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Variant Pharmaceuticals Completes Pre-IND Meeting with FDA on VAR 200 for Focal Segmental Glomerulosclerosis (FSGS), a Rare Kidney Disease

Clinical development to progress directly to phase 2a in adult FSGS patients

IND to be submitted H2-2018, with clinical trial initiation following IND acceptance

WESTON, Fla., April 16, 2018 /PRNewswire/ -- Variant Pharmaceuticals, Inc. (Variant), a clinical stage orphan drug company developing first-in-class drugs for patients with rare diseases, announced today receipt of FDA minutes from the March 12, 2018 Pre-IND meeting regarding development of VAR 200 (2-hydroxypropyl-β-cyclodextrin, or HPβCD) for treatment of Focal Segmental Glomerulosclerosis (FSGS) in adults. FSGS is a rare kidney disease affecting up to 80,000 people in the U.S. and up to 250,000 people worldwide.

"We are pleased that FDA concurred with our plans to progress directly into phase 2a clinical trials in adult patients with FSGS, which will shorten our development time significantly," 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."

"Based on our productive Pre-IND meeting with the FDA, we can confirm that the IND filing will not require any additional nonclinical data before proceeding directly to the phase 2a trial in FSGS adults. Likewise, we are working with FDA to establish parameters for clinical development of VAR 200 in pediatrics, and we have obtained a clearer understanding of our path forward for longer-term phase 2b/3 trials in adults," noted Mr. Glover.

VAR 200 has the potential to induce remission of proteinuria and delay FSGS progression, fulfilling an unmet need in this population.

About FSGS

FSGS, affecting up to 80,000 people in the U.S. and 250,000 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, or hypertension. As FSGS progresses, kidney function worsens. It has been reported that more than 35% of FSGS patients develop kidney failure, or end stage

kidney disease, 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 (HPβCD), is a phase 2a-ready asset intended to minimize or prevent kidney cell damage and maintain adequate kidney function in patients with FSGS. This is thought to be accomplished through trapping and removal of excess intracellular cholesterol and lipids. HPβCD 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., and Diabetic Kidney Disease, affecting up to 12 Million 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 orphan drug candidate is 2-hydroxypropyl-β-cyclodextrin (HPβCD) for chronic treatment of two orphan indications, Focal Segmental Glomerulosclerosis (FSGS) and Alport Syndrome (AS), rare progressive forms of kidney disease with an addressable market greater than $2 Billion.

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