5. Quality of Life in Thalassemia

Principles

- Since mortality in thalassemia is fortunately a rare event, health care providers should focus on minimising morbidity and maximising quality of life (QOL). As clearly demonstrated in the pediatric data, better overall health care has a large impact.
- Children treated in developed countries have self-reporting QOL scores that approach that of the healthy population. Safe, regular blood transfusions and access to iron chelation therapy improve patients' QOL.
- State of the art thalassemia care has also been shown to improve fertility.[21] The new oral iron chelation agents have been shown in a randomized control trial to improve SF-36v2 scores in thalassemia patients.22 Finally, bone marrow transplant, the only available cure for the disease, significantly improves PedsQL scores pre-transplant from 63 out of 100 to 94 eighteen months post-transplant.[23]

Recommendation

- Patients with thalassemia should have QOL assessment performed regularly by health care providers.
- QOL impact should be factored into management decisions for patients with thalassemia.

Background

With modern treatment, the life expectancy of patients with thalassemia has improved dramatically.[24] This places a heightened emphasis on evaluating morbidity and quality of life (QOL). The impact of thalassemia on patient's QOL is two-fold. The disease itself can affect patients' primarily thorough decreased energy and disease complications, and the transfusion and iron chelation treatment creates a burden on the patient and family. The World Health Organisation has been defined QOL as "an individuals' perceptions of their position in life, in the context of the culture and value systems in which they live, and in relation to their goals, expectations, standards, and concerns".[25] A systematic review done in 2006 found only 4 studies that measured QOL in patients with thalassemia.[26] Since that time there have been fortunately many more studies published involving both children and adults, which consistently report that patients with thalassemia report lower QOL scores compared to the general population.Studies in children, using the Pediatric Quality of Life Inventory (PedsQL™ 4.0) consistently showed significantly lower scores in all four domains of the measure, including physical, emotional, social and school functioning. Children in developing countries such as Egypt had the worst scores with a mean total score of 64 out of 100, increasing to 77 in Thailand and approaching the normal population in North America at 85.[27,28] One report from Turkey found that 19% of adolescents self-reported significant anxiety and 20% being depressed. Do not forget about the impact of the disease on caregivers, since the same study showed that 29% reported having significant depression.[29] In adults, the Medical Outcomes Study 35-item Short Form Health Survey (SF36V2) is the tool used most often, with a recent Thalassemia Clinical Research Network study finding significantly worse QOL in 5 of the 8 sub domains with the largest effect size being in general health, with a smaller effect size in physical functioning, role-physical, social functioning, and role emotional. Patients with more disease complications and the elderly had the lowest scores.[22, 30] Recently, a disease-specific tool, called the TranQol, was developed in North America for children and adult with transfusion-dependent thalassemia. The tool was found to be valid, reliable and responsive to change, and provides a good option for assessing QOL in this population.[27]

Intervention

- Quality of Life should be assessed at every clinic visit, including physical, emotional, and family health, as well as the impact of the disease on school/career. Areas of concern should be communicated to the rest of the Health Care Providers (HCP) in the team so that appropriate corrective action can be taken.
- A standardized QOL tool, either a generic QOL tool such as the PedsQL for children and the SF 36v2 for adults and/or a disease-specific QOL tool such as the TranQol, should be measured at regular intervals every 1-2 years. The result of the standardised assessment should be tracked in the patient's Electronic Medical Record (EMR).
- When multiple options for therapy are available and of similar medical efficacy, patient preference should be accommodated, with the goal of minimizing negative impacts of treatment on QOL.