August 10, 2017

Study Results Published in The Lancet Demonstrate Safety and Efficacy of Intrathecal VTS-270 in Niemann-Pick Disease Type C1

ROCKVILLE, Md., August 10, 2017 - Sucampo Pharmaceuticals, Inc. (Sucampo) (Nasdaq:SCMP), a global biopharmaceutical company, today announced the publication of results from a Phase 1/2a study of intrathecal administration of VTS-270, a 2-hydoxypropyl-β-cyclodextrin (HPβCD) under investigation for treatment of Niemann-Pick Disease Type C1 (NPC-1). The results of the study were published in the August 10, 2017 issue of *The Lancet* and can be found at http://www.thelancet.com/pdfs/journals/lancet/PIIS0140-6736(17)31465-4.pdf.

The open-label, dose-escalation phase 1/2a trial studied safety, tolerability and clinical efficacy of intrathecal administration of VTS-270 after 12 months and 18 months of treatment. The study, with data from 14 patients, demonstrated clinically meaningful reduction in signs and symptoms of disease progression as measured by the NPC Clinical Severity Scale (or NPC-SS, which looks at, among other domains, ambulation, fine motor ability, cognition, speech, memory and swallowing), compared to a natural history cohort. No serious adverse events were observed.

Additional details on the full study results are available at https://medicine.wustl.edu/?p=46079&preview=1&_ppp=45e1cd1a37 and https://www.nih.gov/news-events/news-releases/experimental-treatment-niemann-pick-disease-type-c1-appears-safe-effective.

NPC-1 is a rare, progressive and ultimately lethal genetic disorder affecting an estimated 2,000 to 3,000 patients globally. Effective treatment of NPC remains a high unmet need, with no approved products for patients in the U.S. VTS-270 has been granted orphan drug designation in both the U.S. and Europe.

About Sucampo Pharmaceuticals, Inc.

Sucampo Pharmaceuticals, Inc. is a biopharmaceutical company focused on the development and commercialization of highly specialized medicines. Sucampo has a late-stage pipeline of product candidates in clinical development for orphan disease areas, including VTS-270, a 2-hydroxypropyl-beta-cyclodextrin product with a specific compositional fingerprint that has been granted orphan designation in the U.S. and Europe and is in a pivotal Phase 2b/3 clinical trial for the treatment of Niemann-Pick Disease Type C-1, a rare progressive genetic disorder. VTS-270 also has been granted breakthrough therapy designation in the U.S. Sucampo has an exclusive option for the North American rights to CPP1-x/sulindac, which is in Phase 3 development for the

treatment of familial adenomatous polyposis and has been granted orphan drug designation in the U.S. The company has two marketed products - AMITIZA and RESCULA. For more information, please visit www.sucampo.com.

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