Dementia treatment shows promise in CRISPR lab tests

Salk Institute scientists reported [March 15] that they've invented a new version of the technology that works on RNA, combining CRISPR/Cas9's precision with the ability to turn its effects on and off at will. And because it leaves DNA untouched, it's safer.

A form of dementia may eventually be treatable with the technology, called CasRx. But much more work needs to be done before it can be tried in patients.

Working in cultures of brain cells, the scientists corrected a protein imbalance that causes frontotemporal dementia with parkinsonism. They are preparing to test CasRx in live animals, said Patrick Hsu, a Salk researcher and study leader.

If that work goes well, the method could be tried in patients. Moreover, CasRx could become a general platform technology for treating genetic diseases, the study said.

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Because the new CRISPR method leaves the DNA intact, its effects aren't permanent, Hsu said. RNA is continually generated. So withdrawing the treatment allows the RNA function to return to its previous state.

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Perhaps even more important is the novel method the scientists used to discover this new family of CRISPR, [scientist Floyd] Romesberg said. Their approach could unlock a trove of new genetic engineering tools.

Read full, original post: Salk scientists adapt powerful gene-editing system to correct dementia in lab