

Clinigen K.K., GC Pharma sign pact to market Hunterase ICV

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Hunterase (Idursulfase-beta) ICV, a human recombinant iduronate-2-sulfatase (IDS) used in enzyme replacement therapy for the treatment of Hunter syndrome



Clinigen K.K. and GC Pharma have recently announced an exclusive licensing agreement in Japan to commercialize Hunterase (Idursulfase-beta) ICV, a human recombinant iduronate-2-sulfatase (IDS) used in enzyme replacement therapy for the treatment of Hunter syndrome. Used as one of the methods for Hunter syndrome treatment, intravenous injection does not penetrate the blood brain barrier in clinically adequate amounts.

Hunterase (Idursulfase-beta) ICV developed by GC Pharma is delivered directly to cerebral ventricles by intracerebroventricular (ICV) administration, in order to reach the cells of the brain and central nervous system.

Hunterase (Idursulfase-beta) ICV is expected to meet the unmet needs of severe patients in improving their quality of life, as a method that can achieve what previous intravenous injection could not. The Phase 1/2 clinical trial conducted as an investigator-initiated trial by Prof. Torayuki Okuyama in National Center for Child Health and Development in Japan showed a significant decrease in Heparan sulfate which causes mental retardation.