

## **Lay Summary**

**BRAIN UK Ref: 23/009**

### **SPCA in ALS**

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This project will develop a new treatment for amyotrophic lateral sclerosis (ALS)/motor neuron disease, addressing the dysfunction of a small part inside the nerve cell, named Golgi. The earliest event in motor neuron disease is malfunction of the Golgi but it has yet to be targeted with a treatment in ALS. The nerve cells responsible for muscle movement have a particular signature, named SPCA. The malfunction of SPCA disrupts the Golgi structure and leads to cell death. This proposal brings together our expertise on the nervous system, on the biology of SPCAs and in drug development to explore a new therapeutic direction for ALS. We hypothesize that targeting SPCA activity will correct Golgi dysfunction and provide therapeutic benefit in patients with ALS. In order to do this, we will first demonstrate the exact localization of SPCA in postmortem tissue from patients with ALS.