



## **ARCA BIOPHARMA ANNOUNCES FISCAL YEAR 2016 FINANCIAL RESULTS AND PROVIDES BUSINESS UPDATE**

### **GENETIC-AF Phase 2B/3 Clinical Trial Evaluating Gencaro as Potentially First Genetically-Targeted Treatment for Atrial Fibrillation in Heart Failure Patients**

#### **Outcome of GENETIC-AF Phase 2B Interim Efficacy Analysis Anticipated in the Third Quarter of 2017**

*Westminster, CO, March 21, 2017* – ARCA biopharma, Inc. (Nasdaq: ABIO), a biopharmaceutical company applying a precision medicine approach to developing genetically-targeted therapies for cardiovascular diseases, today reported financial results for the year ended December 31, 2016, and provided a business update.

“During 2016, we continued to make progress advancing the GENETIC-AF clinical trial evaluating Gencaro as potentially the first genetically-targeted treatment for atrial fibrillation,” commented Dr. Michael Bristow, ARCA’s President and Chief Executive Officer. “The American Heart Association estimates that approximately 5.2 million Americans had atrial fibrillation in 2015, with medical and indirect costs totaling an estimated \$31 billion. We believe that a precision medicine approach to drug development, tailoring medical treatment to the individual genetic characteristics of each patient, can potentially enable more effective therapies, improve patient outcomes and reduce healthcare costs. The GENETIC-AF trial interim efficacy analysis outcome expected in the third quarter of this year will be a major milestone for ARCA, the Gencaro development program and our targeted approach to cardiovascular drug development.”

#### **2016 Summary Financial Results**

**Cash, cash equivalents and marketable securities** totaled \$23.5 million as of December 31, 2016, compared to \$38.8 million as of December 31, 2015. ARCA believes that its current cash, cash equivalents and marketable securities will be sufficient to fund its operations, at its projected cost structure, through the end of 2017.

**Research and development (R&D) expenses** for the year ended December 31, 2016 totaled \$12.3 million compared to \$7.1 million for 2015. The \$5.3 million increase in research and development expenses in 2016 as compared to 2015 was primarily due to increased expenses for the GENETIC-AF clinical trial, including contract research organization costs, clinical site initiation and monitoring activities, patient visit costs and increased costs to support expanding the trial into Europe. The Company expects R&D expenses in 2017 to be higher than 2016 as it activates new clinical sites and enrolls additional patients in the GENETIC-AF clinical trial.

**General and administrative (G&A) expenses** for the year ended December 31, 2016 were \$4.3 million compared to \$4.4 million in 2015. The decrease in expenses during 2016 was comprised primarily of 2015 costs related to a reverse stock split in September 2015, with no corresponding transaction in 2016, and lower corporate franchise taxes in 2016 as compared to 2015. ARCA expects G&A expenses in 2017 to be consistent with those in 2016 as it maintains administrative activities to support the GENETIC-AF clinical trial.

**Total operating expenses** for the year ended December 31, 2016 were \$16.6 million compared to \$11.5 million in 2015. The increase in total operating expenses for 2016 was primarily due to the increase in R&D expense due to the increased clinical expense of the GENETIC-AF clinical trial.

**Net loss** was \$16.4 million, or \$1.81 per share, for 2016 compared to \$11.4 million, or \$1.82 per share, for 2015.

### **GENETIC-AF Clinical Trial**

GENETIC-AF is an adaptive, seamless design Phase 2B/3, multi-center, randomized, double-blind, clinical superiority trial comparing the safety and efficacy of Gencaro to TOPROL-XL (metoprolol succinate) for the treatment and prevention of recurrent atrial fibrillation or flutter (AF/AFL) in heart failure patients with reduced left ventricular ejection fraction (HFrEF). Eligible patients will have HFrEF, a history of paroxysmal AF (episodes lasting 7 days or less) or persistent AF (episodes lasting more than 7 days and less than 1 year) in the past 6 months, and the beta-1 389 arginine homozygous genotype that the Company believes responds most favorably to Gencaro. The primary endpoint of the study is time to first event of symptomatic AF/AFL or all-cause mortality. The trial is currently enrolling patients in the United States, Canada and Europe.

### **Phase 2B Interim Efficacy Analysis**

GENETIC-AF, as an adaptive seamless design Phase 2B/3 superiority trial, includes an interim data analysis of efficacy and safety. The GENETIC-AF Data and Safety Monitoring Board (DSMB) will perform a pre-specified interim analysis of unblinded efficacy data when at least 150 patients have evaluable data. A randomized patient has evaluable data either when they experience their first composite endpoint event, AF/AFL or all-cause mortality, or after completion of the 24-week primary endpoint follow-up period. The analysis will be conducted for detection of evidence of safety and superior efficacy of Gencaro versus the active comparator, metoprolol succinate (TOPROL-XL).

The prospectively defined features of this analysis include an estimate of Gencaro effectiveness relative to TOPROL-XL and an assessment of safety as characterized by adverse events. The relative benefit estimate will utilize Bayesian statistical methods to calculate the predictive probability of the Phase 3 patient cohort hazard ratio (a measure of an effect of an intervention on an outcome of interest over time) based on the interim Phase 2B data. Prospectively defined ranges of predictive probabilities have been predetermined to define three potential outcomes based on the projection of the Phase 2B interim results:

- 1) transition the trial to Phase 3 based on a likelihood of achieving a statistically significant

- hazard ratio in favor of Gencaro (evidence of an effectiveness signal consistent with pretrial assumptions) and enroll up to a total of 620 patients (including the Phase 2B patients);
- 2) completion of the Phase 2B stage of the trial including 24-week follow-up of all randomized subjects (approximately 250 patients), based on an intermediate result that is potentially favorable but does not support transition of the trial to Phase 3 or;
  - 3) immediate termination of the trial due to futility.

ARCA, in collaboration with the GENETIC-AF Steering Committee, will determine the most appropriate path forward for the trial based on the DSMB recommendation from this interim analysis and the Company's available capital. The unblinded statistical data available to the DSMB will not be disclosed to ARCA or the public. ARCA projects that the outcome of the DSMB interim analysis and recommendation will be available in the third quarter of 2017.

### **Atrial Fibrillation (AF)**

Atrial fibrillation, the most common sustained cardiac arrhythmia, is considered an epidemic cardiovascular disease and a major public health burden. The estimated number of individuals with AF globally in 2010 was 33.5 million. According to the 2017 American Heart Association report on Cardiovascular Disease, approximately 5.2 million people in the United States had atrial fibrillation in 2015. Hospitalization rates for AF increased by 23% among U.S. adults from 2000 to 2010 and hospitalizations account for the majority of the economic cost burden associated with AF.

### **About ARCA biopharma**

ARCA biopharma is dedicated to developing genetically-targeted therapies for cardiovascular diseases through a precision medicine approach to drug development. ARCA's lead product candidate, Gencaro™ (bucindolol hydrochloride), is an investigational, pharmacologically unique beta-blocker and mild vasodilator being developed for the potential treatment of patients with atrial fibrillation and chronic heart failure. ARCA has identified common genetic variations that it believes predict individual patient response to Gencaro, giving it the potential to be the first genetically-targeted atrial fibrillation prevention treatment. ARCA has a collaboration with Medtronic, Inc. for support of the GENETIC-AF trial. For more information, please visit [www.arcabio.com](http://www.arcabio.com).

### **Safe Harbor Statement**

*This press release contains "forward-looking statements" for purposes of the safe harbor provided by the Private Securities Litigation Reform Act of 1995. These statements include, but are not limited to, statements regarding, potential timing for patient enrollment in the GENETIC-AF trial, potential timeline for GENETIC-AF trial activities and related recommendations of the DSMB, the potential that the data from at least 150 patients will support a recommendation that the GENETIC-AF trial transition to Phase 3, or completion of Phase 2B, the sufficiency of the Company's capital to support its operations, the potential for genetic variations to predict individual patient response to Gencaro, Gencaro's potential to treat atrial fibrillation, future treatment options for patients with atrial fibrillation, and the potential for Gencaro to be the first*

*genetically-targeted atrial fibrillation prevention treatment. Such statements are based on management's current expectations and involve risks and uncertainties. Actual results and performance could differ materially from those projected in the forward-looking statements as a result of many factors, including, without limitation, the risks and uncertainties associated with: the Company's financial resources and whether they will be sufficient to meet the Company's business objectives and operational requirements; results of earlier clinical trials may not be confirmed in future trials, the protection and market exclusivity provided by the Company's intellectual property; risks related to the drug discovery and the regulatory approval process; and, the impact of competitive products and technological changes. These and other factors are identified and described in more detail in ARCA's filings with the SEC, including without limitation the Company's annual report on Form 10-K for the year ended December 31, 2016, and subsequent filings. The Company disclaims any intent or obligation to update these forward-looking statements.*

**Investor & Media Contact:**

Derek Cole

720.940.2163

[derek.cole@arcabio.com](mailto:derek.cole@arcabio.com)

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(Tables Follow)

**ARCA BIOPHARMA, INC.**  
**BALANCE SHEET DATA**  
(in thousands)

	<u>December 31, 2016</u>	<u>December 31, 2015</u>
Cash, cash equivalents & marketable securities	\$23,515	\$38,802
Working capital	\$19,049	\$37,412
Total assets	\$24,629	\$39,574
Total stockholders' equity	\$22,194	\$38,070

**ARCA BIOPHARMA, INC.**  
**STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**

	<u>Years Ended December 31,</u>	
	<b>2016</b>	<b>2015</b>
	(in thousands, except share and per share amounts)	
<b>Costs and expenses:</b>		
Research and development	\$ 12,348	\$ 7,063
General and administrative	4,265	4,392
Total costs and expenses	16,613	11,455
Loss from operations	(16,613)	(11,455)
Interest and other income	169	14
Interest expense	—	(4)
Net loss	<u>\$ (16,444)</u>	<u>\$ (11,445)</u>
Change in unrealized loss on marketable securities	(19)	—
Comprehensive loss	<u>\$ (16,463)</u>	<u>\$ (11,445)</u>
<b>Net loss per share:</b>		
Basic and diluted	\$ (1.81)	\$ (1.82)
<b>Weighted average shares outstanding:</b>		
Basic and diluted	9,067,438	6,289,305