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China approves first hemophilia B gene therapy

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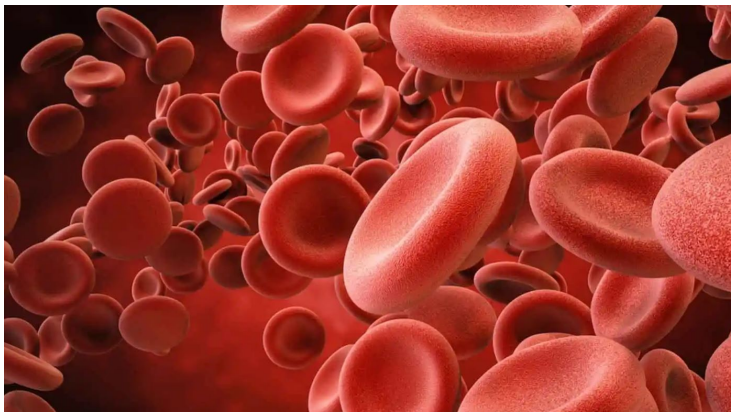
Contributing to the high-quality development of China's rare disease industry



Belief BioMed (BBM) and Takeda China have jointly announced that BBM-H901 (generic name: Dalnacogene Ponparvovec Injection), has been officially approved by the National Medical Products Administration (NMPA) for the treatment of adult patients with moderate to severe hemophilia B (congenital coagulation factor IX deficiency).

As the first approved hemophilia B gene therapy in China, BBM-H901 is developed and manufactured by BBM, and Takeda China is responsible for its commercialisation in mainland China, Hong Kong and Macau.

The two parties will integrate their respective resource advantages to accelerate the provision of this breakthrough gene therapy to patients and jointly open up a new landscape in the field of hemophilia B treatment.

Hemophilia B is an inherited bleeding disorder caused by the deficiency of factor IX (FIX). For a long time, patients can only rely on prothrombin complex concentrate (PCC) or FIX as a replacement therapy. Persistent and frequent bleeding can easily lead to damage of joint structure and function, resulting in a high disability rate. This not only brings great physical pain and inconvenience to patients, but also comes with the risk of infection, blood clots, etc. The high medical treatment costs also bring a heavy and constant economic burden on the patient's family.

BBM-H901, based on a recombinant adeno-associated virus (rAAV) vector, can deliver the optimised human coagulation FIX gene into liver cells of patients. Then, coagulation FIX is continuously expressed and secreted into the bloodstream using the host cell gene transcription system, thereby promoting coagulation.