

May 4, 2017



# Synthetic Biologics Reports First Quarter 2017 Operational Highlights and Financial Results

**-- Results from Phase 2b Proof-of-Concept Study Exploratory Endpoints Demonstrate Ribaxamase Protects and Preserves Gut Microbiome from Ceftriaxone-mediated Dysbiosis --**

**-- Two Ribaxamase and Two SYN-010 Presentations to be Included at Digestive Disease Week (DDW 2017) --**

**-- Conference Call Today, May 4, 2017, at 4:30 p.m. (EDT) --**

ROCKVILLE, Md., May 4, 2017 /PRNewswire/ -- [Synthetic Biologics, Inc.](#) (NYSE MKT: SYN), a late-stage clinical company developing therapeutics that preserve the microbiome to protect and restore the health of patients, today provided an operational update and reported financial results for the three months ended March 31, 2017.



"During the first quarter of 2017, we announced several important clinical milestones which continue to drive momentum for Synthetic Biologics," said Jeffrey Riley, President and Chief Executive Officer. "We announced positive results from our Phase 2b proof-of-concept clinical trial for ribaxamase demonstrating the achievement of the primary endpoint of significantly reducing the incidence of primary *Clostridium difficile* infection (CDI). In addition, data from this study demonstrated that ribaxamase significantly lowered the incidence of new colonization by vancomycin-resistant enterococci (VRE), compared to placebo. Most

recently, we shared supportive data from exploratory endpoints in this trial with the CDC demonstrating ribaxamase successfully protected and preserved the naturally occurring composition of the gut microbiome from the dysbiotic effects of IV ceftriaxone, compared to placebo. These results position ribaxamase as a leader in clinical development for microbiome-based interventions specifically designed to prevent the incidence of primary CDI and antimicrobial resistance (AMR) amongst at-risk patients. We continue to analyze data from this study and expect to share results in the coming months from several additional exploratory endpoints designed to evaluate ribaxamase's ability to prevent the emergence and proliferation of AMR in the gut microbiome."

Mr. Riley continued, "Since the beginning of the first quarter, we also shared positive preclinical findings for SYN-005, our orphan drug program designed to treat and prevent pertussis, which if translated to humans may provide a much-needed approach to protecting and treating the more than 50 million global at-risk individuals, especially newborns in the developing world. Looking ahead, we are focused on building upon the progress of the first quarter during the remainder of 2017. With the foundation of our Phase 2b/3 adaptive design pivotal trial in place for SYN-010, intended to treat an underlying cause of IBS-C, we remain focused on solidifying the infrastructure to support its continued successful clinical advancement."

### **Microbiome-Focused Clinical Program Progress**

#### **SYN-004 – Prevention of *C. difficile* infection (CDI), antibiotic-associated diarrhea (AAD) and the emergence of antimicrobial resistance (AMR):**

- Reported positive topline data from global Phase 2b proof-of-concept randomized, double-blind, placebo controlled clinical trial of 412 patients (1Q 2017)
  - Achieved primary endpoint, demonstrating a statistically significant relative risk reduction of 71.4% ( $p=0.045$ ) in CDI rates, compared to placebo
  - Demonstrated a significant reduction in new colonization by vancomycin-resistant enterococci (VRE) for patients receiving ribaxamase, compared to placebo ( $p=0.0002$ )
- Reported supportive data from several exploratory endpoints from Phase 2b proof-of-concept clinical trial demonstrating ribaxamase protected the gut microbiome from antibiotic-mediated dysbiosis in patients receiving ribaxamase, compared to placebo
  - Patients receiving ribaxamase demonstrated significantly better maintenance of and recovery of the composition of the gut microbiome, compared to placebo
  - Patients receiving ribaxamase demonstrated lower incidences of new colonization by VRE and other opportunistic and potentially pathogenic microorganisms, compared to placebo
  - These results are consistent with protection of the gut microbiome and with ribaxamase's mechanism of action during IV administration of ceftriaxone
- Expect to share results from several additional exploratory endpoints designed to evaluate ribaxamase's ability to protect the gut microbiome from opportunistic bacteria and prevent the emergence of antimicrobial resistance (AMR) in the gut microbiome (2H 2017)
- Anticipate requesting end of Phase 2 meeting with FDA (2H 2017)
- Plan to initiate Phase 3 clinical trial(s) (1H 2018)

#### **SYN-010 – Treatment of irritable bowel syndrome with constipation (IBS-C):**

- Confirmed key elements of Pivotal Phase 2b/3 clinical trial pursuant to consultations with the FDA (1Q 2017)
  - A 12-week, multi-center, double-blind, placebo-controlled, adaptive design clinical trial
  - A study population of approximately 840 adult subjects diagnosed with IBS-C
  - Evaluation of efficacy and safety of two dose strengths of SYN-010 (21 mg and 42 mg) compared to placebo
  - Conducted in approximately 150 clinical sites in North America
  - Study subjects will be randomized in a 1:1:1 ratio, receiving either 21 mg of SYN-010, 42 mg of SYN-010, or placebo
  - Enrollment is open to all IBS-C patients; breath-methane will be measured at baseline to ensure a comparable ratio of high-to-low breath methane IBS-C patients in each treatment arm
  - An interim futility analysis may be conducted when approximately 50% of patients in each dosing arm have completed treatment

### **SYN-005 – Treatment and Prevention of Pertussis (whooping cough)**

- Reported positive preclinical research findings from neonatal non-human primate study designed to determine if administration of hu1B7, one component of SYN-005, at two days of age could protect study animals from a subsequent pertussis infection
  - Control animals (n=6), challenged with *Bordetella pertussis* (*B. pertussis*) at five weeks of age, demonstrated marked elevations in white blood cell counts and most exhibited behavioral signs of pertussis including coughing and diminished activity
  - Treatment animals (n=7), treated with hu1B7 at two days of age and then infected five weeks later, had significantly lower peak white blood cell counts (p=0.004) that remained within the normal range or were only slightly elevated
  - All seven treatment animals that received prophylactic hu1B7 appeared healthy and none exhibited any behavioral signs of pertussis

Synthetic Biologics also announced that four abstracts have been accepted for presentation at Digestive Disease Week ® 2017. Members of the Synthetic Biologics team will present two posters on SYN-010 and two talks on ribaxamase at DDW ®.

### **Digestive Disease Week ® (DDW)**

May 7, 2017

- **Poster Su2012:** Poster Su2012: Increased pulmonary CO<sub>2</sub> Excretion in Patients with Small Intestinal Bacterial Overgrowth (SIBO): Possible Implications for COPD
  - Session Number 7245 from 12:00 p.m. – 2:00 p.m. CDT
  - Venue: McCormick Place, South Hall
- **Poster Su1562:** Initial Paradoxical Peaks on Breath Testing (first sample high) do not Affect the Diagnostic Utility of the Spot-methane Breath Test
  - Session Number 7130 from 12:00 p.m. – 2:00 p.m. CDT
  - Venue: McCormick Place, South Hall
- **Lecture 332g:** An Orally Delivered Beta-Lactamase Protects the Gut Microbiome from Antibiotic-Mediated Damage and Mitigates the Propagation of Antibiotic-Resistance Genes in a Porcine Dysbiosis Model.

- Session Number 3505 from 2:15 p.m. – 2:30 p.m. CDT
- Venue: McCormick Place, room S503

May 9, 2017

- **Lecture 874j:** SYN-004 (ribaxamase), an Oral  $\beta$ -Lactamase, Prevented *Clostridium difficile* Infection and Protected Patients from Colonization by Antimicrobial Resistant Pathogens by Preserving Gut Microbiome Diversity in a Phase 2b Clinical Trial
  - Session Number 5235 from 10:30 a.m. – 10:45 a.m. CDT
  - Venue: McCormick Place, room S103

### **Quarter Ended March 31, 2017 Financial Results**

General and administrative expenses were \$2.1 million for the three months ended March 31, 2017, compared to \$2.4 million for the same period in 2016. This decrease is primarily the result of lower employee salary expense and related benefits costs along with reduced travel and legal expenses. The charge related to stock-based compensation expense was \$698,000 for the three months ended March 31, 2017, compared to \$643,000 the same period in 2016.

Research and development expenses were \$6.0 million for the three months ended March 31, 2017, compared to \$8.1 million for the same period in 2016. This decrease is primarily the result of lower ribaxamase program costs associated with its clinical development program, as well as manufacturing and research activities within our other microbiome-focused research and development activities. The charge related to non-cash stock-based compensation expense was \$437,000 for the three months ended March 31, 2017, compared to \$409,000 for the same period in 2016.

Other income was \$5.1 million for the three months ended March 31, 2017, compared to other expense of \$0.5 million for the same period in 2016. Other income for the three months ended March 31, 2017 is due to non-cash income of \$5.1 million from the change in fair value of warrants. The decrease in the fair value of the warrants was due to the decrease in our stock price from the prior quarter.

Cash and cash equivalents as of March 31, 2017 was \$13.5 million, a decrease of \$5.6 million from December 31, 2017.

### **Conference Call**

Synthetic Biologics will hold a conference call today, Thursday, May 4, 2017, at 4:30 p.m. (EDT). The dial-in information for the call is as follows, U.S. toll free: 1-888-347-5280 or International: +1 412-902-4280. Participants are asked to dial in 15 minutes before the start of the call to register. The call will also be webcast over the Internet at <https://www.webcaster4.com/Webcast/Page/1096/20882>. An archive of the call will be available for replay at the same URL, <https://www.webcaster4.com/Webcast/Page/1096/20882>, for 90 days after the call.

### **About Synthetic Biologics, Inc.**

Synthetic Biologics, Inc. (NYSE MKT: SYN) is a late-stage clinical company developing therapeutics designed to preserve the microbiome to protect and restore the health of

patients. The Company's lead candidates poised for Phase 3 development are: (1) SYN-010 which is intended to reduce the impact of methane producing organisms in the gut microbiome to treat an underlying cause of irritable bowel syndrome with constipation (IBS-C), and (2) SYN-004 (ribaxamase) which is designed to protect the gut microbiome from the effects of certain commonly used intravenous (IV) beta-lactam antibiotics for the prevention of *C. difficile* infection (CDI), antibiotic-associated diarrhea (AAD) and the emergence of antimicrobial resistance (AMR). The Company is also developing preclinical stage monoclonal antibody therapies for the prevention and treatment of pertussis and novel discovery stage biotherapeutics for the treatment of phenylketonuria (PKU). For more information, please visit Synthetic Biologics' website at [www.syntheticbiologics.com](http://www.syntheticbiologics.com).

*This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. In some cases forward-looking statements can be identified by terminology such as "may," "should," "potential," "continue," "expects," "anticipates," "intends," "plans," "believes," "estimates," and similar expressions, and include statements regarding the results of Synthetic Biologics' Phase 2b proof-of-concept clinical trial for ribaxamase positioning ribaxamase as a leader in clinical development for microbiome-based interventions specifically designed to prevent the incidence of primary CDI and antimicrobial resistance (AMR) amongst at-risk patients, the continued analysis of data from this study, the expected sharing of results in the coming months from several additional exploratory endpoints designed to evaluate ribaxamase's ability to prevent the emergence and proliferation of AMR in the gut microbiome, the ability of SYN-005 to provide an approach to protecting and treating the more than 50 million global at-risk individuals for pertussis, the size of the pertussis market, the continued clinical advancement of SYN-010 for the treatment of IBS-C, the anticipated requesting end of Phase 2 meeting with FDA, expected initiation of Phase 3 clinical trials for SYN-004 and Phase 2b/3 clinical trial for SYN-010 and the timing of the initiation, and the potential benefits of SYN-004 and SYN-010. These forward-looking statements are based on management's expectations and assumptions as of the date of this press release and are subject to a number of risks and uncertainties, many of which are difficult to predict that could cause actual results to differ materially from current expectations and assumptions from those set forth or implied by any forward-looking statements. Important factors that could cause actual results to differ materially from current expectations include, among others, Synthetic Biologics' product candidates demonstrating safety and effectiveness, as well as results that are consistent with prior results, Synthetic Biologics' ability to initiate clinical trials and if initiated, to complete them on time and achieve desired results and benefits, Synthetic Biologics' ability to successfully design a protocol and a corresponding statistical analysis plan to support the execution of its pivotal clinical trials, Synthetic Biologics' clinical trials continuing enrollment as expected, Synthetic Biologics' ability to obtain regulatory approvals for commercialization of product candidates or to comply with ongoing regulatory requirements, regulatory limitations relating to Synthetic Biologics' ability to promote or commercialize its product candidates for specific indications, acceptance of its product candidates in the marketplace and the successful development, marketing or sale of Synthetic Biologics' products by competitors that render Synthetic Biologics' products obsolete or non-competitive, Synthetic Biologics' ability to maintain its license agreements, the continued maintenance and growth of Synthetic Biologics' patent estate, Synthetic Biologics becoming and remaining profitable, Synthetic Biologics' ability to establish and maintain collaborations, Synthetic Biologics' ability to obtain or maintain the capital or grants necessary to fund its research and development activities, a loss of any of Synthetic Biologics' key scientists or*

management personnel, and other factors described in Synthetic Biologics' Annual Report on Form 10-K for the year ended December 31, 2016 and its other filings with the SEC. The information in this release is provided only as of the date of this release, and Synthetic Biologics undertakes no obligation to update any forward-looking statements contained in this release on account of new information, future events, or otherwise, except as required by law.

- Financial Tables Follow -

**Synthetic Biologics, Inc. and Subsidiaries**  
(in thousands, except share and per share amounts)

**Condensed Consolidated Balance Sheets**

|   | <b>March 31,<br/>2017</b> | <b>December 31,<br/>2016</b> |
|---|---------------------------|------------------------------|
|   | <b>(unaudited)</b>        |                              |
| <b>Assets</b>                                     |                           |                              |
| Cash and cash equivalents                         | \$ 13,471                 | \$ 19,055                    |
| Prepaid expenses and other current assets         | 3,366                     | 2,515                        |
| Property and equipment, net                       | 859                       | 905                          |
| Deposits and other assets                         | 23                        | 23                           |
|   | <b>\$ 17,719</b>          | <b>\$ 22,498</b>             |
| <b>Liabilities and Stockholders' Equity</b>       |                           |                              |
| Current liabilities                               | \$ 16,488                 | \$ 19,757                    |
| Long-term deferred rent                           | 470                       | 492                          |
| Total stockholders' equity                        | 761                       | 2,249                        |
|   | <b>\$ 17,719</b>          | <b>\$ 22,498</b>             |
| <b>Total Liabilities and Stockholders' Equity</b> |                           |                              |

**Condensed Consolidated Statements of Operations**

|  | <b>For the three months ended<br/>March 31,</b> |                    |
|--|---|--------------------|
|  | <b>2017</b>                                     | <b>2016</b>        |
|  | <b>(unaudited)</b>                              |                    |
| <b>Operating Costs and Expenses</b>  |   |                    |
| General and administrative   | \$ 2,090  | \$ 2,426           |
| Research and development   | 6,059   | 8,155              |
| <b>Total Operating Costs and Expenses</b>  | <b>8,149</b>                                    | <b>10,581</b>      |
| <b>Loss from Operations</b>  | <b>(8,149)</b>                                  | <b>(10,581)</b>    |
| <b>Other Income (Expense)</b>  |   |                    |
| Change in fair value of warrant liability  | 5,090   | (498)              |
| Interest income  | 1   | 1                  |
| <b>Total Other Expense, net</b>  | <b>5,091</b>                                    | <b>(497)</b>       |
| <b>Net Loss</b>  | <b>(3,058)</b>                                  | <b>(11,078)</b>    |
| <b>Net Loss Attributable to Non-controlling Interest</b>   | <b>(212)</b>                                    | <b>(233)</b>       |
|  | <b>\$ (2,846)</b>                               | <b>\$ (10,845)</b> |
| <b>Net Loss Attributable to Synthetic Biologics, Inc. and Subsidiaries</b>                         | <b>\$ (0.02)</b>                                | <b>\$ (0.12)</b>   |
| <b>Weighted average number of common shares outstanding during the period - Basic and Dilutive</b> | <b>117,447,260</b>                              | <b>90,826,752</b>  |

To view the original version on PR Newswire, visit <http://www.prnewswire.com/news-releases/synthetic-biologics-reports-first-quarter-2017-operational-highlights-and-financial-results-300451867.html>

SOURCE Synthetic Biologics, Inc.