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Study identifies two chemicals that could lead to new drugs for genetic disorders

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UCLA scientists have identified two chemicals that convince cells to ignore premature signals to stop producing important proteins. Published in the Sept. 28 edition of the *Journal of Experimental Medicine*, the findings could lead to new medications for genetic diseases, such as cancer and muscular dystrophy, that are sparked by missing proteins.

"When DNA changes, such as nonsense mutations, occur in the middle rather than the end of a protein-producing signal, they act like a stop sign that tells the cell to prematurely interrupt protein synthesis," explained Dr. Richard Gatti, professor of pathology and laboratory medicine and human genetics at the David Geffen School of Medicine at UCLA. "These nonsense mutations cause the loss of vital proteins that can lead to deadly genetic disorders."

Gatti's lab specializes in studying ataxia-telangiectasia (A-T), a progressive neurological disease that strikes young children, often killing them by their late teens or early 20s.

For four years, the UCLA Molecular Shared Screening Resources Center of the campus' California NanoSystems Institute has screened 35,000 chemicals, searching for those that ignore premature stop signals.

First author Liutao Du developed the screening technology in Gatti's laboratory.

"Of the dozens of active chemicals we discovered, only two were linked

to the appearance and function of ATM, the protein missing from the cells of children with A-T," said Du. "These two chemicals also induced the production of dystrophin, a protein that is missing in the cells of mice with a nonsense mutation in the muscular dystrophy gene."

The UCLA team is optimistic that their discovery will aid pharmaceutical companies in creating drugs that correct genetic disorders caused by nonsense mutations. This could affect one in five patients with most genetic diseases, including hundreds of thousands of people suffering from incurable diseases. Because nonsense mutations can lead to cancer, such drugs may also find uses in cancer treatment.

Source: University of California - Los Angeles

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