RNA base editor: Efficient platform for Usher Syndrome treatment

Market: Point mutations, especially G>A are one of the most common mutations in Inherited Retinal Diseases (IRDs). Usher Syndrome (US) is an example and is characterised by vision and hearing impairment from birth. The incidence of 1 in 6,000.

Current Solution: There is currently no approved treatment to treat blindness associated with US.

Problem:

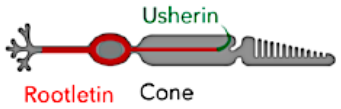
- Approx. 45% of genes associated with IRDs cannot be easily incorporated into generic vector platforms due to large gene size (US is an example)
- Current therapies are mainly designed for a specific disease with limited ability to repurpose readily

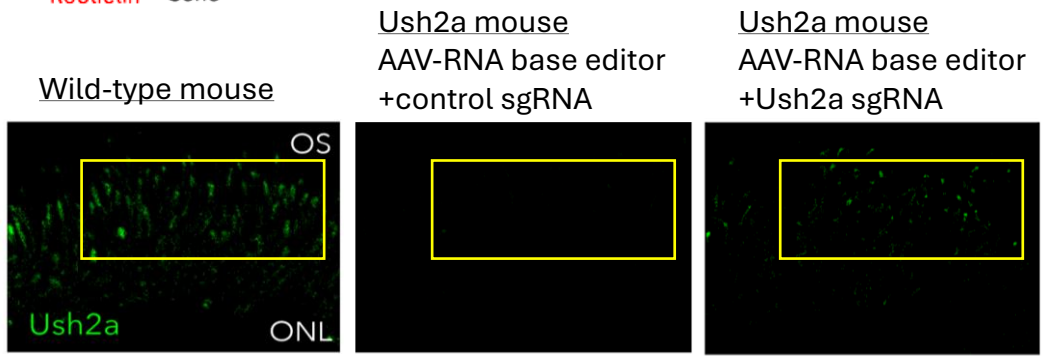
Our therapy is a **patented RNA editing base editor platform** able to efficiently correct G>A mutations with high specificity. Advantages include:

- Effective solution for *ca.* 24% of all IRDs associated with point mutations
- Once-off treatment

In-vivo POC in Ush2a mouse model

A/Prof Rick Liu





Next milestones:

- Demonstrate POC in human retinal organoids with patient specific mutations
- Lead selection studies
- Demonstrate commercially viable manufacture of lead construct

Strategic partnership and investment opportunities to accelerate validation. Contact: stan@cera.org.au