RESEARCH / DR. CLELLAND STUDY BRIEF

Mapping delivery to the human brain

A proposed human CNS delivery-mapping study with Dr. Clelland's lab at UCSF

> **We can edit disease-linked genes in patient-derived cells. The harder step is getting treatments safely to the brain and spinal cord. [1, 2]**

The problem

Dr. Clelland's lab is developing CRISPR approaches for certain inherited forms of ALS, frontotemporal dementia and Alzheimer's disease. The blood-brain barrier complicates delivery to the central nervous system, and animal models often fail to predict human drug responses. [1, 2]

The idea

In the lab's proposed platform, a deceased donor whose circulation is physiologically maintained would receive many candidate delivery particles in a single experiment. DNA and RNA barcodes would let researchers see, about 48 hours later, where those particles traveled. Family consent and clinical oversight are part of the described approach. This is not an isolated brain kept alive outside the body. [1, 2]

What the map could show

The spinal cord is the main readout for central nervous system delivery because the brain is injured in brain death. The team also plans to map distribution elsewhere in the body. This is a proposed discovery platform, not a therapy, clinical result, or proof that any particle is safe in living patients. [1, 2]

Why this team

Dr. Clelland's lab works on gene editing and delivery; Dr. Neel Singhal leads the clinical team. The lab credits Dr. Doug Pet and Brendan Parent for ethics work. Dr. James Gardner's separate UCSF VITAL core supports access to donated human tissues, but the public lab description does not identify that core as operating this proposed study. [1, 3]

Why early support matters

The lab describes high-risk, high-reward delivery research that needs philanthropic support. Meredith's notes from her conversation with Dr. Clelland also describe difficulty funding projects before feasibility is established. The platform has not yet yielded the human delivery map described here. [1, 2, 4]

The promise is a better starting point for future research, not a cure in hand.