



Are You Considering Stem Cell Transplantation? Questions to Ask Your Provider.

Klane White, MD

Stem cell transplantation can be an overwhelming prospect, especially for a family already facing the usually fresh diagnosis of MPS or related disease. Typically, the time line is short, and the amount of information that needs to be assimilated is daunting. A framework of questions for understanding transplant is presented here. The intent is to provide a list of questions you can bring to your health care provider, to help decide whether or not stem cell transplant is right for you and your family. For many of these questions, there is no single correct answer. Do not be afraid to compare answers between institutions or challenge answers that do not make sense to you. Please take time to contact families who have gone through the process. The National MPS Society can put you into contact with families who are more than happy to discuss their own experience. There are many unanticipated and individual courses that may not be explained by your health care provider. They may even offer their own opinions about these questions which may be insightful.

1) Who benefits from transplant?

Which MPS and related diseases have a proven track record with stem cell transplant?
Are enzyme levels or mutation analysis suggestive of an outcome that would be benefited by transplantation?

Are there preexisting medical problems which would make transplant too risky (heart, lungs)?

2) What type of transplant is best?

What kind of stem cell transplant is most appropriate? Cord blood, sibling bone marrow, unrelated bone marrow? What are the pros and cons of each?

What is the institutional bias/experience with each? What is the expected mortality?

3) Where should we go?

Where should you go for stem cell transplant?

Should you go to a regional or national center (such as Duke or Minnesota)?

Are there any physicians with MPS and related diseases experience at that institution?

How many MPS children has that institution transplanted?

What are their specific outcomes - what percentage of patients are successfully engrafted, what is their mortality rate, what is their incidence of graft versus host disease, both acute and chronic?

Does that institution provide supporting subspecialists with MPS experience?

4) When should the transplant be performed?
What are the timing considerations for finding a match?
What medical work-up/tests is are necessary before proceeding?

5) Why should transplant be considered?
Will brain function benefit from stem cell transplant?
How is enzyme replacement therapy different from transplant?

6) I the procedure be done?
Will enzyme replacement therapy be used in addition to
transplant? What are the logistics of the overall process?
What is the preparative regimen (chemotherapy, fractionated radiation, localized
radiation, whole body radiation)?
How long is the expected recovery?
What immunosuppressives are used post transplant?

7) What are the financial and personal
costs? What is the estimated cost of the
procedure? Will my insurance cover the
cost?
How long will we need to stay near the transplant center after
discharge? What follow-up will be required after we return home?

This fact sheet is not intended to replace medical advice or care. The contents of and opinions expressed in the fact sheet do not necessarily reflect the views of the National MPS Society or its membership.

REV 2020