Gene control, delivered directly to the brain , could help cure diseases

A biotech company called <u>Alnylam</u> announced today that a small clinical trial for a genetic therapy based on RNA interference, or RNAi, suggests that the technique can have a powerful effect on its target gene. The therapeutic effect lasted for over a month with just one dose. The company is also working with a medical device maker, Medtronic, on a way to deliver RNAi treatment directly to the brain, in order to treat the degenerative brain disease Huntington's.

The patients in the trial have a genetic disorder that originates in the liver and leads to the buildup of protein deposits in many organs. <u>Alnylam</u>, a Cambridge, Massachusetts-based company, says its RNAi therapeutic, given at its highest dose, reduces the amount of the faulty protein that spurs the disease by almost 94 percent.

View the original article here: Gene Control, Delivered Directly to the Brain – Technology Review