

## **Strongbridge Biopharma plc Provides Corporate and Financial Update**

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*~ Expands Rare Disease Portfolio through Acquisition of U.S. Rights to KEVEYIS®, the First and Only FDA-Approved Treatment for Hyperkalemic, Hypokalemic, and Related Variants of Primary Periodic Paralysis ~*

*~ Provides Update on Timelines and Strengthens COR-003 Clinical Development Program with Additional Phase 3 Study*

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*~ Selects Proprietary Technology for COR-005 Long-Acting Release Formulation ~*

*~ Announces \$35 Million in Equity Financing; Finalizing \$40 Million Credit Facility of which \$20 Million to be Borrowed Initially, Extending Cash Runway at Least Through 2018 ~*

DUBLIN, Ireland and TREVOSTOWN, Pa., Dec. 23, 2016 (GLOBE NEWSWIRE) -- Strongbridge Biopharma plc, (Nasdaq:SBBP), a global commercial-stage biopharmaceutical company focused on the development and commercialization of therapies for rare diseases with significant unmet needs, today announced a corporate and financial update.

“Throughout the second half of 2016, Strongbridge has been focused on building value in three key areas: enhancing the clinical development program and the probability of regulatory success for COR-003, pursuing a commercial-stage rare disease therapy resulting in the acquisition of the U.S. rights to KEVEYIS® (dichlorphenamide), and strengthening the financial condition of the Company,” said Matthew Pauls, president and chief executive officer of Strongbridge. “Executing on these initiatives strengthens our presence in rare diseases and sets the stage for a number of value-creating events in 2017, including commercial sales of KEVEYIS and completing enrollment in the SONICS study,” Pauls added.

***Expands Rare Disease Portfolio through Acquisition of U.S. Rights to KEVEYIS®, the First and Only FDA-Approved Treatment for Hyperkalemic, Hypokalemic, and Related Variants of Primary Periodic Paralysis***

Earlier today, Strongbridge announced in a separate press release that the Company has acquired the U.S. rights to KEVEYIS, the first and only U.S. Food and Drug Administration (FDA)-approved product to treat hyperkalemic, hypokalemic, and related variants of Primary Periodic Paralysis, which is a group of rare hereditary disorders that causes potentially severe episodes of muscle weakness and/or paralysis. KEVEYIS is Strongbridge’s first commercial product, and expands the Company’s rare disease franchises to include both neuromuscular and endocrine diseases.

***Provides Update on Timelines and Strengthens COR-003 Clinical Development Program with Additional Phase 3 Study***

The global Phase 3 SONICS clinical trial evaluating COR-003 (levoketoconazole) for the treatment of endogenous Cushing’s syndrome is more than two-thirds enrolled. Based upon an analysis of recent enrollment trends, Strongbridge anticipates that the study will be fully enrolled in Q2 2017, with top-line data for the primary efficacy analysis available in Q1 2018.

In October 2016, at the fourth semi-annual Data and Safety Monitoring Board (DSMB) review of the SONICS data, the DSMB recommended that the SONICS study continue as planned.

In addition, based upon Strongbridge’s ongoing dialog with the FDA and observation of the current regulatory landscape, the Company plans to initiate LOGICS, a second global Phase 3 study of COR-003 for the treatment of endogenous Cushing’s syndrome. The LOGICS study will supplement the long-term efficacy and safety data from the ongoing SONICS study in a randomized, double-blind, placebo-controlled study that will enroll approximately 35 patients, of which approximately two-thirds will have completed the SONICS study. LOGICS enrollment is anticipated to begin

mid-year 2017 and top-line data are expected in Q3 2018. Additional detail regarding the LOGICS study will be provided upon initiation.

“While recruitment in SONICS has been slower than anticipated, we have implemented several initiatives including additional site activations, new recruitment tactics, and protocol changes to improve enrollment rates,” said Fredric Cohen, M.D., chief medical officer of Strongbridge. “We believe that SONICS will provide scientifically-rigorous and clinically-relevant evidence characterizing the efficacy and safety of COR-003. The addition of LOGICS will provide an opportunity to enhance the body of evidence for COR-003 by generating data in a well-controlled study,” Cohen added.

### ***Strongbridge Selects Proprietary Technology for COR-005 Long-Acting Release Formulation***

COR-005 (veldoreotide) is a novel, investigational somatostatin analogue (SSA) with proven and differentiated pharmacology in pre-clinical models and patients with acromegaly. COR-005 may provide the acromegaly community with a new treatment option offering a unique clinical profile and delivery system. COR-005 has been granted orphan drug designation by the FDA and the European Medicines Agency (EMA). Strongbridge has completed the screening of potential long-acting release (LAR) technologies for COR-005, and selected a proprietary formulation based upon PLGA microspheres. PLGA is a well-known polymer, which has been widely applied in LAR formulations due to its biocompatibility, biodegradability, and favorable release kinetics.

### ***Announces \$35 Million in Equity Financing; Finalizing \$40 Million Credit Facility of which \$20 Million to be Borrowed Initially, Extending Cash Runway at Least Through 2018***

Strongbridge announced today in a separate press release that the Company has entered into a definitive securities purchase agreement for a \$35 million equity financing led by CAM Capital and Vivo Capital, with additional participation from Broadfin Capital, Boxer Capital of the Tavistock Group and HealthCap, as well as several new and existing institutional and individual investors. In addition to this equity financing, the Company is in the process of finalizing credit documentation for a \$40 million credit facility, which the Company anticipates will close on or about December 28, 2016, and of which \$20 million would be borrowed initially. Under the credit facility, the Company anticipates having access to two additional tranches of \$10 million each, which would be available to the Company subject to the achievement of certain specified milestones. It is anticipated that the credit facility would mature after 48 months, provide interest-only payments initially for the first 18 months of the loan followed by an amortization period of 30 months, provide for a final payment fee equal to 8% of the amount borrowed, and bear interest at a rate equal to the sum of 8.22% plus the greater of 0.53% or the 30-day US LIBOR rate. It is also anticipated that the credit facility will provide that if the Company satisfies certain milestones and borrows the final \$10 million tranche, the interest-only period would be extended by an additional six months and the amortization period would be 24 months. Under the credit facility, the Company would grant a security interest in substantially all of its existing and after-acquired assets, including intellectual property. The credit facility would contain facility and prepayment fees, and customary affirmative and negative covenants, and events of default.

The Company’s pro forma cash and cash equivalents balance as of September 30, 2016 is \$77.1 million, inclusive of estimated net proceeds from the equity financing and the initial \$20 million borrowing under the credit facility. The Company believes it has sufficient financial resources, excluding any additional borrowings under the credit facility, to fund planned operations at least through 2018.

### ***Reports Financial Results***

#### **Second Quarter 2016 Results**

For the three months ended June 30, 2016, net loss attributable to ordinary shareholders was \$12.8 million, or \$0.61 per basic and diluted share, compared to \$17.1 million, or \$1.19 per basic and diluted share, for the same period in the prior year.

Research and development expenses were \$4.6 million for the three months ended June 30, 2016, compared to \$7.7 million for the same period in the prior year. Research and development expenses for the three months ended June 30, 2015 included \$3.9 million of the \$5.0 million in aggregate cash paid to Antisense Therapeutics upon entering into a license agreement in May 2015, with the remaining \$1.1 million of cash paid recorded as the initial carrying value of our investment in the equity of Antisense Therapeutics. Research and development expenses for the three months ended June 30, 2016 included \$1.8 million of increased expenses related to the global Phase 3 SONICS clinical trial and supporting development activities for COR-003, formulation development activities for COR-005, and compensation and related personnel costs of increased employee count. These expenses were partially offset by a \$1.0 million decrease in non-cash stock-based compensation costs resulting from the departure of certain research and development personnel.

General and administrative expenses were \$4.0 million for the three months ended June 30, 2016, compared to \$9.5 million for the same period in the prior year. General and administrative expenses for the three months ended June 30, 2015 included \$3.4 million of transaction fees and expenses related to the acquisition of COR-005 from Aspireo Pharmaceuticals, the license of COR-004 from Antisense Therapeutics, and other business development activities. General and administrative expenses for the three months ended June 30, 2015 also included \$1.5 million of legal and accounting fees related to the indirect activities necessary to prepare the Company's financial records for the U.S. initial public offering, which was completed in October 2015. General and administrative expenses for the three months ended June 30, 2016 included a \$0.6 million decrease in non-cash stock based compensation costs due to liability-classified treatment of certain stock option awards in 2015.

As a result of the termination of the license agreement between BioPancreate and Cornell Center for Technology Enterprise and Commercialization (CCTEC), we recorded an impairment charge related to the in-process research and development of \$5.2 million for the three months ended June 30, 2016.

### **Third Quarter 2016 Results**

For the three months ended September 30, 2016, net loss attributable to ordinary shareholders was \$7.6 million, or \$0.36 per basic and diluted share, compared to \$9.7 million, or \$0.51 per basic and diluted share, for the same period in the prior year.

Research and development expenses were \$4.5 million for the three months ended September 30, 2016, compared to \$4.1 million for the same period in the prior year. The increase was primarily attributable to expenses related to the global Phase 3 SONICS clinical trial and supporting development activities for COR-003, and compensation and related personnel costs of increased employee count. These expenses were partially offset by a decrease in development spending for the former COR-004 project.

General and administrative expenses were \$3.1 million for the three months ended September 30, 2016, compared to \$5.5 million for the same period in the prior year. General and administrative expenses for the three months ended September 30, 2015 included \$1.7 million of legal and accounting fees related to the indirect activities necessary to prepare the Company's financial records for the U.S. initial public offering, which was completed in October 2015. The remaining net decrease was primarily due to lower legal fees in support of general corporate matters, and lower employee recruiting fees and consulting fees for general business efforts. Partially offsetting these costs were higher cash compensation and non-cash stock based compensation costs due to increased employee count.

For the nine months ended September 30, 2016, net loss attributable to ordinary shareholders was \$32.7 million, or \$1.54 per basic and diluted share, compared to \$33.2 million, or \$2.18 per basic and diluted share, for the same period in the prior year.

Research and development expenses were \$15.9 million for the nine months ended September 30, 2016, compared to \$14.3 million for the same period in the prior year. Research and development expenses for the nine months ended September 30, 2015 included \$3.9 million of the \$5.0 million in aggregate cash paid to Antisense Therapeutics upon

entering into a license agreement in May 2015, with the remaining \$1.1 million of cash paid recorded as the initial carrying value of our investment in the equity of Antisense Therapeutics. The remaining net increase was primarily attributable to expenses related to the global Phase 3 SONICS clinical trial and supporting development activities for COR-003, development spending for the former COR-004 project, formulation development activities for COR-005, and compensation and related personnel costs on increased employee count. These expenses were partially offset by a decrease in non-cash stock-based compensation costs due to the departure of certain research and development employees.

General and administrative expenses were \$11.3 million for the nine months ended September 30, 2016, compared to \$18.2 million for the same period in the prior year. General and administrative expenses for the nine months ended September 30, 2015 included \$3.7 million of legal and accounting fees related to the indirect activities necessary to prepare the Company's financial records for the U.S. initial public offering, which was completed in October 2015. General and administrative expenses for the nine months ended September 30, 2015 also included \$3.6 million of transaction fees and expenses related to the acquisition of COR-005 from Aspireo Pharmaceuticals, the license of COR-004 from Antisense Therapeutics, and other business development activities. The remaining net increase was primarily due to higher cash compensation and non-cash stock based compensation costs due to increased employee count, partially offset by decreased legal fees in support of general corporate matters, lower employee recruiting fees and consulting fees for general business efforts.

As a result of the termination of the license agreement between BioPancreate and Cornell Center for Technology Enterprise and Commercialization (CCTEC), we recorded an impairment charge related to the In-process research and development of \$5.2 million for the nine months ended September 30, 2016.

### **About Strongbridge Biopharma**

Strongbridge Biopharma is a global commercial-stage biopharmaceutical company focused on the development and commercialization of therapies for rare diseases with significant unmet needs. Strongbridge's first commercial product is KEVEYIS® (dichlorphenamide), the first and only FDA-approved treatment for hyperkalemic, hypokalemic, and related variants of Primary Periodic Paralysis. KEVEYIS has orphan drug exclusivity status in the U.S. through August 7, 2022. In addition to establishing this neuromuscular disease franchise, the Company has a clinical-stage pipeline of therapies for rare endocrine diseases. Strongbridge's lead compounds include COR-003 (levoketoconazole), a cortisol synthesis inhibitor currently being studied for the treatment of endogenous Cushing's syndrome, and COR-005, a next-generation somatostatin analog (SSA) being investigated for the treatment of acromegaly, with potential additional applications in Cushing's syndrome and neuroendocrine tumors. Both COR-003 and COR-005 have received orphan designation from the U.S. Food and Drug Administration and the European Medicines Agency. For more information, visit [www.strongbridgebio.com](http://www.strongbridgebio.com).

### **ABOUT KEVEYIS**

#### **KEVEYIS® Indication**

KEVEYIS® (dichlorphenamide) is indicated for the treatment of primary hyperkalemic periodic paralysis, primary hypokalemic periodic paralysis, and related variants.

#### **KEVEYIS Important Safety Information**

In clinical studies, the most common side effects of KEVEYIS were a numbness or tingling, difficulty thinking and paying attention, changes in taste, and confusion. These are not all of the possible side effects that you may experience with KEVEYIS. Talk to your doctor if you have any symptoms that bother you or do not go away.

KEVEYIS is not for everyone. Do not take KEVEYIS if you:

- Are on a high-dose aspirin regimen
- Are allergic to sulfa-based drugs

- Have liver, kidney, or certain lung conditions
- Are pregnant, planning to become pregnant, or nursing
- Are under 18 years old

Taking KEVEYIS may cause a drop in the amount of potassium (an electrolyte) in your body, which can lead to heart problems. Ask your doctor if you need to eat foods that contain high amounts of potassium while taking KEVEYIS.

Your body may produce too much acid or may not be able to remove enough acid from body fluids while taking KEVEYIS. Your doctor will run tests on a regular basis to check for signs of acid buildup and may reduce your dose or stop your treatment with KEVEYIS.

KEVEYIS may also increase the risk of falls, especially in elderly patients and patients taking high doses of KEVEYIS. Use caution when driving, operating machinery, or performing any other hazardous activities while taking KEVEYIS, as this medication may cause drowsiness.

You are encouraged to report side effects to Strongbridge Biopharma at 1-855-324-8912, or to the FDA at 1-800-FDA-1088 or visit [www.fda.gov/medwatch/](http://www.fda.gov/medwatch/). For more information, go to [www.keveyis.com](http://www.keveyis.com).

For additional KEVEYIS important safety information, please see full prescribing information at [www.keveyis.com](http://www.keveyis.com).

STRONGBRIDGE BIOPHARMA™ is a trademark of Strongbridge Biopharma plc.

KEVEYIS® is a registered trademark licensed exclusively in the U.S. to Strongbridge Biopharma plc.

### Forward-Looking Statements

*This press release contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this press release, are forward-looking statements. These statements relate to future events and involve known and unknown risks, including, without limitation, uncertainties regarding Strongbridge's strategy, plans, future financial position, anticipated investments, costs and results, outcomes of product development efforts, status and results of clinical trials and objectives of management for future operations. The words "anticipate," "estimate," "expect," "intend," "may," "plan," "potential," "project," "target," "will," "would," or the negative of these terms or other similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. These forward-looking statements are based on current expectations, estimates, forecasts and projections and are not guarantees of future performance or development and involve known and unknown risks, uncertainties and other factors. The forward-looking statements contained in this press release are made as of the date of this press release, and Strongbridge Biopharma does not assume any obligation to update any forward-looking statements except as required by applicable law.*

### STRONGBRIDGE BIOPHARMA plc

#### Selected Consolidated Balance Sheet Information

(in thousands, except share and per share data)

	September 30, 2016	June 30, 2016	December 31, 2015
<b>Consolidated Balance Sheet Data:</b>			
Cash and cash equivalents	\$ 25,469	\$ 33,884	\$ 51,623
Total assets	64,555	73,079	97,330

Total liabilities	4,240	4,835	6,403
Total shareholders' equity	60,315	68,244	90,927

**STRONGBRIDGE BIOPHARMA plc**

**Consolidated Statement of Operations and Comprehensive Loss**

(in thousands, except share and per share data)

Consolidated Statement of Operations Data:	Three Months Ended June 30,		Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015	2016	2015
Operating expenses:						
Research and development	\$ 4,572	\$ 7,718	\$ 4,516	\$ 4,110	\$ 15,882	\$ 14,328
General and administrative	4,014	9,512	3,117	5,535	11,260	18,155
Impairment of in-process research and development	5,228	—	—	—	5,228	—
Total operating expenses	13,814	17,230	7,633	9,645	32,370	32,483
Operating loss	(13,814 )	(17,230 )	(7,633 )	(9,645 )	(32,370 )	(32,483 )
Other income (expense), net:						
Foreign exchange loss	3	466	(20 )	(136 )	(64 )	(450 )
Other income (expense), net	44	(502 )	35	(13 )	(1,211 )	(556 )
Total other income (expense), net	47	(36 )	15	(149 )	(1,275 )	(1,006 )
Loss before income taxes	(13,767 )	(17,266 )	(7,618 )	(9,794 )	(33,645 )	(33,489 )
Income tax benefit	871	123	—	128	926	306
Net loss	(12,896 )	(17,143 )	(7,618 )	(9,666 )	(32,719 )	(33,183 )
Net loss attributable to non-controlling interest	55	—	17	10	122	10
Net loss attributable to Strongbridge Biopharma	\$ (12,841 )	\$ (17,143 )	\$ (7,601 )	\$ (9,656 )	\$ (32,597 )	\$ (33,173 )

Net loss attributable to common shareholders, basic and diluted	\$ (12,841 )	\$ (17,143 )	\$ (7,601 )	\$ (9,656 )	\$ (32,597 )	\$ (33,173 )
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Net loss per share attributable to common shareholders, basic and diluted	\$ (0.61 )	\$ (1.19 )	\$ (0.36 )	\$ (0.51 )	\$ (1.54 )	\$ (2.18 )
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Weighted-average shares used in computing net loss per share attributable to common shareholders, basic and diluted	21,205,382	14,461,867	21,205,382	18,784,190	21,205,382	15,203,440
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