GW Research initiated a clinical trial of cannabidiol (CBD, GWP42003-P) in Rett syndrome in May 2019.

GW42003-P (cannabidiol) is believed to affect several pharmacological pathways and may have the potential to modulate some of the pathophysiological mechanisms thought to underlie the neurobehavioral deficits present in Rett syndrome including excitatory/inhibitory neuronal imbalance, neuroinflammation and antioxidant dysfunction.

The aim of the clinical trial is to evaluate the safety and efficacy of GWP42003-P in reducing symptom severity in females, aged 2 to 18 years, with Rett syndrome. Symptom severity will be evaluated using caregiver and clinician measures that assess multiple symptoms and therefore reflect the overall condition. Measuring changes in seizures frequency or severity is not, per se, a key objective of the trial. Several safety outcomes such as side effects, vital signs, lab assessments and tests will be analyzed.

The effect of two doses will be compared with a placebo control across a 24-week treatment period. Patients completing the study will have the opportunity to take part in a 6-month extension study where all patients will receive GWP42003-P. In total, approximately 252 patients are expected to be included.

One of the eligibility requirements is for patients to refrain from taking medicinal cannabis, cannabinoid-based medications or cannabidiol oral solutions for at least 3 months prior to screening in the study. The full list of eligibility criteria can be found on clinicaltrials.gov [ClinicalTrials.gov Identifier: NCT03848832].

A trial site in Greenwood, SC is now recruiting.

Further US sites are expected to open from June 2019 onwards in New York, NY; Cincinnati, OH; Nashville, TN; Houston, TX; Chicago, IL; San Diego, CA; Birmingham, AL; Philadelphia, PA; Boston, MA; St Paul, MN; Aurora, CO; Baltimore, MD; St Louis, MO.

GW is looking forward to working with the Rett Community on this important study investigating the safety and efficacy of GWP42003-P in people with Rett Syndrome.

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