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# Commencement of R&D of Gene Therapy Product for Hemophilia B

Mitsubishi Tanabe Pharma Corporation (Head Office: Osaka; President & Representative Director, CEO: Masayuki Mitsuka) and Jichi Medical University (Location: Shimotsuke-shi, Tochigi-ken; President: Ryozo Nagai), will commence R&D of gene therapy drug (gene therapy product) for hemophilia B in Japan.

In October 2018, the R&D project, which Mitsubishi Tanabe Pharma proposed as the representative organization with the title of "R&D of gene therapy product for hemophilia B", was selected in the FY 2018 Cyclic Innovation for Clinical Empowerment (CiCLE) program of Japan Agency for Medical Research and Development (AMED). In August 2019, Mitsubishi Tanabe Pharma concluded a consignment R&D agreement with AMED.

Hemophilia[*1] is a congenital bleeding disorder resulting from genetic abnormality in a blood coagulation factor. The blood coagulation factors that coagulate the blood during hemorrhage are lacking or insufficient, and consequently when there is a hemorrhage due to a wound, injury, etc., long time is required until the bleeding stops, and in some cases the bleeding is very severe. In hemophilia A, coagulation factor VIII, one of the blood coagulation factors, is reduced/insufficient, while in hemophilia B, coagulation factor IX is reduced/insufficient.

This R&D initiative of gene therapy product will focus on using the adeno-associated virus (AAV) vector[*2] as a gene carrier. The product will be administered to patients with hemophilia B, and genes producing coagulation factor IX will be incorporated into the cells of patients, resulting in production of functional coagulation factors. If this treatment method is established, a single administration of the gene therapy product will enable patients to break away from regular coagulation factor IX replacement therapy.

Moving forward, with the support of AMED, Mitsubishi Tanabe Pharma and Jichi Medical University will work together to advance R&D targeting the creation of gene therapy product for hemophilia B. In addition, the partners will aim to extend the gene therapy technologies obtained through this R&D to the treatment of other diseases caused by genetic abnormalities in the future.

**For further information:**

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***1 Hemophilia**

Hemophilia is a disease in which the coagulation factors that coagulate the blood during hemorrhage are congenitally lacking or insufficient. Consequently, when there is a hemorrhage due to a wound, injury, etc. long time is required until the bleeding stops.

In hemophilia A, coagulation factor VIII, one of the coagulation factors, is reduced/insufficient, and in hemophilia B coagulation factor IX is reduced/insufficient.
As of May 31, 2018, the number of reported hemophilia patients in Japan was 8,751. Of these, reports indicated there were 5,301 hemophilia A patients and 1,156 hemophilia B patients. (From Nationwide Survey on Coagulation Disorders 2018)

The basic treatment for hemophilia is replacement therapy, in which coagulation factors are periodically supplied through injections. The treatment agents in replacement therapy are coagulation factor VIII preparations for hemophilia A and coagulation factor IX preparations for hemophilia B.

***2 Adeno associated virus (AAV) vector**

The adeno associated virus (AAV) is a naturally occurring virus that is not pathogenic for humans. The AAV vector is a vector (carrier) produced through modification of this virus. The use of this vector to introduce genes into cells is drawing attention as a promising technology for gene therapy.