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Molecules which induce targeted protein relocalization for therapeutic applications

In many diseases, such as cancers and neurodegenerative disorders, the mislocalization of proteins can disrupt cellular functions and drive disease progression. Traditional therapeutic approaches often fail to address these specific localization issues. To tackle this problem, the researchers at Stanford have developed bifunctional compounds, termed Targeted Relocalization Activating Molecules (TRAMs) which couple the trafficking of a target protein to the trafficking of a shuttle protein, which have strong native localization sequences.

Using this strategy of hijacking protein trafficking mechanisms, the researchers have successfully demonstrated the potential to mitigate disease phenotypes, such as reducing stress granules and slowing axonal degeneration. This approach enables gain-of-function pharmacology through relocalization rewiring.

Stage of Development
Proof of concept including in *in vitro* work and primary neurons.

Applications

- Cancer
- Neurodegenerative diseases
- Metabolic diseases

Advantages

- Specifically targets protein redistribution
- Allows control over subcellular location

- Enables gain-of-function pharmacology

Publications

- Ng, C.S.C., Liu, A., Cui, B. et al. Targeted protein relocalization via protein transport coupling. *Nature* (2024). https://doi.org/10.1038/s41586-024-07950-8
- McClellan, Rebecca. Bringing lost proteins back home Stanford News. September 20, 2024.
- Christine S. C. Ng, Aofei Liu, Bianxiao Cui, Steven M. Banik (2023). Targeted Protein Relocalization via Protein Transport Coupling. bioRxiv, 2023-10. In press at *Nature*.

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