# UNITED STATES
# SECURITIES AND EXCHANGE COMMISSION

**Washington, D.C. 20549**

# FORM 8-K

**CURRENT REPORT**

**Pursuant to Section 13 or 15(d) of the**
**Securities Exchange Act of 1934**

Date of Report (Date of earliest event reported): **March 31, 2017**

# Sucampo Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

**Delaware**
(State or other jurisdiction of incorporation)

| **001-33609** | **30-0520478** |
|---|---|
| (Commission File No.) | (IRS Employer Identification No.) |

**805 King Farm Blvd, Suite 550**
**Rockville, Maryland 20850**
(Address of principal executive offices and zip code)

Registrant's telephone number, including area code: **(301) 961-3400**

(Former name or former address, if changed since last report.)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

[ ] Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

[ ] Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

[ ] Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

[ ] Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

**Item 1.01 Entry into a Material Definitive Agreement.**

On March 31, 2017, Sucampo Pharmaceuticals, Inc. (the "***Registrant***"), entered into an Agreement and Plan of Merger (the "***Merger Agreement***") with Vtesse Inc., a Delaware corporation ("***Vtesse***"), Saber Merger Sub, Inc., a Delaware corporation and wholly owned subsidiary of the Registrant ("***Transitory Subsidiary***"), and Fortis Advisors LLC, as representative of the holders of Vtesse equity (the "***Company Equityholder Representative***"). The Merger Agreement provides for Transitory Subsidiary to merge with and into Vtesse (the "***Merger***"), with Vtesse surviving as a wholly owned subsidiary of the Registrant, subject to the terms and conditions set forth in the Merger Agreement.

Pursuant to the Merger Agreement, and upon the terms and subject to the conditions thereof, at the closing, the Registrant is required to pay holders of Vtesse's capital stock and options to purchase Vtesse's common stock (collectively, the "***Company Equityholders***"), upfront consideration equal to approximately $200,000,000, consisting of, and subject to adjustment with respect to, the following (A) an amount in cash equal to (i) $170,000,000, plus (ii) all unrestricted cash and cash equivalents held by Vtesse at Closing, plus (iii) a portion of the aggregate exercise price of outstanding options (which amount shall be subsequently deducted from the amounts otherwise payable to the holders of such options), minus (iv) the amount by which the Vtesse net working capital is less than zero, minus (v) an amount set aside in escrow, minus (vi) the unpaid company transaction expenses, minus (vii) an amount set aside for expenses incurred by the Company Equityholder Representative and minus (viii) an amount equal to any indebtedness of Vtesse at closing, and (B) approximately 2,782,678 shares of Registrant's Class A Common Stock. The Company Equityholders receiving Registrant's Class A Common Stock are required to sign lock-up agreements that, among other things, provide for a lock-up period of three (3) months for all shares of Registrant's Class A Common Stock issued in the Merger.

The Registrant has also agreed to pay the Company Equityholders (A) contingent consideration based on mid-single-digit to double-digit royalties on global net sales of Vtesse's VTS-270 product, tiered based on increasing net sales levels, and (B) a share of net proceeds that may be generated from the monetization of any priority review voucher that may be granted to Vtesse in the future.

The Merger Agreement contains customary representations, warranties, covenants and indemnities of each of the Registrant and Vtesse. In addition, the Merger Agreement requires the Registrant to register for resale, after the applicable lock-up period, with the Securities and Exchange Commission all shares of its Class A Common Stock issued as part of the consideration payable in the Merger.

The foregoing summary is qualified in its entirety by reference to the Merger Agreement, which will be filed as an exhibit to the Registrant's Quarterly Report on Form 10-Q for the quarter ended March 31, 2017. The representations, warranties and covenants contained in the Merger Agreement were made only for the purposes of the Merger Agreement, were made as of specific dates, were made solely for the benefit of the parties to the Merger Agreement and may not have been intended to be statements of fact but, rather, as a method of allocating risk and governing the contractual rights and relationships among the parties to the Merger Agreement. The assertions embodied in those representations and warranties may be subject to important qualifications and limitations agreed to by the Registrant and Vtesse in connection with negotiating their respective terms. Moreover, the representations and warranties may be subject to a contractual standard of materiality that may be different from what may be viewed as material to stockholders of the Registrant. For the foregoing reasons, none of the Registrant's stockholders or any other person should rely on such representations and warranties, or any characterizations thereof, as statements of factual information at the time they were made or otherwise.

The Merger Agreement and the transactions contemplated in the Merger Agreement have been unanimously approved by the Registrant's board of directors.

**Item 2.01 Completion of Acquisition or Disposition of Assets.**

On April 3, 2017, the Registrant completed the Merger described in Item 1.01 of this Current Report and paid all consideration required to be paid at the closing pursuant to the Merger Agreements.

**Item 3.02 Unregistered Sale of Equity Securities.**

The information contained in Item 1.01 of this Current Report is incorporated by reference into this Item 3.02. The Registrant's shares to be issued and sold pursuant to the Merger Agreement will be issued and sold in reliance on an exemption provided by Section 4(a)(2) of the Securities Act of 1933, as amended.

**Item 7.01 Regulation FD Disclosure**

On April 3, 2017, the Registrant issued a press release (the "Press Release") announcing the closing of the Merger. A copy of the Press Release is included as Exhibit 99.1 hereto and is incorporated by reference.

In addition, the Company will be providing supplemental information regarding the Merger in connection with a presentation to investors on April 3, 2017. The slides to be used in connection with this investor presentation are included as Exhibit 99.2 hereto and are incorporated herein by reference.

The foregoing information in this Item 7.01, including Exhibits 99.1 and 99.2 hereto, shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "***Exchange Act***"), or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference in any of the Registrant's filings under the Securities Act of 1933, as amended, or the Exchange Act, whether made before or after the date hereof, regardless of any incorporation language in such a filing, except as expressly set forth by specific reference in such a filing.

**Item 9.01. Financial Statements and Exhibits.**

**(d) Exhibits**

| Exhibit Number | Exhibit Description |
|---|---|
| 99.1 | Press Release, dated April 3, 2017 |
| 99.2 | April 3, 2017 conference call presentation materials |

**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: April 3, 2017

**SUCAMPO PHARMACEUTICALS, INC.**

By: /s/ Peter Pfreundschuh
Peter Pfreundschuh
Chief Financial Officer

**EXHIBIT INDEX**

| Exhibit Number | Exhibit Description |
|---|---|
| 99.1 | Press Release, dated April 3, 2017 |
| 99.2 | April 3, 2017 conference call presentation materials |

**Exhibit 99.1**

**Sucampo Acquires Vtesse Inc.**

***Transaction Valued at $200 Million Upfront***

***Diversifies Pipeline with Late Stage Program in Niemann-Pick Disease Type C1 (NPC-1)***

***Increases Company Focus on Specialized Diseases with High Unmet Need***

***Leverages Focus on Orphan and Pediatric Diseases***

***Expected to be Accretive to Earnings Beginning in 2019***

***Shareholders and Investors of Sucampo and Vtesse to Establish Foundation to Support Research Related to NPC Disease***

***Company to Host Conference Call Today at 8:30 a.m. EDT***

ROCKVILLE, Md., and GAITHERSBURG, Md., April 03, 2017 (GLOBE NEWSWIRE) -- Sucampo Pharmaceuticals, Inc. (Sucampo) (NASDAQ:SCMP), a global biopharmaceutical company, and Vtesse Inc. (Vtesse), a privately-held rare disease company, today announced that Sucampo has acquired Vtesse for upfront consideration of $200 million. Sucampo funded the acquisition through the issuance of 2,782,678 shares of Sucampo Class A common stock and $170 million of cash on hand; no external financing was utilized.

**Strategic and Financial Benefits of the Transaction**

- Acquisition provides Sucampo with VTS-270, which is in a pivotal study for the treatment of Niemann-Pick Disease Type C1 (NPC-1)
- Builds on Sucampo's capabilities, global development platform and focus on specialized areas of high, unmet medical need
- Fully-enrolled global pivotal clinical trial, with results expected in mid-2018
- Sucampo provides capabilities and resources to accelerate the global development and potential commercialization of VTS-270
- Aligns with Sucampo's patient-focused mission and contributes to goal of building an increasingly diversified, global biopharmaceutical company
- Product is expected to be launched and accretive to earnings beginning in 2019
- Vtesse team will continue to support the advancement of VTS-270

**About Niemann-Pick Disease Type C1**

- Rare genetic disorder that begins impacting the lives of those affected from birth to early adulthood. Clinical symptoms do not slow or reverse, with complications from neurological manifestations being the primary cause of eventual fatalities
- Incidence of NPC-1 is estimated between 1:100,000 to 1:150,000 live births
- Estimated 2,000-3,000 cases globally
- NPC-1 results in early death in the vast majority of cases
- Currently no approved treatments for the disease in the U.S.

**About VTS-270**

VTS-270 is a well-characterized mixture of 2-hydroxypropyl-ß-cyclodextrins (HPßCD) with a specific compositional fingerprint that distinguishes it from other HPßCD mixtures. It is administered by an intrathecal infusion to directly address the neurological manifestations of disease. Preclinical and early clinical studies suggest that the administration of VTS-270 may slow or stop certain indicators of NPC-1, an ultra-orphan, progressive and fatal disease caused by a defect in lipid transport within the cell. VTS-270, which is currently in a pivotal Phase 2b/3 trial, has been granted breakthrough therapy designation in the U.S. and orphan designation in both the U.S. and EU. Effective treatment of NPC remains a high unmet need, with no approved products for patients in the U.S. Results from the pivotal trial are expected in mid-2018.

"We are extremely pleased to announce the acquisition of Vtesse. Sucampo brings significant capabilities to Vtesse and its program, and we believe that this acquisition not only has the potential to make an important difference in the lives of patients, their families, and the dedicated physicians who care for them, but also to create value for shareholders. We welcome the employees of Vtesse to our team and look forward to accelerating the global development of VTS-270 in the hopes of bringing this novel treatment to patients afflicted by Niemann-Pick Disease Type C1 in the U.S. and around the globe," said Peter Greenleaf, Chairman and Chief Executive Officer of Sucampo.

"The Vtesse team remains fully committed to the NPC community and will provide continuity to the patients, families, and clinical sites in cooperation with Sucampo. We recognize that Sucampo shares our commitment to the patients and caregivers of NPC and provides us with the best opportunity to bring this important treatment to NPC-1 patients in the U.S. and around the globe. Together, we will accelerate the global development and commercialization of VTS-270, relying on the complementary capabilities at Sucampo. Our commitment to the patients, families and physicians remains steadfast," said Ben Machielse, Drs, President, Founder and Chief Executive Officer of Vtesse Inc.

Since its launch in January 2015, the Vtesse team has fully enrolled the registrational study of VTS-270 in NPC-1 at 20 clinical trial sites across the globe, providing broad access for study-eligible patients. The team has also been developing a device to assist healthcare providers with administration of VTS-270 to patients, and has supported compassionate use of the drug candidate.

**Terms of the Transaction**

Sucampo has acquired Vtesse for an upfront consideration of $200 million, and has agreed to pay Vtesse shareholders contingent consideration based on mid-single digit to double-digit royalties on global net sales of the product based on increasing net sales levels, and a

share of net proceeds that may be generated from the monetization of the pediatric review voucher, which is expected to be granted in connection with the approval of the product in the U.S. The upfront payment was made in the form of issuance of 2,782,678 Sucampo Class A common shares to the Vtesse shareholders and the payment of $170 million (subject to a working capital adjustment) in cash on hand. No external financing was required for this acquisition. The Vtesse shareholders have agreed to a three-month lock-up of the common shares that were issued, and Sucampo has agreed to register the common shares for resale after the lock-up expires.

Vtesse employees are expected to join Sucampo to continue the important mission they have embarked upon at Vtesse of bringing an NPC therapy to market.

Additionally, Vtesse and Sucampo intend to establish a foundation after the closing of the acquisition to support research related to NPC disease. The establishment of this foundation is a testament to the high level of commitment the Vtesse and Sucampo teams have to scientific advancement regarding NPC. Subject to finalizing the terms of the foundation, Vtesse's equity holders have set aside a portion of the transaction proceeds to contribute to the foundation, and Sucampo intends to match the Vtesse shareholder contribution from its corporate funds.

"At the time of Vtesse's launch in January 2015, Vtesse's original investors recognized the imperative of driving VTS-270 rapidly through clinical development to secure the data for regulatory approvals and to deliver the drug candidate to the NPC-1 community. Sucampo is a global partner that is fully behind the original mission of Vtesse and its investment group. We're very proud to join their shareholders in establishing a foundation that will support further research of and awareness-building for NPC disease," said David Mott, General Partner, NEA, and Vtesse's Board Chair.

**Guidance**

Sucampo today updated its guidance for the full year ending December 31, 2017, incorporating the Vtesse acquisition, as follows:

| **Guidance ($'s M) except EPS** | **Previous Full Year 2017 Guidance Pre Vtesse** | **Revised Full Year 2017 Guidance Post Vtesse** |
|---|---|---|
| Revenue | $220 – $230 | $220 – $230 |
| Adj. Net Income | $80 – $90 | $56 – $66 |
| Adj. EBITDA | $145 – $155 | $109 – $119 |
| Adj. EPS | $1.35 – $1.50 | $1.00 – $1.10 |
| Free Cash Flow | $106 – $116 | $86 – $96 |

**Advisors**

Jefferies LLC served as financial advisor to Sucampo and Leerink Partners served as financial advisor to Vtesse; Cooley LLP served as legal advisor to Sucampo, and Wilmer Cutler Pickering Hale and Dorr LLP served as legal advisor to Vtesse.

**Non-GAAP Financial Measures**

This press release contains four financial metrics **(Adjusted Net Income, EBITDA, Adjusted EBITA and Free Cash Flow)** that are considered "non-GAAP" financial metrics under applicable Securities and Exchange Commission rules and regulations. These non-GAAP financial metrics should be considered supplemental to and not a substitute for financial information prepared in accordance with generally accepted accounting principles. The company's definition of these non-GAAP metrics may differ from similarly titled metrics used by others. Adjusted Net Income adjusts for specified items that can be highly variable or difficult to predict, and various non-cash items, which includes amortization of acquired intangibles, inventory step-up adjustment, R&D intangible asset impairment, restructuring costs, legal settlement, acquisition related expenses, amortization of debt financing costs, debt extinguishment, R&D license option expense, acquisition related acceleration of deferred revenue, foreign currency translations and the tax impact of these adjustments. EBITDA reflects net income excluding the impact of provision for income taxes, interest expense, interest income, depreciation, R&D intangible asset impairment, amortization of acquired intangibles and inventory step-up adjustments. Adjusted EBITDA reflects EBITDA and adjusts for specified items that can be highly variable or difficult to predict, and various non-cash items, which includes share based compensation expense, restructuring costs, acquisition related expenses, debt extinguishment, R&D license option, legal settlement, foreign currency translations and the acquisition related acceleration of deferred revenue. Free cash flow reflects net cash provided by operating activities less expenditures made for property and equipment. The company views these non-GAAP financial metrics as a means to facilitate management's financial and operational decision-making, including evaluation of the company's historical operating results and comparison to competitors' operating results. These non-GAAP financial metrics reflect an additional way of viewing aspects of the company's operations that, when viewed with GAAP results may provide a more complete understanding of factors and trends affecting the company's business.

The determination of the amounts that are excluded from these non-GAAP financial metrics is a matter of management judgment and depends upon, among other factors, the nature of the underlying expense or income amounts. Because non-GAAP financial metrics exclude the effect of items that will increase or decrease the company's reported results of operations, management strongly encourages investors to review the company's consolidated financial statements and publicly-filed reports in their entirety.

**Company to Host Conference Call Today**

Sucampo will host a conference call and webcast today, Monday, April 3, 2017 at 8:30 am ET. Conference call and Webcast participation details are as follows:
Dial-in number: 888-636-8238 (domestic) or 484-747-6635 (international)
Passcode: 98372393
Webcast link: http://www.sucampo.com/investors/events-presentations/

Conference call replay:
Dates: Starting at 11:30 AM ET, April 3, 2017 a replay of the teleconference and webcast will be available
Dial-in number: 855-859-2056 (domestic) or 404-537-3406 (international)
Passcode: 98372393
Webcast link: http://www.sucampo.com/investors/events-presentations/; then click ‘Archived Events’

**About Sucampo Pharmaceuticals, Inc.**

Sucampo Pharmaceuticals, Inc. is focused on the development and commercialization of medicines that meet major unmet medical needs of patients worldwide. Sucampo has two marketed products – AMITIZA, its lead product, and RESCULA – and a pipeline of product candidates in clinical development. A global company, Sucampo is headquartered in Rockville, Maryland, and has operations in Japan and Switzerland. For more information, please visit www.sucampo.com.

The Sucampo logo and the tagline, The Science of Innovation, are registered trademarks of Sucampo AG. AMITIZA is a registered trademark of Sucampo AG.

Follow us on Twitter (@Sucampo_Pharma). Follow us on LinkedIn (Sucampo Pharmaceuticals).
Twitter LinkedIn

**About Vtesse Inc.**

Vtesse Inc. is a rare disease company dedicated to developing drugs for patients suffering from underserved diseases. Vtesse closely collaborates with the National Institutes of Health (NIH), parents, patient support groups and other academic institutions to advance VTS-270 towards regulatory approval. Vtesse is also progressing earlier stage programs for lysosomal storage diseases, including next-generation therapeutics for NPC. Vtesse is based in Gaithersburg, Maryland. For more information, visit www.vtessepharma.com.

**Forward-Looking Statement**

This press release contains "forward-looking statements" as that term is defined in the Private Securities Litigation Reform Act of 1995. These include statements about the development and potential commercialization of VTS-270, the timing of expected clinical trial results, the accretiveness of VTS-270 to earnings, if approved, and the timing of such accretiveness, the potential issuance of a pediatric review voucher and updated financial guidance. These statements are based on management's current expectations and involve risks and uncertainties, which may cause results to differ materially from those set forth in the statements. The forward-looking statements may include statements regarding the development or commercial potential of Sucampo’s products, and other statements that are not historical facts. The following factors, among others, could cause actual results to differ from those set forth in the forward-looking statements: the impact of pharmaceutical industry regulation and health care legislation; Sucampo's ability to accurately predict future market conditions; Sucampo’s ability to successfully integrate the operations of acquired businesses; dependence on the effectiveness of Sucampo's patents and other protections for innovative products; the effects of competitive products on Sucampo’s products; and exposure to litigation and/or regulatory actions.

No forward-looking statement can be guaranteed and actual results may differ materially from those projected. Sucampo undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events, or otherwise. Forward-looking statements in this press release should be evaluated together with the many uncertainties that affect Sucampo's business, particularly those mentioned in the risk factors and cautionary statements in Sucampo's most recent Form 10-K as filed with the Securities and Exchange Commission on March 8, 2017, as well as its filings with the Securities and Exchange Commission on Forms 8-K and 10-Q since the filing of the Form 10-K, all of which Sucampo incorporates by reference.

Contact:
Sucampo Pharmaceuticals, Inc.
Silvia Taylor
Senior Vice President, Investor Relations and Corporate Affairs
1-240-223-3718
staylor@sucampo.com

**Exhibit 99.2**

SUCAMPO
The Science of Innovation

# Acquisition of Vtesse Inc.

## Increased Focus on Orphan and Pediatric Diseases

# Forward Looking Statement

This presentation contains "forward-looking statements" as that term is defined in the Private Securities Litigation Reform Act of 1995. These include statements about the development and potential commercialization of VTS-270, the timing of expected clinical trial results, the accretiveness of VTS-270 to earnings, if approved, and the timing of such accretiveness, the potential issuance of a pediatric review voucher and updated financial guidance. These statements are based on management's current expectations and involve risks and uncertainties, which may cause results to differ materially from those set forth in the statements. The forward-looking statements may include statements regarding the development or commercial potential of Sucampo's products, and other statements that are not historical facts. The following factors, among others, could cause actual results to differ from those set forth in the forward-looking statements: the impact of pharmaceutical industry regulation and health care legislation; Sucampo's ability to accurately predict future market conditions; Sucampo's ability to successfully integrate the operations of acquired businesses; dependence on the effectiveness of Sucampo's patents and other protections for innovative products; the effects of competitive products on Sucampo's products; and exposure to litigation and/or regulatory actions.

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# Non-GAAP Metrics

This presentation contains four financial metrics **(Adjusted Net Income, EBITDA, Adjusted EBITA and Free Cash Flow)** that are considered "non-GAAP" financial metrics under applicable Securities and Exchange Commission rules and regulations. These non-GAAP financial metrics should be considered supplemental to and not a substitute for financial information prepared in accordance with generally accepted accounting principles. The company's definition of these non-GAAP metrics may differ from similarly titled metrics used by others. Adjusted Net Income adjusts for specified items that can be highly variable or difficult to predict, and various non-cash items, which includes amortization of acquired intangibles, inventory step-up adjustment, R&D intangible asset impairment, restructuring costs, legal settlement, acquisition related expenses, amortization of debt financing costs, debt extinguishment, R&D license option expense, acquisition related acceleration of deferred revenue, foreign currency translations and the tax impact of these adjustments. EBITDA reflects net income excluding the impact of provision for income taxes, interest expense, interest income, depreciation, R&D intangible asset impairment, amortization of acquired intangibles, and inventory step-up adjustments. Adjusted EBITDA reflects EBITDA and adjusts for specified items that can be highly variable or difficult to predict, and various non-cash items, which includes share based compensation expense, restructuring costs, acquisition related expenses, debt extinguishment, R&D license option, legal settlement, foreign currency translations and the acquisition related acceleration of deferred revenue. Free cash flow reflects net cash provided by operating activities less expenditures made for property and equipment. The company views these non-GAAP financial metrics as a means to facilitate management's financial and operational decision-making, including evaluation of the company's historical operating results and comparison to competitors' operating results. These non-GAAP financial metrics reflect an additional way of viewing aspects of the company's operations that, when viewed with GAAP results may provide a more complete understanding of factors and trends affecting the company's business.

The determination of the amounts that are excluded from these non-GAAP financial metrics is a matter of management judgment and depends upon, among other factors, the nature of the underlying expense or income amounts. Because non-GAAP financial metrics exclude the effect of items that will increase or decrease the company's reported results of operations, management strongly encourages investors to review the company's consolidated financial statements and publicly-filed reports in their entirety.

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# Overview of Vtesse Inc. Acquisition

## Overview

- Sucampo has acquired privately owned Vtesse Inc. and its lead orphan drug candidate for Niemann-Pick Type C1, VTS-270, for $200 million upfront consideration
- No external financing required

## Strategic Benefits

- Builds on Sucampo's capabilities, global development platform and focus on specialized areas of high, unmet medical need
  - Complementary to FAP program with CPP
  - Additive to orphan and pediatric development focus
- VTS-270 for the Treatment of Niemann-Pick Type C1 (NPC-1) in global pivotal registration program
  - Ultra-rare disorder with devastating and ultimately fatal outcome
  - Fully enrolled, results in 2018
- Sucampo provides capabilities and resources to accelerate development and successfully commercialize VTS-270
- Aligns with Sucampo's patient-focused mission and contributes to goal to build an increasingly diversified, global biopharmaceutical company
- Accretive to earnings beginning in 2019

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# Transaction Overview

- Sucampo to acquire Vtesse for a total upfront consideration of $200 million
- Acquisition to be financed through the issuance of 2,782,678 Sucampo Class A common shares and $170 million in cash on hand, subject to customary adjustments; no external financing required
- Contingent earn-out payments consist of mid-single digit to double-digit royalties on global net sales of the product at increasing net sales levels
- Split of the proceeds should a pediatric review voucher be issued and monetized in connection with the approval of VTS-270
- 3-month lock-up period for Vtesse shareholders that were issued common shares; Sucampo has agreed to register the common shares for resale after the lock-up expires
- Portion of deal proceeds set aside for establishment of a foundation to support research related to NPC disease

Vtesse

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# Vtesse: A Patient-Focused, Rare Disease Company

- Privately-held company focused on developing drugs for patients suffering from devastating diseases
- VTS-270 for treatment of Niemann-Pick Disease Type C1 (NPC-1)
  - Mechanism and intrathecal route of administration directly address most devastating and commonly fatal neurological disease manifestations
  - Pivotal fully-enrolled multi-national study
  - Orphan drug designation in the U.S. and Europe
    - FDA and EMA approval: single pivotal trial sufficient for registration
    - Breakthrough designation in the U.S.
  - Compelling pre-clinical and Phase 1 data with clinically significant benefit as measured by disease specific outcomes; safety experience supports chronic treatment
  - 10 employees with unique patient-focused drug development capabilities
    - Complementarity in capabilities with Sucampo
  - Headquartered in Gaithersburg, MD

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# NPC-1: Ultra-Rare, Fatal Pediatric Disease with Urgent Patient Need

- NPC-1 is an ultra-rare, progressive and fatal disease caused by defects in lipid transformation within the cell
- Diagnosis of NPC-1 most common outside infantile ages
  - Progressive and irreversible neurological manifestations
  - Estimated 2,000-3,000 cases globally
  - Under- and mis-diagnosed
- NPC-1 results in early death in the vast majority of cases
- Currently no approved treatments for the disease in the U.S.
- Highly motivated and involved patients, families and physicians with strong commitment to development

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# NPC-1 Overview, cont.

- NPC genes (NPC1 and NPC2) code for proteins that act sequentially to release cholesterol into cells
- Mutations in either of these genes cause un-esterified cholesterol and other lipids to become sequestered and impair transport to plasma membrane and endoplasmic reticulum
- Lipid build up in the brain can kill cells and, over time, leads to neurological, systemic, and/or psychiatric problems, generally leading to mortality
- Leads to excessive accumulation of lipids in the brain, liver and spleen

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# VTS-270 for Treatment of NPC-1

- VTS-270 is a highly-purified mixture of 2-hydroxypropyl-ß-cyclodextrins with a specific compositional fingerprint that targets cholesterol and sphingolipid storage
- Initially evaluated by consortium of academic labs led by NIH in collaboration with parent and patient advocacy groups
- In preclinical studies, VTS-270 had a profound effect on depleting intracellular cholesterol stores and lysosomal accumulation
- In multiple preclinical animal models of NPC-1, VTS-270 has also shown results in preventing dysfunctions such as ataxia and profoundly impacting survival

On-going formulation studies may culminate in new intellectual property for VTS-270

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# Clinical Data Phase 1/2 Study

## 18 Month Treatment Results Reveal that VTS-270 Shows Robust Disease Modification with Durable Effects Over 18 Months


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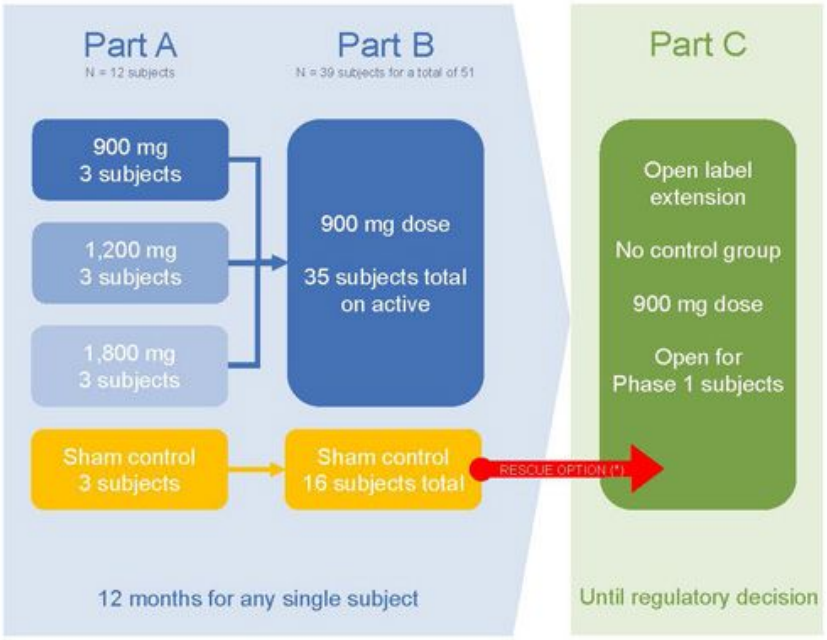
# VTS-270 Pivotal Trial




- IT injections every 2 weeks
- Trial fully enrolled
- Pivotal data expected in mid-2018
- Potential regulatory approval in U.S. and EU in 1H19
- Phase 1 patients currently in Part C on therapy >36 months

## Phase 3 Study Endpoints

1. Composite score evaluating the progression of the neurological manifestations in NPC-SS four major domains: ambulation, fine motor, cognition, and swallowing
2. Physician global score: Clinical Global Impression of Change
3. Long-term safety and tolerability of the drug administered by lumbar punctures every 2 weeks

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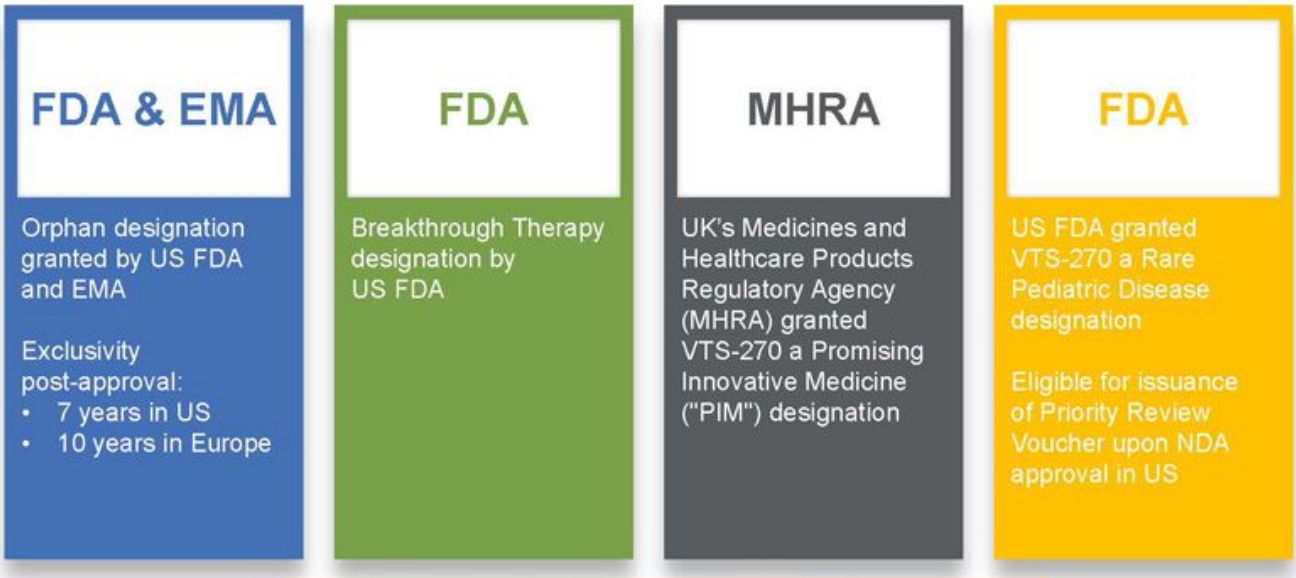
# VTS-270 Regulatory Status




Potential IP could extend exclusivity through 2036, including current patent applications and new patents and orphan exclusivity

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# Financial Impact

- Full Year 2017 Guidance Pre and Post Acquisition

| Guidance ($'s M) except EPS | Previous Full Year 2017 Guidance Pre Vtesse | Revised Full Year 2017 Guidance Post Vtesse |
|---|---|---|
| Revenue | $220 – $230 | $220 – $230 |
| Adj. Net Income | $80 – $90 | $56 – $66 |
| Adj. EBITDA | $145 – $155 | $109 – $119 |
| Adj. EPS | $1.35 – $1.50 | $1.00 – $1.10 |
| Free Cash Flow | $106 – $116 | $86 – $96 |

- Accretive to earnings beginning in 2019

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# Conclusion

Aligns with Sucampo's focus on patients and meeting the challenges of diseases with high unmet medical need

Diversifies company and expands further in rare, orphan diseases and pediatric arena

Sucampo brings global capabilities and resources to accelerate approval and commercialize VTS-270

Significant step in our goal of building a global biopharmaceutical company

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Q&A Session

SUCAMPO
The Science of Innovation