

Questions and Answers from Bone Marrow, Stem Cell Transplant and Your Long Term Health

Answers provided by Dr. K. Scott Baker, University of Minnesota

1. What information do you need to know and share with future medical providers?

The number of patients we see in our clinic that know how they were treated is very, very low. We currently give all of our patients a treatment summary to try to correct that. The important things to know are:

-What type of transplant you had. You should know if it was an autologous transplant where they gave you your own marrow back or an allogenic transplant where the marrow came from a sibling or other related or unrelated donor.

- The other important thing is the treatment you had with the transplant. Did you receive total body radiation and other chemotherapy drugs? Know the names of the drugs and whether or not you received radiation therapy.

- The final thing is if they had graft vs. host disease and if they had to be treated for it. The details of that are not as important.

The main thing is the type of transplant and the actual type of preparative regimen they received before the transplant.

2. My daughter is 2 1/2 and has medullablastoma in the cerebellum. She has completed five cycles of chemotherapy and is going into autologous bone marrow transplant in a month. They have already completed stem cell collection. Do your numbers change at all on risks and survival if a child gets her own bone marrow back versus a donor?

The risks are lower when you receive your own marrow. Just because a lot of the complications that we see during transplant are not as common in the autologous transplant as when getting bone marrow from a related or unrelated individual. On a long term basis the issues are mostly the same. It comes back to how the transplant is done and the actual chemotherapy or radiation therapy that's delivered with it. Certainly all the issues potentially related to graft vs. host disease and the long-term effects of that are out of the picture in an autologous transplant. So the list of possibilities is shorter, and in general, not quite as severe as the allogenic transplant.

Because of her age and the effects of radiation on children under a certain age, we avoided radiation. We didn't want to stifle her cognitive abilities. But we did use drugs like methotrexate along with cytoxin, cisplatin and VP16. Does an autologous bone marrow transplant without radiation help reduce the possible side effects?

The most significant late effects that we see in children, whether they receive a transplant or not, are really from the radiation. So avoiding radiation avoids a lot of the more significant kinds of long-term complications. Now, having said that, there are certain situations where that is just not avoidable and certain situations where we know that total body radiation (TBI) with the transplant provides the best chance of cure. For your

daughter, the transplant seems to be a way to avoid the radiation given her young age. That is another approach that is taken fairly commonly because we know the effects of radiation on a child who is two are going to be much greater than giving that same radiation to a child who is ten. Transplant is a way to spare radiation for right now. Some of those protocols have actually come back after the child is over three and given them radiation. Again, it is an age related issue.

3. I have a 14 year old daughter who was diagnosed with MDS about two and a half years ago. She has a non-related donor. I was wondering about the malignancies related to MDS. Can you elaborate on this?

Therapy related MDS and AML is mainly an issue that is seen in patient's who have autologous transplant. There is a higher risk of new leukemia, either MDS or AML, that develops in patient's who receive an autologous transplant, mainly because their bone marrow has already been exposed to fairly intensive chemotherapy. Then they have an autologous transplant to get their own bone marrow back. There has been a higher rate of a new leukemia developing in their bone marrow again.

In your daughter's situation, since she is getting an unrelated donor the bone marrow has not been exposed to these drugs and so her risk of these types of diseases is not going to be any higher than the general population.

4. My son is five and half years post-transplant and we just found a big cyst, bigger than a silver dollar, on his growth plate on his right knee. We have not been to our transplant center. The doctors here are saying don't worry about it. Do you have any information about cysts like this?

There is hardly anything that has actually been studied or published on those kinds of problems, but from our own clinic's experience we see similar kinds of things pretty frequently. Osteochondroma, a benign tumor of the bone, is one of the things that show up like this. These are pretty common and typically do not need to be dealt with in any way unless they are causing pain or problems. Then they may need to have something done with them. Has your son been seen by an orthopedic surgeon?

Yes, he was seen by a pediatric orthopedic surgeon.

Run it by the transplant center when you go. If they said just watch it, it should probably be fine. If he had not been seen by an orthopedic doctor, I would recommend that.

Does it effect growth at all?

No, not usually. Some patients have had multiple ones of these. Typically, unless they are really causing pain they do not do anything with them because sometimes they just come back after the surgery.

5. If there isn't a late effects clinic near you, what do you recommend?

There doesn't have to necessarily have to be a specific late effects clinic, but. I would never hesitate to go back to the primary oncologist or the transplant center. We have seen the patient who has had difficulty going back to a local primary care physician who has been reluctant to get too concerned about something they were complaining about or to sort of minimize it and not do any test to investigate it further. Sometimes you have to say I had a transplant or my child had a transplant and this could be related to that. You have to be fairly proactive in situations like that. You can always contact your primary oncologist by phone for recommendations. At our clinic, we see patients who weren't necessarily treated here. So there are other options.

6. I have an 11 year old child who is high risk ALL. She has an 80% chance of bone marrow transplant. I am on an on-line support group. In the discussions of children who have relapsed, people state you should know your relapse protocol and how it will effect future treatment. I don't understand what they mean by that. Are there different choices that can effect what you do further down the road?

From a transplant standpoint, not really. I am not sure what they would be referring to. For our leukemia patients that come to transplant because of relapse there is really not anything that they would be treated with prior to transplant that would effect their future treatment.

Are there multiple things that you have to look at before having another transplant, if a transplant doesn't work or fails?

Once you have had a transplant, the issue is you have probably received total body radiation and you can not receive it again. A second transplant would be with chemotherapy only. Most ALL patients that are not infants would be treated with total body radiation and so with that disease a second transplant, if a second transplant was considered, would have to be a chemotherapy only transplant.

7. My child has had multiple complications and a very difficult two years of treatment. She was diagnosed with aspergillus fungal infection and has been diagnosed with avascular necrosis (AVN) in the shoulder and hips. She has not been able to continue on steroids and had a severe reaction to the l-asparaginase. She had to stop taking it after the third injection. I am concerned that she would not be able to survive the medication because she has been so sensitive to her current protocol. Have you seen more problems or the inability to go to transplant in children who have been very sensitive to things? If she were to go to transplant, how would her reaction to steroids and the AVN impact it?

AVN is definitely a problem. It would not preclude her from having a transplant. If during the transplant she would need more steroids than that probably could get even worse. Her doctors may be more reluctant to put her right on steroids if there is some other way to get around it.

The l-asparaginase reactions are very common. We don't really see any problems with transplant patients that have had reactions similar to your daughter's. The most concerning thing is the aspergillus infection, unless that has been completely cleared up.

She does have scar tissue on the lungs from the aspergillus. Would that affect the lungs in a transplant?

What we would typically do is keep a patient on anti-fungal antibiotics throughout the transplant process. With other patients, we would not necessarily start those drugs unless there was potential for fungal infection. Basically all the things that she has had are not that uncommon to patients before coming to transplant. They should not affect her ability to have one.

The only issue is the more treatment you have, the more of a toll it takes on your body. Her overall general health may not be as good as another patient going into it. Having said that I have learned that you can not really predict how well a patient is going to tolerate it. You see ones that are really high risk going through transplant that just sail through and ones that seem to be in perfect health don't. It is hard to predict this. There is nothing that seems that out of the ordinary with her that they would not consider her for transplant.

8. My son is a year and half post-transplant. He had neuroblastoma. I have a follow-up plan from the transplant facility. It doesn't seem as detailed as the treatment plan you included. I was under the impression that my son would be transferred to a long-term care program when he was a little further out from transplant. Some of the scans listed he is not getting one year post transplant.

There may be a separate list of labs that they do from a long-term follow-up standpoint compared to more of the disease specific follow-up. In our clinic we have a separate set of orders that are based on the disease that would cover the scans. The handout is more from the long-term follow-up standpoint.

A lot of places do not have a grid or protocol that they follow for the regular long-term follow-up kind of issues. They may order them through their common knowledge or practice without having something specific that they follow from.

Should I call the long-term follow-up facility at the transplant facility?

There may be a standard time point where they would refer your child into that clinic. We typically start seeing patients in our long term follow-up clinic at their third year anniversary of post-transplant. The first year screening test would be done in the regular transplant clinic.

The other thing that I would mention about the grid, sometimes we have patients that have not been back for ten years. We start at year one with them and screen everything. If your transplant facility has a long-term follow-up clinic, check with them or the

transplant doctor to see if there is a time point when they would transfer them to long term clinic.

There is not protocol for when they transfer to a long term follow-up clinic?

Some transplant programs see all of their long-term patients in the same clinic as they see the regular patients and are doing both simultaneously. There is not a protocol on that.