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New Study Published in PLOS ONE Advances Science Around VTS-270 for Treatment of Niemann-Pick Disease Type C1

ROCKVILLE, Md., April 18, 2017 (GLOBE NEWSWIRE) -- Sucampo Pharmaceuticals, Inc. (Sucampo) (NASDAQ:SCMP), a global biopharmaceutical company, today announced the publication of a new study in an independent scientific journal that confirms the unique composition of VTS-270, a 2-Hydroxypropyl-beta-cyclodextrin (HP β CD) product under investigation as a novel treatment for Niemann-Pick Disease Type C1 (NPC-1).

NPC-1 is a rare, progressive and ultimately lethal genetic disorder affecting an estimated 2,000-3,000 patients globally. The results were published today in an article entitled, "Characterization of hydroxypropyl-beta-cyclodextrins used in the treatment of Niemann-Pick Disease type C1," in *PLOS ONE*, the world's first multidisciplinary, Open Access journal, and are available at <http://journals.plos.org/plosone/article?id=10.1371/journal.pone.0175478>.

In this study, researchers demonstrated that VTS-270 has a low degree of consistently specific hydroxypropylation, or degree of substitution (DS), as well as a low level of doubly charged hetero-dimers as seen through an ion mobility false color representation, or heat map. The analysis was based on ion distribution and abundance profiles using mass spectrometry methodology as a means for assessing key molecular activity.

"We are continuing to advance the science around VTS-270 as a potential treatment for NPC," said Peter Kiener, D. Phil, Chief Scientific Officer of Sucampo. "These study results further support the specific compositional fingerprint and purity of VTS-270 that distinguishes it from other HP β CD mixtures. The defined and reproducible fingerprint is likely inextricably linked to the specific potential clinical efficacy and safety of VTS-270."

HP β CDs are complex mixtures of different species, and variations in production may lead to differences in composition including differing degrees of hydroxypropylation of the cyclodextrin ring, also known as degrees of substitution. The average degree of substitution is a critical characteristic of the complexity of HP β CDs mixtures, as it influences the ability to bind to other molecules, and affects the degree of aqueous solubility, both of which may ultimately influence the biological activity.

About Sucampo Pharmaceuticals, Inc.

Sucampo Pharmaceuticals, Inc. is focused on the development and commercialization of specialized medicines that meet major unmet medical needs of patients worldwide.

Sucampo has two marketed products – AMITIZA, its lead product, and RESCULA – and a late-stage pipeline of product candidates in clinical development for orphan disease areas. VTS-270 is a mixture of 2-hydroxypropyl-B-cyclodextrins with a specific compositional fingerprint that has been granted orphan designation in the U.S. and Europe and is in a pivotal Phase 2/3 clinical trial for the treatment of Niemann-Pick Disease Type C-1. Sucampo has an 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. A global company, Sucampo is headquartered in Rockville, Maryland, and has operations in Japan and Switzerland. For more information, please visit www.sucampo.com.

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