

## **Transfusion Dependent Thalassemia (TDT) Comprehensive Monitoring Guidelines**

Annual visits to a comprehensive thalassemia center are strongly recommended for care from experienced providers. Assessment of compliance with guidelines, counseling, and discussion of modifications to the treatment paradigm can be provided as needed.

In addition to monitoring iron burden and adherence to chelation therapy, the structure, function and overall integrity of organ systems must also be monitored for development of abnormalities related to a transfusion and chelation regimen.

### **Heart**

The ideal method for assessing cardiac function is magnetic resonance assessment of chamber dimensions, ventricular filling and ejection fractions. This provides indicators of systolic as well as diastolic function. Conventional echocardiography does not provide adequate assessment of diastolic function, and is suboptimal because cardiac dysfunction in iron-induced cardiac disease is mainly diastolic, with systolic function well preserved until late in the course of iron-induced restrictive cardiomyopathy. Normal results may lead to a false sense of well-being and non-compliance with chelation. Serial measurements may provide useful trend information, and may signal the need for closer MRI evaluation. Despite preserved global function, tissue Doppler echocardiography-detected regional wall motion abnormalities may represent an early sign of cardiac disease. Special echocardiographic techniques may be of limited use, but annual magnetic resonance-based cardiac function assessment, performed at the same time as T2\* measurement, is the ideal method of monitoring. Holter monitoring for development of arrhythmias is also recommended annually.

### **Liver and Kidney**

Regular assessment of hepatic and renal function should also be part of comprehensive care. In addition to screening for transfusion-associated viral infections, frequent assessment is also required to monitor for chelator toxicity. A comprehensive metabolic panel, including liver and kidney function, is recommended once per month. In addition, urine is sent monthly for beta2 microglobulin, for patients on deferasirox.

### **Endocrine System**

Comprehensive annual endocrine evaluation, including thyroid and parathyroid function (calcium and phosphorus levels and bone mineral density), pancreatic function (oral glucose tolerance tests are required because the HbA1C is not useful in chronically transfused individuals), and gonadal function testing (testosterone and estrogen levels) are recommended. An endocrinologist is included as part of a team for overall endocrine management. For growing children and adolescents, close monitoring of growth, achievement of puberty and bone maturation are followed by endocrinology.

**Other**

Vision and hearing should be evaluated annually in patients on desferrioxamine and deferasirox. Pulmonary function testing is also recommended, though less frequently. Chelator toxicity should also be monitored.