Variants in the KCNT1 gene cause severe childhood epilepsies. At the molecular level, these gene variants lead to abnormally rapid potassium channel activity, which causes the potassium channel encoded by the KCNT1 gene to be overactive. New drugs and gene therapies have been developed to inhibit the channel and show promise on KCNT1 mice at Virginia Tech. We expect future repurposing trials to take place in the clinic.

Sometimes treating the wrong part of the brain is counterproductive, leading to ineffective therapy and additional suffering. This is why it is crucial to understand which cells to treat for maximum benefit. By helping scientists study individual brain cells from mice with KCNT1-related epilepsy, we've learned that restricting treatment to just these cells can improve both the efficacy and safety of potential therapies. Despite these promising findings, whether such restricted treatments are applicable in people remains unknown.

Following a similar logic, we are also investigating whether restricting treatment to these cells could improve outcomes in people. We hypothesize that targeting only the most severely affected cells will lead to fewer side effects and more effective treatment, making it possible to increase the dosage and the likelihood of success.

To test these hypotheses, we are currently studying the therapeutic impact of restricting treatment to different cell types in a mouse model of KCNT1 epilepsy. Our findings will help us understand how to optimize treatments for people with KCNT1 epilepsy.

As we continue to explore the potential benefits of restricting treatments, we are also working on developing more effective ways to select the right cells to treat. This involves identifying the specific features of cells that contribute to seizures and developing methods to directly image and target these cells in vivo.

We believe that by targeting the right cells, we can significantly improve treatment outcomes for people with KCNT1 epilepsy. Our goal is to develop a precision medicine approach that can be tailored to each individual's unique needs, leading to a brighter future for people affected by this condition.