Guest Editorial: Inherited bone marrow failure and myelodysplasia in children: how much do we know about pediatric diseases?

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Apart from acute lymphoblastic leukemia, pediatric diseases may not be well recognized by general hematologists focused on adults. However, several important diseases are manifested from infancy in the earliest stages of the human lifespan. Particular attention should be paid to inherited bone marrow failure syndromes (IBMFs) and myelodysplasia in children, as such diseases often remain latent until adulthood.

In the Progress in Hematology series in this issue of the *International Journal of Hematology*, four experts describe recent advances in pediatric bone marrow failure and myelodysplasia. Dr. Daisuke Hasegawa begins with a discussion of the state of knowledge and controversies regarding refractory cytopenia of childhood (RCC), the most common type of pediatric myelodysplasia. This classification was first proposed by a European group and later endorsed by the World Health Organisation. Dr. Hasegawa provides an overview of disease characteristics enhanced by the availability of data from the renowned central review system of the Japanese Society of Pediatric Hematology–Oncology.

Next, Dr. Satoshi Saida proposes an evolutionary model of myeloid leukemia from transient abnormal myelopoiesis (TAM) in children with Down syndrome. He describes the typical multistep leukemogenesis process in detail, based on his labor-intensive experiments using xenograft models.

Third, Dr. Jean Hugues Dalle explains the current recommendations for hematopoietic stem cell transplantation (HSCT) in children with IMBFS. IMBFS consists

of a variety of diseases including Fanconi anemia, severe congenital neutropenia, Shwachman–Diamond syndrome, Diamond Blackfan anemia, congenital amegakaryocytic thrombocytopenia, and dyskeratosis congenita. This is a rapidly expanding field, and new associated gene defects are reported every year for each form of IBMFS. Indications and procedures for HSCT vary by condition. Dr. Dalle provides a wonderful synthesis of this broad and challenging field.

Finally, Dr. Hoon Kook describes the current consensus regarding the management of childhood acquired aplastic anemia. The roles of immunosuppressive therapy and HSCT for this disease category continue to evolve in dramatic fashion, and Dr. Kook clearly addresses this issue incorporating data from the Korean Society of Pediatric Hematology–Oncology. This will doubtless represent highly useful practical information for many readers of this journal.

I would like to conclude my Guest Editorial by thanking Drs. Kazuma Ohyashiki and Toshio Kitamura for giving us the valuable opportunity to contribute to this series.

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