Thalassemia & Hemoglobinopathy Program

The Pediatric Red Cell Disorders Program follows children with inherited conditions such as thalassemia, sickle cell anemia, spherocytosis, and other red cell disorders, including children with anemia and iron excess/deficiency.

We are able to provide transfusions on an outpatient basis, and have state-of-the-art radiology facilities for procedures like MRI for assessing tissue iron. Ongoing clinical trials in thalassemia are investigating the safety and efficacy of novel treatments which have the potential for significantly reducing the burden of this condition.

Services & Programs

Comprehensive Thalassemia Center

Our Comprehensive Thalassemia Center is the oldest and one of the largest centers of its kind in the United States. Individuals with thalassemia receive their transfusions in a new state-of-the-art day hospital setting, with a multidisciplinary team providing psychosocial services, thalassemia screening, and genetic counseling.
Directed by Dr. Sujit Sheth, the New York Comprehensive Thalassemia Center coordinates and links multiple services with the assistance of a nurse coordinator and a collaborative team of pediatric consultants including cardiologists, endocrinologists and gastroenterologists. Services are also provided by genetic counselors, social workers and psychologists. Optimal care requires routine surveillance and monitoring of patients’ conditions, treatment response, efficacy, safety and early interventions to minimize complications. A complex network of linked services are provided, ranging from screening, diagnosis, genetic counseling, prenatal diagnosis and psychosocial services, to specialized medical care and stem cell transplantation.

**Transition Program**

Many age-related health issues arise as patients with thalassemia transition from childhood into adulthood, including delayed puberty, fertility complications and early-onset osteoporosis. Transitioning is crucial to maintaining continuity of care and providing patients with best possible outcomes as adults.

Our team works with transitioning patients to increase their knowledge about their disease, discuss the importance of keeping appointments and calling the thalassemia center about any problems, encourage compliance and self-administration of medications and/or treatments with less parental involvement, and eventually transfer decision-making from parents to patients. This process gradually encourages more independent patient treatments and self-sufficient care.

**Adult Program**

The adult transfusion program is located on the 3rd floor of the Starr Pavilion at the NewYork-Presbyterian Hospital/Weill Cornell Medical Center (NYPH/WCMC). To accommodate the needs of our adult population, this state-of-the-art facility conducts several transfusion sessions per week, including two evening sessions that allow patients to attend college classes or go to work during the day. As our patients with thalassemia survive longer through improvements in blood supply, transfusion therapy and iron chelation, they may require specialists to monitor for potential complications. These include cardiologists, endocrinologists, infectious disease specialists, hepatologists, nephrologists and others. Emergency services are available 24 hours per day, and a telephone answering service is available after the clinic is closed.

A home care program for the administration of iron chelation is also coordinated within the Comprehensive Thalassemia Center. 24-hour emergency services, a telephone answering service, and access to hospital care for emergencies are available through the ER service at NYPH.
Diagnosis & Treatment

The treatment center coordinates and links multiple services with the assistance of a nurse coordinator and a collaborative team of pediatric consultants including cardiologists, endocrinologists and gastroenterologists. Services are also provided by genetic counselors, social workers and psychologists.

We administer red blood cell transfusions and timely initiation of iron chelation to prevent the complications of transfusional iron overload. Quarterly, comprehensive evaluations and annual studies are provided to address the regular surveillance, monitoring, prevention and treatment of hepatic, endocrine and cardiac complications related to iron overload and the potential infectious contamination or alloimmunization from transfusions.

What to expect

Research

The Comprehensive Thalassemia Center is committed to advancing the care of individuals with thalassemia through research and participation in clinical trials.

We have several research initiatives, including studies assessing the effectiveness of comprehensive care and quality of life, research utilizing MRI techniques to assess iron-related liver disease, and a clinical trial assessing the benefits of using fresher blood to reduce transfusion requirements.

We have several industry-sponsored, multicenter clinical trials underway. These include a Luspatercept study (Celgene’s BELIEVE trial), a pathogen inactivation study (Terumo’s PRAISE trial), and a deferasirox granules study (Novartis’ CICL670F2202 trial).
Resources

- **Cooley’s Anemia Foundation**: The Cooley’s Anemia Foundation is dedicated to serving people afflicted with various forms of thalassemia, most notably the major form of this genetic blood disease, Cooley’s anemia/thalassemia major. The mission of the Cooley’s Anemia Foundation is to increase life expectancy and enhance the quality of life for those impacted by thalassemia by funding medical research to advance treatment and curative approaches, by supporting and advising patients and their families and advocating on their behalf and by educating medical professionals and the general public. For over fifty years, the Cooley’s Anemia Foundation, a 501(c)(3) nonprofit organization, has been a strong and supportive partner for families living with thalassemia.

- **Thalassemia International Federation (TIF)**: TIF’s mission is to promote and implement national control programs for the prevention and treatment of thalassemia and other hemoglobin disorders in every affected country. Through the creation of new and the strengthening existing associations, delegation visits to countries of need, a multimodal educational program, networks, partnerships and collaborations with relevant stakeholders, their vision is to ensure equal access to quality health care for every patient with thalassemia and other hemoglobin disorders across the world. The Thalassaemia International Federation is a non-profit, non-governmental organization founded in 1986 by a small group of patients and parents in Cyprus, Greece, the UK, the USA and Italy.

**What Sets us Apart**

- As one of the longest-standing care centers in the country, we have extensive experience and expertise in treating thalassemia.

- New York State Department of Health has recognized our practice as a designated Hemoglobinopathy Specialty Center providing comprehensive care to children and adolescents with disorders such as the thalassemia syndromes, sickle cell anemia, and other red blood cell disorders.

- The New York Comprehensive Thalassemia Center has a rich history of participating in and conducting clinical trials resulting in major advances in care and improvement in outcomes through a reduction in disease.
Thalassemia, Hemoglobinopathy and Red Blood Cell Disorders Program
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