Researchers at the University of South Florida have developed a rat model of Angelman Syndrome that is useful in research on the disease as well as for pre-clinical testing of potential therapeutics.

Angelman Syndrome (AS) is a devastating neurological disorder which affects 1 in every 15,000 people. AS presents with ataxia, frequent smiling, laughter, lack of speech, and severe, debilitating seizures. Epilepsy in AS is often refractory to many prescribed medications, and frequently involves many seizure types. Furthermore, chronic, intractable epilepsy is shown to cause hippocampal damage and is associated with cognitive decline.

Nearly all cases of AS result from the disruption of a single gene, UBE3A. Mouse models have been developed using different strategies to disrupt the UBE3A gene. While these mouse models effectively disrupt maternal UBE3A gene expression, there remain various advantages and disadvantages to these models.

Our scientists have developed a rat model of Angelman Syndrome that closely mimics the complete deletion of the UBE3A gene – containing region of chromosome 15 found in approximately 70% of Angelman cases. The rat model is completely UBE3A deficient with a genome lacking the entire UBE3A gene (including all isoforms and alternative promoters). This rat model may be used for basic research on the disease as well as applied research for pre-clinical testing of potential therapeutics.

**ADVANTAGES:**
- Lacks the entire UBE3A gene
- Removal of all isoforms
- Removal of all alternative promoters

**Complete Absence of Entire UBE3A Gene**

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