DR. NUTTER'S RESEARCH

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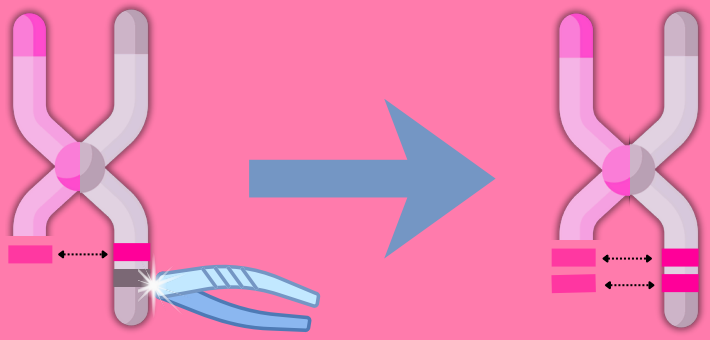
Aim 1

Method:
Analyze patient-derived neural cells to identify which deleted X-linked genes naturally escape from the inactive X-chromosome.

Goal:
Reveal which genes are already compensated for and which may still require targeted treatment.

Natural Escape vs. CRISPR-Induced Reactivation



Arrows show how escaped genes (pink) compensate for deleted ones. The CRISPR tool (blue tweezers) reactivates silenced genes (gray → pink), restoring the genetic information lost due to the deletion.

Aim 2

Method:
Apply CRISPR-based gene activators and X-inactivation blockers to test whether silenced genes in X deletion cells can be reactivated.

Goal:
Restore gene expression in order to reduce disease symptoms and support normal cellular function.

Background Info

In females, each cell contains two X-chromosomes, but one is largely silenced through a process called X-chromosome inactivation (XCI). However, some genes "escape" this silencing and are expressed from both X chromosomes. These "escapee" genes can serve as backups when genes on one X chromosome are missing or dysfunctional, a mechanism that becomes especially important in X-linked genetic disorders. Fragile X Syndrome (FXS), a well-studied X-linked condition caused by silencing of the FMR1 gene, has become a model for exploring how reactivation of silenced genes might restore function.

TRAORDINARY JOY!