Mucopolysaccharide IV-A Deficiency (Morquio Disease),

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Technology description

Title:

Proteins with an attached short peptide of acidic amino acids

Category:

Therapeutic

NCS:

Summary:

This invention comprises a fusion protein comprising enzyme N-acetylgalactosamine-6-sulfate sulfatase and a short peptide consisting of 4-15 acidic amino acids attached to the enzyme on its N-terminal side, a pharmaceutical composition containing the fusion protein, and a method for treatment of type A Morquio disease using the fusion protein. Compared with the native enzyme protein, the fusion protein exhibits higher transferability to bone tissues and improved, higher stability in the blood. Type A Morquio disease is an autosomal recessive genetic disease caused by an anomaly in the gene for a lysosomal enzyme, N-acetylgalactosamine-6-sulfate sulfatase (hereinafter referred to as GALNS) and is classified as type IV A mucopolysaccharidosis. GALNS is an enzyme that hydrolyses the sulfate groups of chondroitin-6-sulfate and keratan sulfate, which are species of GAG, and the deficiency of the enzyme causes intra-tissue deposition of GAG and its increased excretion in the urine. One of the clinical characteristics of Type A Morquio disease is bone dysplasia, and thus short statute, scoliokyphasis, brevicollis, coxa valga, and articular hyperextension have been reported to occur. No effective remedy is currently available for type A Morquio disease, and bone marrow transplantation provides no more than a marginal improvement of osteopathy. Thus, most of its treatment is addressed to symptomatic therapy or control of symptoms, like orthopaedic treatment to prevent dislocation in upper cervical vertebrae. On the other hand, as main symptoms are localized in the bone and joints in type A Morquio disease, it is expected that the quality of life of the patients could be greatly improved if their osteopathy is alleviated.

Institution

Saint Louis University

Inventors

Shunji Tomatsu
Assistant Research Professor
Pediatrics
Kazuo Okamura
Chief Researcher
William Sly
Professor and Chair
Biochemistry and Molecular Biology
Shuichi Miyaura
Manager
Research Lab