Current status of treatment for aplastic anemia in Japan

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Aplastic anemia (AA) is a rare disease in which pancytopenia is caused by an immune attack on hematopoietic stem cells. The prevalence of AA in Japan is estimated to be approximately 1 in 10,000. In Japan, guidelines have been developed since 2005 and revised every few years. Recently, however, a revision has been made.

Characteristics of AA treatment in Japan include the following: publicly subsidized medical costs as an intractable disease; use of only rabbit—not horse—antithymocyte globulin (ATG) is permitted; both eltrombopag (EPAG) and romiplostim can be used as thrombopoietin receptor agonist (TPO-RA) preparations; paroxysmal nocturnal hemoglobinuria blood cell levels can be measured at all facilities; and other factors. Horse ATG was recently approved by the relevant health authorities and will be available in the near future.

Romiplostim is not commonly used for AA in the West. In Japan, patients refractory to immunosuppressive therapy (IST) or EPAG have been switched from EPAG to romiplostim with good results. The revised guidelines now specify switching to romiplostim in refractory cases. In severe cases, allogeneic transplantation is the first choice when a human-leukocyte antigen-matched allogeneic donor is available. However, considering the improved outcomes with TPO-RA and IST, and the risks associated with allogeneic transplantation, IST may be considered as the first choice, even in younger patients.

In the present issue, we explain the revision of the Japanese guidelines and the current status of AA treatment in Japan.