Pediatric Hematopoietic Stem Cell Transplant - Experience of an Indian Tertiary Care Center

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Introduction

• Considerable progress has been made in HSCT for pediatric malignant and nonmalignant conditions in terms of:
  ✓ Type of pediatric diseases successfully treated with HSCT
  ✓ Use of alternative donor stem-cell sources
  ✓ New conditioning regimens
  ✓ Awareness of late effects in pediatric HSCT recipients
• We present our transplant data pertaining to the pediatric HSCT done at our center from Jan 2008 till date
Methods

• Patients aged less than 18 years of age at the time of HSCT were defined as pediatric population and included in the analysis

• All the patients irrespective of diagnosis were included in the analysis
Conditioning regimen

- Variety of conditioning regimen were used such as: depending on disease and patient specific issues

1. BuCy (Busulfan, cyclophosphamide)
2. Flu-Mel (fludarabine, melphalan)
3. Bu-Flu (busulphan, fludarabine)
4. TTF (treosulphan, thiotepa and fludarabine)
5. CyATG (cyclophosphamide, ATG)
Facility

• All patients underwent transplant in High efficiency particle air filtration (HEPA) unit equipped with positive pressure ventilation (PPV)
• Transplant unit and nursing team are dedicated - not used for other purpose
• Many of the nursing staff and doctors are working in unit for over 5-7 years
Stem Cell Source

• All of the patients received filgrastim (GSCF) mobilized peripheral blood stem cell (PBSC) graft, except one, for whom bone marrow stem cells were collected
Results

• Data of patients with both, malignant and non-malignant hematological illnesses was analyzed in terms of
  1. rates of Engraftment
  2. Day 100 survival
  3. Disease free survival.
Male & Female Transplant Number - 24

- Male: 19
- Female: 5

Total: 24
Disease wise Distribution of patients

- AML: 9
- Thalassemia: 7
- H L: 3
- Neuroblastoma: 2
- ALL: 1
- N H L: 1
- A A: 1
Haploidentical transplants (current survival 5 out of 8)

Matched Sibling transplants (current survival 8 out of 10)
## Survival Data

<table>
<thead>
<tr>
<th>Disease</th>
<th>Engraftment</th>
<th>Day 100 Survival</th>
<th>Long Term Survival</th>
</tr>
</thead>
<tbody>
<tr>
<td>AML</td>
<td>67%</td>
<td>83%</td>
<td>56%</td>
</tr>
<tr>
<td>Thalassemia</td>
<td>100%</td>
<td>100%</td>
<td>86%</td>
</tr>
<tr>
<td>ALL</td>
<td>100%</td>
<td>100%</td>
<td>100%</td>
</tr>
<tr>
<td>H L</td>
<td>100%</td>
<td>100%</td>
<td>100%</td>
</tr>
<tr>
<td>N H L</td>
<td>100%</td>
<td>100%</td>
<td>100%</td>
</tr>
<tr>
<td>A A</td>
<td>100%</td>
<td>100%</td>
<td>100%</td>
</tr>
<tr>
<td>Neuroblastoma</td>
<td>100%</td>
<td>100%</td>
<td>50%</td>
</tr>
</tbody>
</table>
Conclusions

• HSCT in pediatric population is an important treatment modality especially for malignancies such as Acute Myeloid Leukemia, Acute Lymphoid Leukemia, Neuroblastoma.

• It is also an important treatment for Serious Non malignant diseases like Thalassemia Major, Sickle cell Anemia, Aplastic anemia and others.

• Children tolerate transplant better than adults, and have significantly better results, including lower mortality, low graft versus host disease.
• For non caucasians, finding a match in unrelated donor registries is very uncommon.

• Haplo-identical transplant is an attractive strategy for patients lacking fully matched donor, especially for high risk patients, in urgent need for transplant, with results comparable to matched unrelated donor transplant.
• Results from our center are comparable to those from other centers where pediatric transplants are performed regularly.

• For diseases such as thalassemia, transplants should be considered early in the course of disease given the better results.

• Same is applicable to Sickle Cell Disease.
Thank you

- Dr Chirag A Shah
- Dr Arun Karanwal
- Dr Vinay Bohara
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- Ms Upasana, Ms Dhanya, Ms Sonal and Nursing Team; Ms Khyati
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