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Potential cure for congenital hyperinsulinism found

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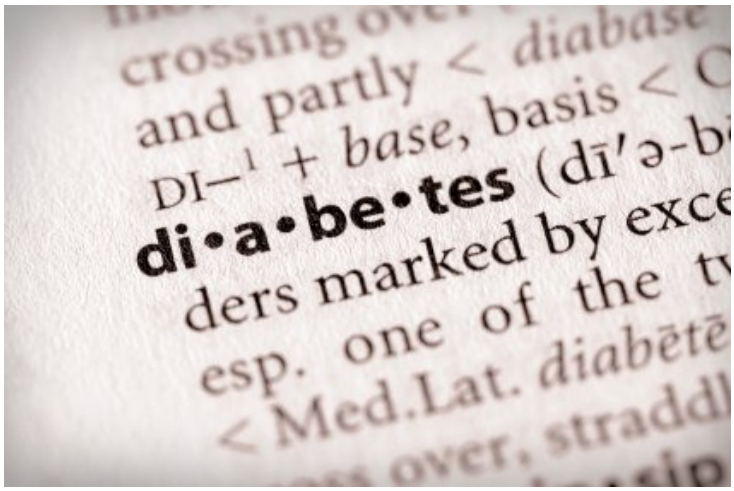
Study finds potential cure for congenital hyperinsulinism



Singapore: A pilot study by the Children's Hospital of Philadelphia in adolescents and adults found that an investigational drug shows promise as the first potential medical treatment for children with the severest type of congenital hyperinsulinism, a rare but potentially devastating disease in which gene mutations cause insulin levels to become dangerously high. The study has been published ahead of print in the journal *Diabetes*.

Dr Diva D De Leon, study leader and pediatric endocrinologist, The Children's Hospital of Philadelphia, said that, "There is currently no effective medicine for children with the most common and most severe form of hyperinsulinism. Our new research shows that this investigational drug, a peptide called exendin-(9-39), controls blood sugar levels in people, a very promising result."

In congenital hyperinsulinism (HI), which is a disease more prevalent in Ashkenazic Jews, mutations disrupt the insulin-secreting beta cells in the pancreas. The standard treatment for some forms of congenital HI is diazoxide, a drug that controls insulin secretion by opening potassium channels in beta cells. However, this drug does not work in the most common types of HI, in which mutations prevent these potassium channels from forming.

The current pilot study included nine subjects, aged 15-to-47 years old, who had hyperinsulinism caused by mutations in potassium channels. None were being treated for HI at the time of the study, but all were at risk of hypoglycemia during periods of fasting.
In all nine subjects, the drug controlled blood glucose levels during fasting. Exendin also controlled insulin secretion in cell studies of beta cells taken from newborns with HI. The current research did not focus on the biological mechanisms that occurred, but De Leon said the results are encouraging enough to progress to a clinical study in children with HI over the next year.

Financial support for this study came from the National Institutes of Health, the Lester and Liesel Baker Foundation, and the Clifford and Katherine Goldsmith Foundation. Dr De Leon's co-authors, all from Children's Hospital, were Dr Charles A Stanley, Dr Andrew C Calabria, Dr Changhong Li, and Mr Paul R Gallagher. In addition to their positions at Children's Hospital, Dr De Leon, Dr Stanley and Dr Li also are in the Perelman school of medicine, at the University of Pennsylvania.