

## In lab, drug-on-the-cob fights rare disease

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Biologists in Canada have made a medical enzyme using geneticallyengineered corn, a feat that could one day slash the cost of treating a lifethreatening inherited disease, a journal reported on Tuesday.

Inserting a section of <u>DNA code</u> into maize seed caused them to make alpha-L-iduronidase in the endosperm, a nutritive tissue in the <u>corn</u> kernel.

Alpha-L-iduronidase breaks down <u>sugar molecules</u> and is deficient in people with mucopolysaccheridosis I (MPS 1).

This is a so-called lysosomal storage disorder, in which sugary debris builds up in cells, damaging tissue in the heart, eyes, skeleton and brain.

Without replacement enzymes, sufferers of MPS 1 often die in childhood.

Until now, the therapy has been produced by coaxing cultures of cells taken from the ovaries of Chinese hamsters, and is hugely expensive.

The existing drug for MPS 1, laronidase (marketed as Aldurazyme) costs around \$300,000 annually for children and \$1 million for adults.

The research, led by Allison Kermode at Simon Fraser University in Burnaby, British Columbia, is published in the journal *Nature Communications*.



The results amount to "proof of concept" for making the enzyme in laboratory conditions, the team say.

Further work would be needed to scale up volume, but this should not be too much of a problem and conventional techniques could be used, they add.

Severe MPS I occurs in approximately in one in 100,000 newborns, according to the website Genetics Home Reference, which is supported by the US <u>health authorities</u>.

A milder form, called attenuated MPS I, occurs in about one in 500,000 births.

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