MedicalXpress

Canadian researchers discover how to measure quality of life for rare blood condition

November 4 2013

A CHEO-led multi-site North American study, headed by Dr. Robert Klaassen, lead investigator at CHEO and associate professor in the University of Ottawa's Department of Pediatrics, has confirmed the validity and reliability of a quality of life measurement tool for children and adults with thalassemia major. The tool was created between 2006 and 2009 through the combined efforts of CHEO, the Hospital for SickKids and Laurentian University.

Symptoms of thalassemia major, a rare genetic condition, include severe anemia in the first year of life, chronic fatigue and failure to thrive. The only cure is bone marrow transplantation. Unfortunately, this option is available only to a minority of patients.

Patients with this blood condition receive multiple red cell transfusions in any given year, which inevitably leads to iron overload, as the body has no natural way to get rid of iron. However, pharmaceutical companies are creating therapies to treat this condition. While in the past, medication was commonly administered by a needle in the arm for 10 hours every night, advances in medicine have introduced oral agents. All treatments are expensive and have side effects.

What interested the research team, whose findings were published today in the *British Journal of Hematology*, was the overall quality of life for these patients over the long term. "Quality of life is moving from a

research tool, where studies are done to show that one drug is better than another drug, and it's evolving into something that practitioners will use to optimize individual patient care," said Klaassen. "The future of health care will incorporate quality of life surveys into electronic patient records, so physicians and allied health care professionals can build a care plan around a patient's physical symptoms, emotional state, support networks, environmental restrictions and so on."

The disease-specific quality of life measurement tool is called TranQol (transfusion quality of life). It includes four sub-domains for a pediatric population, including physical domain, emotional domain, social domain and school/career domain. The research team took three years to build the tool, and conducted almost four years of reliability and validation testing across North America. It performed over 100 interviews with health care professionals with expertise in thalassemia, patients, adults, children and parents. The validation and reliability results are based on self-reporting by patients.

"There is a trend happening now: pharmaceutical companies are chasing rare disorders because there is less competition and a faster, higher potential impact," says Klaassen. "For the first time in 40 years, the hematology field is exploding—and our evidence-based measurement tool is going to be key to understand what, how and why different therapies work, or don't."

To perform the validation and reliability testing, the CHEO research team collaborated with hospitals from Boston, Philadelphia, Vancouver, Sudbury, Toronto and Oakland. Funding was provided by Novartis Canada.

Provided by Children's Hospital of Eastern Ontario Research Institute