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CHUGAI PHARMACEUTICAL CO., LTD.
Roche Group

Zenyaku Kogyo Co., Ltd.
Chugai Pharmaceutical Co., Ltd.

Anti-CD20 Monoclonal Antibody Rituxan® Approved for Treatment of Chronic Idiopathic Thrombocytopenic Purpura in Children

TOKYO, November 22, 2024 -- Zenyaku Kogyo Co., Ltd. (Japanese-only website) and Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced today that Zenyaku obtained regulatory approval from the Ministry of Health, Labour and Welfare (MHLW), for an additional dosage and administration of an anti-CD20 monoclonal antibody Rituxan® intravenous injection 100 mg and 500 mg [generic name: rituximab (genetical recombination)] (hereafter, "Rituxan"), which is co-marketed by both companies, for "chronic idiopathic thrombocytopenic purpura[*1] in children." (Idiopathic Thrombocytopenic Purpura: ITP)

Chronic ITP had previously only been approved for use in adults with specified dosage and administration, and its use in children had not been approved. The Japanese Society of Pediatric Hematology/Oncology requested the addition of dosage and administration for Rituxan for "chronic ITP in children." It was evaluated that this request qualified for a public knowledge-based application at the "58th evaluation committee on unapproved or off-labeled drugs with high medical needs" held on March 22, 2024. It was officially decided that a public knowledge-based application could be submitted at the "Pharmaceutical Affairs Council's First Committee on Drugs" held on April 26, 2024. In response to this, Zenyaku submitted a public knowledge-based application for the addition of dosage and administration on May 24, 2024, and obtained approval.

ITP is an autoimmune disease in which autoantibodies against platelet membrane proteins are expressed[1) 2) 3)], leading to thrombocytopenia due to platelet destruction and impaired production. It is recognized as a designated intractable disease (designated intractable disease 63) by the national government. The etiology of ITP is unknown, and the mechanism of autoantibody production has not been clearly elucidated.
Many newly diagnosed pediatric ITP patients often exhibit severe thrombocytopenia. However, serious bleeding such as intracranial hemorrhage is rare[4) 5) 6)], and often resolve spontaneously. It is estimated that 30-56%[7) 8) 9)] of cases require treatment. On the other hand, some cases may show resistance to primary treatments such as corticosteroids or intravenous immunoglobulin therapy[10)]. Both domestic and international clinical guidelines[10) 11) 12)] recommend Rituxan as one of the treatment options for such pediatric ITP patients.

Rituxan is an anti-CD20 monoclonal antibody that specifically binds to CD20, a protein expressed on B cells, excluding hematopoietic stem cells and plasma cells. It attacks target B cells using the immune system equipped with the human body, and damages cells. The influence

of B cells has been suggested as a pathogenic factor in ITP[13) 14) 15)], and by eliminating B cells with Rituxan, therapeutic effects are expected for chronic ITP that shows resistance to primary treatments.

Zenyaku and Chugai will continue working closely together so that Rituxan can further contribute to the treatment of chronic ITP not only in adults but also in children.

*1 Idiopathic thrombocytopenic purpura is considered an autoimmune disease targeting platelets, and it has also been referred to as "immune thrombocytopenia" in recent years.

*2 Approved dosage and administration
・Chronic idiopathic thrombocytopenic purpura
The usual dose is 375 mg/m^2 of rituximab (genetical recombination) administered as an intravenous infusion once weekly for four weeks.

*3 Rituxan's indication for chronic ITP was initially approved for adults in June 2017, and now additional approval has been obtained for use in children.

Sources

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