blood® Editorial

Introduction to a review series on pediatric hematology

Although many of the hematologic disorders covered in this review series also affect adults, the pathophysiology, diagnostic approach, and clinical management of many of these disorders is distinctly different in children. In this series, we focus on these differences. Our aim is to provide an update about how scientific and clinical advances in hematology have impacted pediatric hematology practice. The development and application of next-generation sequencing has identified hundreds of different disease-causing genes in childhood blood disorders and, as all the articles in this series highlight, this is already leading to very significant changes in how we manage an increasing range of disorders and providing fascinating insight into fundamental biology. We hope this update will be useful for adult hematologists who find themselves consulting on pediatric problems, but also for pediatric hematologists who find that subspecialization makes keeping up with advances in other fields a constant challenge.

The reviews in this series include the following:

- Sarah H. O'Brien and Ayesha Zia, "Hemostatic and thrombotic disorders in the pediatric patient"
- Rachael F. Grace and Michele P. Lambert, "An update on pediatric ITP: differentiating primary ITP, IPD, and PID"
- Inderjeet Dokal, Hemanth Tummala, and Tom Vulliamy, "Inherited bone marrow failure in the pediatric patient"
- Patrick G. Gallagher, "Anemia in the pediatric patient"

The specific challenges of managing hemostatic and thrombotic disorders in the pediatric age group are described by O'Brien and Zia, who not only review significant advances in the field, but also issue a "call to arms" directed toward the urgent need to improve our understanding of pediatric thrombo-embolism. This, they argue, is essential if we are to minimize the prevalence, severity, and sequelae of this often-preventable cause of premature death and disability in infants and children.

A particular difficulty common to many hematologic disorders in childhood is identifying the occasional child where a seemingly common problem (such as anemia or thrombocytopenia) is the first sign of a rare inherited syndrome. This is discussed by Grace and Lambert in their review of ITP, in which they highlight the importance of differentiating ITP from other thrombocytopenic disorders, including IPD and PID. Similar diagnostic dilemmas are intrinsic to the increasing number of inherited bone marrow failure syndromes presenting in children, as discussed by Dokal, Tummala, and Vulliamy, who point out that research on these syndromes has been key to understanding fundamental biological pathways, such as DNA repair (Fanconi anemia genes), telomere maintenance (dyskeratosis congenita genes), and ribosome biogenesis (Shwachman-Diamond syndrome and Diamond-Blackfan anemia genes).

In stark contrast to these rare disorders, anemia is estimated to affect approximately a quarter of the world's population, almost 2 billion people, and is a particular problem in infants and children. As with the other reviews in this series, the challenge faced by hematologists is often knowing when to investigate atypical cases and the most appropriate investigations to use. In the final review of the series, Gallagher describes a stepwise approach to the diagnosis of anemia in infancy and childhood, including the use of next-generation sequencing, and summarizes the exciting advances in management both in novel therapeutics and population-based approaches to nutritional anemias.

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