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Variant Pharmaceuticals Submits Application for U.S. Orphan Drug Designation for VAR 200 (2-Hydroxypropyl- β -Cyclodextrin) To Treat Focal Segmental Glomerulosclerosis (FSGS)

IND to be submitted early 2019, with phase 2a clinical trial initiation following IND acceptance

WESTON, Fla., Oct. 30, 2018 /PRNewswire/ -- Variant Pharmaceuticals, Inc. (Variant), a clinical stage orphan drug company developing first-in-class drugs for patients with rare diseases, announced today that it has filed an application with the FDA for orphan drug designation for VAR 200 (2-hydroxypropyl- β -cyclodextrin, or 2HP β CD) for treatment of FSGS. FSGS is a rare kidney disease affecting up to 80,000 people in the U.S. and up to 300,000 people worldwide.



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The Orphan Drug Act was enacted to establish federal government support for development of products for the diagnosis, prevention or treatment of rare diseases or conditions that affect fewer than 200,000 people in the United States. Achieving Orphan Drug Designation facilitates clinical development with assistance throughout the development process from FDA's Office of Orphan Products Development. This may include tax credits for certain

clinical research costs, exemption or waiver of filing fees, and seven-year marketing exclusivity following drug approval.

"Filing for orphan drug designation is an important step forward in our development plans for VAR 200," stated Stephen C. Glover, Variant's Co-founder, Chairman and Chief Executive Officer. "With no currently available disease-specific treatments approved for FSGS, a high percentage of patients progress to end-stage renal disease within 10 years, requiring dialysis and/or kidney transplant, which has a high recurrence rate. With the potential to delay FSGS progression demonstrated in our pre-clinical program, we are anxious to advance our development program for VAR 200 as efficiently and as quickly as possible. Achieving Orphan Drug Designation will support this goal."

About FSGS

FSGS, affecting up to 80,000 people in the U.S. and 300,000 people globally, is a progressive form of kidney disease associated with accumulation of cholesterol and lipids in the part of the kidneys that filters waste from the blood (glomeruli). Damage to the glomeruli causes protein from the blood to leak into the urine, a condition known as proteinuria. As the level of protein increases in the urine, kidney damage continues and patients develop a serious condition known as nephrotic syndrome. Symptoms of nephrotic syndrome include swelling, especially in the legs and around the eyes, and difficult-to-treat high blood pressure. As FSGS progresses, kidney function worsens. It has been reported that more than 35% of FSGS patients develop kidney failure within 10 years, requiring dialysis and ultimately kidney transplant to survive. Approximately 1,000 FSGS patients receive a kidney transplant each year.

About VAR 200

VAR 200, 2-hydroxypropyl- β -cyclodextrin (2HP β CD), is a phase 2a-ready asset intended to induce remission of proteinuria and delay disease progression in patients with FSGS. This is thought to be accomplished through trapping and removal of excess intracellular cholesterol and lipids in the kidneys. 2HP β CD has potential for treating other kidney conditions associated with the damaging effects of intracellular accumulation of cholesterol and lipids, including Alport Syndrome, a rare disease affecting up to 60,000 people in the U.S.

About Variant

Variant Pharmaceuticals, a clinical stage orphan drug company focusing on restoring health and transforming the lives of patients with rare diseases through innovation, was established in 2014, with the mission to become a leading orphan drug company. Our evolving product pipeline is targeted to the \$100+ billion orphan drug market. Our lead drug candidate is 2-hydroxypropyl- β -cyclodextrin (2HP β CD) for chronic treatment of two orphan indications, Focal Segmental Glomerulosclerosis (FSGS) and Alport Syndrome (AS), rare progressive forms of kidney disease.

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