2011)

- MDS is a group of bone marrow disorders. The bone marrow is the factory for the production of blood cells including red blood cells, white blood cells, and platelets. In MDS, the bone marrow is abnormal because of a variety of malignant changes. The result is ineffective production of normal mature blood cells, resulting in low blood counts (cytopenias). Various subtypes of the disease exist with variable prognoses, treatment options, and risk of developing leukemia.

Is MDS cancer? (Bejar et al., 2011)

- The diagnosis of MDS requires a bone marrow biopsy and aspirate. The specimen is analyzed by pathologists specializing in blood disorders. The diagnosis of MDS requires specific malignant features such as dysplasia or cytogenetic abnormalities. Research has identified molecular abnormalities thought to play a role in the development of MDS. Given the underlying malignant features of the disease, MDS is considered a form of blood cancer.

What causes MDS? (Greenberg et al., 2011; Sekeres, 2011; Sekeres et al., 2011)

- The cause of MDS is unknown in more than 80% of diagnosed patients. It is more common in men (male to female ratio is 4.5:2 per 100,000). As with many types of cancer, older age is a predisposing factor. The majority (86%) of patients with MDS are older than age 60. Exposure to chemicals such as benzene and other solvents and tobacco smoke are known to increase the risk of developing MDS. Patients who receive certain types of chemotherapy or radiation treatment for other cancers may be at increased risk of developing treatment-related MDS.

Is MDS inheritable? (Sekeres, 2011)

- Inherited genetic predisposition for developing MDS and congenital abnormalities is rare.
- Before 1973, only 143 cases of MDS were reported. Today, based on data analysis techniques, the estimated incidence varies from 15,000–162,000 cases per year. The wide variation in these data highlights the challenging diagnostic features of MDS. As diagnostic features of MDS become more familiar to clinicians, MDS is detected more often in patients presenting with cytopenias (low blood counts). The development of therapeutic options may increase the number of patients considered for diagnostic evaluation. Increasing numbers of patients are being treated with cytotoxic therapies, raising the potential for secondary malignancies, including MDS (Cogle et al., 2011; Ma et al., 2007; Sekeres, 2011).

What are the symptoms of MDS? (Kurtin, 2011)

- Many patients are asymptomatic and are diagnosed on routine screening. Others present with vague symptoms associated with one or more cytopenias (low blood counts).
 - Fatigue, shortness of breath, palpitations (common anemia symptoms)
 - Fever, recurrent or prolonged infections (common neutropenia symptoms)
 - Bruising, petechiae, or bleeding (common thrombocytopenia symptoms)

How is MDS diagnosed? (Kurtin, 2011; National Comprehensive Cancer Network, 2011)

- The initial patient evaluation most often includes a complete blood count (CBC), which reveals normocytic or macrocytic anemia, normal to decreased numbers of neutrophils, and variable platelet counts. Anemia is observed in 90% of patients with MDS, either at initial

presentation or during the course of their disease. A careful history and additional laboratory analysis should be pursued to exclude other causes of cytopenias.

What are my treatment options? (Greenberg et al., 2011)

- Treatment selection for MDS is individualized based on recognized disease characteristics and risk analysis. Treatment options vary by region based on approval mechanisms. The goals of therapy for MDS are based on individualized disease characteristics, patient characteristics, and risk category. In the United States, the International Prognostic Scoring System (IPSS) categorizes the MDS subtypes into two major groups: low- and intermediate-1–risk or intermediate-2– or high-risk. The goal of therapy for each category differs based on expected survival and risk of leukemic transformation. A revised IPSS is being developed to further refine these risk categories and guide treatment selection. The World Health Organization Prognostic Scoring System, with similar treatment guidelines, is commonly used in Europe.

How likely am I to get better with the treatment?

- The response to treatment for patients with MDS varies according to IPSS risk categories as well as other prognostic indices. Allogeneic bone marrow transplantation remains the only potential cure to date. However, patients may benefit from currently available therapies, and durable responses have been reported.

How long will the treatment take to work?

- A minimum of four to six months of treatment is required to evaluate initial response, and the best response may not be evident until after as many as nine months of therapy.

How long can I expect to be treated? (Kurtin, 2011)

- Because of the limited number of treatment options and the incurable nature of the disease, disease-modifying treatments for MDS are continued until disease progression or unacceptable toxicity.

What are the common side effects of treatment, and what can be done to control them? (Kurtin, 2011; Kurtin & Demakos, 2010)

- The most common side effect for all therapies for MDS is myelosuppression including anemia, neutropenia, and thrombocytopenia.
 - Weekly complete blood count, differential, and platelet counts are recommended for the first eight weeks of treatment.
 - Cytopenias are expected to get worse before they get better.
 - Supportive care strategies are encouraged, including growth factors and transfusions.
 - Drug-specific guidelines for dose modifications or holidays are provided by each drug manufacturer based on clinical trials.
- Nausea and vomiting: all agents
 - Administration of antiemetic medication is an effective strategy to minimize nausea and vomiting.
- Constipation: all agents—also thought to be related to administration of 5HT₃ antagonist antiemetics
 - A regular bowel regimen that includes a stool softener and laxatives, as needed, will reduce the severity of constipation associated with treatment.
- Renal and hepatic toxicities—more common in older adults
 - Baseline and ongoing laboratory analysis will allow early identification and prompt intervention for potential renal and hepatic toxicities associated with treatment.
- Drug-specific adverse events

(Continued on the next page)

APPENDIX A. Most Frequently Asked Questions by Patients With Myelodysplastic Syndromes (MDS) and Their Caregivers Participating in the MDS Foundation Patient Advocacy Programs or Quality-of-Life Sessions

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- Azacitidine: injection-site reactions
- Lenalidomide: rash, pruritus, diarrhea, safety program for lenalidomide
- Iron overload
 - Chelation therapy may be associated with cytopenias and renal and hepatic toxicities.

What new treatments are on the horizon to treat patients with MDS? (Garcia-Manero, 2011, Kurtin, 2011)

- Clinical trials continue to explore treatment options for MDS and are always recommended for diseases that have limited treatment options, such as MDS. These trials offer hope to patients who have had limited benefit from approved therapies or have high-risk disease thought to have limited potential for benefit from these therapies. Each country has approved mechanisms for clinical trial oversight and drug approval.

Are blood transfusions dangerous? (Kurtin, 2011; National Comprehensive Cancer Network, 2011)

- The normal body mechanism for control of iron stores is highly efficient. Each unit of transfused blood delivers iron in excess of the normal daily requirements. After repeated transfusions, excess iron storage exceeds the levels that can be controlled by normal iron homeostatic mechanisms, leading to the formation of toxic iron storage and subsequent cellular damage.
- A strong correlation exists between transfusion intensity (number of units received over time) and organ damage.
- Iron accumulation results in end-organ damage.
 - Heart: congestive heart failure

- Liver: elevated liver function tests, hepatomegaly, pain
- Endocrine glands: diabetes
- Bone marrow: dysfunctional hematopoiesis
- Based on these data, transfusion dependence is considered an indication to initiate disease-modifying treatment for MDS

How do I select a bone marrow transplantation center? (National Marrow Donor Program, 2011)

- There are many factors to consider when choosing a transplantation center. Some patients look at a center's experience with certain diseases or ages of patients. Other patients choose a center close to their family and friends. Some things you and your referring doctor can find out about transplantation centers are the following.
 - What experience does this transplantation center have?
 - What do transplantation center survival statistics mean?
 - How does the number of transplantations conducted for your disease at this center compare with other centers?
 - What are the patient- and donor-matching levels required at this center?
 - What are some of the pretransplantation costs at this center?
 - Is this center covered under your insurance plan?

What can I do to keep myself healthy?

- The general principles of a healthy lifestyle remain important. A balanced diet, daily activity and exercise as tolerated, and participation in activities of enjoyment are important to maintain optimal health and well-being. Ongoing management of other health conditions is important to optimal health and continued eligibility for future treatment options.

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