Gene therapy for hemophilia B

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Dear Editor,

I wish to report that the first gene therapy procedure in Italy for the treatment of severe or moderately severe hemophilia B has been successfully administered at the Policlinico of Milan.

Hemophilia B is a rare, inherited bleeding disorder caused by a deficiency of coagulation factor IX.[1] The therapy, currently indicated only for adult patients, was performed at the Hemophilia Center of the Policlinico of Milan, directed by Flora Peyvandi. The Center is recognized nationally and internationally as a reference facility for congenital bleeding disorders and serves as the Hub Center for the Lombardy Region's Network for Congenital Bleeding Disorders (MEC).

The treatment consists of a single, non-repeatable infusion designed to enable endogenous production of factor IX, thereby markedly reducing the need for the periodic infusions required by conventional replacement therapies.

Hemophilia B is characterized by a hereditary deficiency of factor IX, an essential protein in the coagulation cascade. Affected individuals exhibit a pronounced tendency to develop spontaneous bleeding or hemorrhages triggered by minimal trauma, with associated risks of chronic pain, progressive joint damage, and substantial limitations in daily activities.[2]

The Policlinico of Milan has also performed Italian gene therapy procedures for hemophilia A and has actively participated in international research efforts on rare coagulation disorders, integrating clinical care, research, and training.[3,4]

According to Peyvandi, for the first time, a treatment is available that is capable of enabling patients to produce adequate levels of factor IX and to lessen the burden of the disease on everyday life. This achievement reflects the collaborative work of clinical teams, institutions, and patient associations.

This milestone represents a step forward in our mission to provide equitable access to the most advanced therapeutic options available.

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