WORTHINESS OF BONE MARROW TRANSPLANTATION AS A TREATMENT OPTION FOR THALASSEMIA PATIENTS IN DEVELOPING COUNTRIES

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Introduction

- Thalassemia is a type of hemoglobinopathy due to either decreased or absent normal globin components (β-globin, α-globin)

- Conventional treatment consists of:
  - **Blood transfusion** to correct anemia
  - **chelation therapy** for transfusion-associated iron load
  - **Treatment of other complications** resulting from either anemia or iron overload. e.g. heart failure, viral infections, and endocrinopathies
Examples

• In Egypt
  - carrier rate: up to 9%
  - estimate of 1,000/1.5 million per year live births suffers from thalassemia.

• In Albania:
  - about 300 thousand carriers (9-8% of population)

• Global disease burden ~ 1 million (concentrated in developing countries).
Problem: \textbf{(Disease > Recourses)} ..... 

- Continuing supportive care costs:
  - \textbf{25,400 euros per year} in Albania, and
  - \textbf{12,300 euros per year} in Egypt

- Albania has a shortage of facilities for supportive care:
  - Lack of blood (blood not always available to patients)
  - Treatment costs approaches \textit{European standards}.

- In Egypt:
  - Not enough resources to manage increasing patient load.
  - High carrier rate with lack of efficient preventive program.
  - HSCT demand exceeds current centres capacity; priority is given to urgent malignant cases.
Solution....(HSCT?)

• First BMT for a thalassemia case done in the 1980’s.

• Stem cell transplantation is the only conclusive cure for thalassemia:
  • **Young ages** with disease complications is still minimal.
  • **Matched** related donors (more likely due to large family size)

• BMT greatly improves the quality of life for both patients and families by decreasing medical, psychological and financial burdens of continuous treatment.
Vision....

• The experience of the Cure2Children Foundation-supported BMT networks in developing countries, namely, in Pakistan and India:
  o cooperation programs with local institutions.
  o training of local health professionals
  o Long-term assisted follow up (easier to maintain in patient’s home country than travelling abroad)
  o BMT-associated prevention program.

• Hospital infection control: Probably more important and cost-effective than air filtration (HEPA) and positive pressure:
  ➢ Hand washing and disinfection
  ➢ Easily cleanable room
  ➢ Dry environment
  ➢ Surfaces and air sample monitoring
• Protocol used (Lucarelli’s regimen 6.1):
  ➢ Thiotepa (10mg/kg)
  ➢ Oral busulfan (14mg/kg)
  ➢ Cyclophosphamide (200 mg/kg)

• For GVHD prophylaxis:
  - Triple drug (prednisone/methotrexate/cyclosporin)
Outcome....

- Low-risk matched-related BMT in children younger than 6 years and liver less than 2 cm could deliver:
  - 92% thalassemia-free survival
  - with 100% performance score
  - no extensive chronic GVHD,
  - no Aspergillosis
  - average cost: 10,000-15,000 USD

Analysis of 12 initial cases performed in 2 start up institutions within a close cooperation program.
Median follow-up: 7.5 months (3.5.33.5)
Conclusions

Curing thalassemia in early age with once-in-a-life bone marrow transplantation can be the solution for long-standing health and financial suffering from the disease.

In young children with a compatible donor high cure rates can be achieved in developing countries within close cooperation programs...

...and cost-effectively!
Take home message...

Promote awareness of BMT also in needy communities.... it could be more accessible and effective than many people think!
THANK YOU!