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Study of a Tau Antisense Oligonucleotide in Corticobasal Syndrome (STACS)

Is a novel genetic therapy for a specific type of dementia safe and tolerable?

Background
The tau protein helps maintain the structure of brain cells and transport nutrients within cells. In Alzheimer's and over twenty other brain diseases known as tauopathies, the shape of tau protein becomes modified or "misfolded," a change that may contribute to the accumulation of tau tangles (a hallmark of these diseases) and subsequent nerve cell damage.

Corticobasal syndrome (CBS) is a tauopathy that can cause changes in movement, language skills, and cognition. As tau and/or other proteins accumulate in the nerve cells, the cells become damaged and eventually die, causing affected parts of the brain to shrink.

Preliminary studies in animal models have shown that a compound called BIIB080 may reduce tau accumulation and nerve cell death. BIIB080 targets a gene named MAPT (microtubule-associated protein tau), which provides instructions for making the tau protein, and blocks its activity.

Research Plan
Dr. VandeVrede and colleagues will conduct a phase1a/2b clinical trial of BIIB080 in individuals with CBS. They will recruit 24 patients and treat them with either BIIB080 or a placebo. After six months, the researchers will assess the drug's safety, tolerability, and pharmacodynamics (how the drug is absorbed and eliminated by the body). The team will also measure changes in tau levels in samples of blood and cerebrospinal fluid (a biological fluid surrounding the brain and spinal cord), as well as potential cognitive and behavioral changes.

Impact
If successful, this study may support BIIB080 as a potential therapeutic strategy to delay the progression of corticobasal syndrome and other tauopathies.