PRESIDENT'S PAGE

Championing the Cause of Rare Blood Disorders in Children

GV Basavaraja

National President, Indian Academy of Pediatrics, 2024
president@iapindia.org

Dear IAPians,

As we celebrate the World Thalassemia Day on May 8, let us turn our attention to the commonly overlooked realm of rare blood disorders. While these conditions may not always command the spotlight of regulatory bodies due to the perceived lack of significant economic impact, as pediatricians we do understand that for the children afflicted by these conditions and their families, these disorders are a lifelong challenge.

Thalassemia, an uncommon hemoglobinopathy, profoundly impacts the lives of those affected by this blood disorder. Dependence on recurrent blood transfusions for survival is only one of the several challenges faced by individuals with thalassemia. A lack of awareness among families further exacerbates this problem as it leads to erratic medical visits. The consequences of this neglect manifest as symptoms of chronic fatigue, abnormal physical features, and enlargement of the liver and spleen. Additionally, the very treatment meant to sustain life - transfusions carry their own set of risks, including transfusion reactions and the risk of transmitting infections like hepatitis B and HIV. However, the most ominous threat that looms over these patients remains iron overload which wreaks havoc on vital organs like the liver and heart, stunts growth, causes hypogonadism and a host of complications including hormonal imbalances and diabetes [1]. Unfortunately, not all patients are able to access the chelators needed to tackle the iron overload due to issues of availability and also because these are not available free of cost at all centres. Despite the existence of hematopoietic stem cell transplantation, the potentially curative treatment, such interventions remain elusive for most patients as they are costly and available at select centres. There is a need for garnering more awareness for blood donation initiatives and increasing the access to comprehensive care for those living with thalassemia.

On April 17, we also celebrated the World Hemophilia Day and we must continue our efforts to increase awareness about this rare clotting disorder. Although, we have transitioned from our reliance on blood components like cryoprecipitate and fresh frozen plasma to the use of recombinant factors not only for treating hemophilia but also for prophylaxis, the access to these treatments is not uniform across all states in India [2]. Sadly, individuals with hemophilia continue to face the spectre of protracted and excessive bleeding triggered by even minor injuries, and in some cases, spontaneously. Joint hematomas, a frequent complication, not only inflict agonizing pain but also carry the potential for permanent disability if left untreated [3]. Intracranial bleeds remain the most dreaded complication, underscoring the urgency for raising awareness about this condition.

On the bright side, with the declaration of the Rights of Persons with Disabilities Act, 2016, blood disorders including thalassemia, hemophilia and sickle cell anemia were recognised as a benchmark disabilities [4]. This lifted off the veil covering the face of these blood disorders. In 2024, the Director General of Health Services (DGHS) has directed all hospitals to maintain disease-specific transition registries and organise joint clinics for transition of patients suffering from chronic diseases including thalassemia and hemophilia, to an adult care team in the same hospital for uninterrupted comprehensive treatment [5]. These developments have given us direction and motivation to strive for better care for these patients as we offer them the hope of increased quality survival. Together, through education, advocacy, and compassionate care, we can work towards a future where no child's life is defined or diminished by the burden of these conditions.

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