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Miglustat

National Cancer Institute

Source

National Cancer Institute. *Miglustat*. NCI Thesaurus. Code C1222.

A synthetic, N-alkylated imino analogue of D-glucose. Miglustat competitively and reversibly binds to and inhibits the activity of UDP-glucose ceramide glucosyltransferase, which catalyzes the initial step in the synthesis of glycosphingolipids (GSL). This leads to a decrease in the production of glycosphingolipid, which has important roles in various cellular processes. Miglustat can be used in substrate reduction therapy in diseases in which the enzyme glucocerebrosidase, that is responsible for the breakdown of GSL, is deficient.