



ARCA BIOPHARMA ANNOUNCES FIRST QUARTER 2017 FINANCIAL RESULTS AND PROVIDES BUSINESS UPDATE

Outcome of GENETIC-AF Phase 2B Interim Efficacy Analysis Anticipated in September 2017

Westminster, CO, May 15, 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 quarter ended March 31, 2017, and provided a business update.

“We’ve made great progress so far this year, recently achieving enrollment of the 200th patient in the GENETIC-AF clinical trial. The strategies we’ve implemented have increased the rate of patient enrollment. We’d like to thank the investigators and the study patients, whose support and enthusiasm suggests a high unmet medical need for this patient population and potentially a substantial market opportunity if Gencaro is approved,” commented Dr. Michael Bristow, ARCA’s President and Chief Executive Officer. “We are focused on the upcoming DSMB interim efficacy analysis that will determine the next steps for this adaptive, seamless design trial – transitioning to Phase 3 and enrolling an additional 370 patients; completing Phase 2B with 250 patients; or, stopping immediately for futility. We expect the outcome of this interim efficacy analysis in September 2017.”

Dr. Bristow further commented, “Atrial fibrillation is considered an epidemic cardiovascular disease based on the pace of increase in incidence in the United States and industrialized countries. There is also increasing evidence that compared to heart failure with sinus rhythm, heart failure with atrial fibrillation responds differently to medical therapy and needs to be approached as a separate disorder. We believe that a precision medicine approach to drug development in atrial fibrillation-heart failure, tailoring medical treatment to the individual genetic characteristics of patient subgroups, can potentially enable more effective therapies, improve patient outcomes and reduce healthcare costs.”

First Quarter 2017 Summary Financial Results

Cash, cash equivalents and marketable securities totaled \$19.2 million as of March 31, 2017, compared to \$23.5 million as of December 31, 2016. 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. ARCA had approximately 9.2 million outstanding shares of common stock as of March 31, 2017.

Research and development (R&D) expenses for the three months ended March 31, 2017 totaled

\$3.2 million compared to \$2.6 million for the corresponding period of 2016, an increase of approximately \$0.7 million. The increase in R&D expenses in the first quarter of 2017 as compared to the first quarter of 2016 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 three months ended March 31, 2017 were \$1.1 million compared to \$1.1 million for the corresponding period in 2016, a net increase of approximately \$60,000. The increase in expenses during the first quarter of 2017 was comprised primarily of higher consulting costs. This increase is partially offset by decreased non-cash, stock-based compensation expense in 2017, as compared to the corresponding period in 2016. ARCA expects G&A expenses in 2017 to be higher than in 2016 as it increases administrative activities to support the GENETIC-AF clinical trial.

Total operating expenses for the three months ended March 31, 2017 were \$4.4 million compared to \$3.7 million for the corresponding period in 2016. The increase in total operating expenses during the first quarter of 2017 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 \$4.3 million, or \$0.48 per share, for the first quarter of 2017 compared to \$3.6 million, or \$0.40 per share, for the first quarter of 2016.

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 ARCA 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

The GENETIC-AF Data 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 to evaluate the evidence for 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 trial Steering Committee, will determine the most appropriate path forward for the trial based on the DSMB recommendation from this interim analysis. The unblinded statistical data available to the DSMB will not be disclosed to the Company or the public. ARCA expects the outcome of this interim efficacy analysis in September 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, with medical and indirect costs totaling an estimated \$31 billion. 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 HFrEF. 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.

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, the potential that the data from 150 patients will support a recommendation that the GENETIC-AF trial transition to Phase 3, the potential timeline for GENETIC-AF trial activities and related recommendations of the DSMB, potential timing for patient enrollment in the GENETIC-AF trial, the sufficiency of ARCA's capital to support its operations, the potential for genetic variations to predict individual patient response to Gencaro, Gencaro's potential to treat AF, future treatment options for patients with AF, and the potential for Gencaro to be the first genetically-targeted AF 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: ARCA's financial resources and whether they will be sufficient to meet its business objectives and operational requirements; results of earlier clinical trials may not be confirmed in future trials; the protection and market exclusivity provided by ARCA'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 Securities and Exchange Commission, including without limitation ARCA's annual report on Form 10-K for the year ended December 31, 2016, and subsequent filings. ARCA disclaims any intent or obligation to update these forward-looking statements.

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(Tables Follow)

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

	<u>March 31, 2017</u>	<u>December 31, 2016</u>
Cash, cash equivalents & marketable securities	\$19,211	\$23,515
Working capital	\$17,336	\$19,049
Total assets	\$20,589	\$24,629
Total stockholders' equity	\$18,036	\$22,194

ARCA BIOPHARMA, INC.
STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(unaudited)

	Three Months Ended	
	March 31,	
	2017	2016
	(in thousands, except share and per share amounts)	
Costs and expenses:		
Research and development	\$ 3,246	\$ 2,594
General and administrative	1,135	1,074
Total costs and expenses	4,381	3,668
Loss from operations	(4,381)	(3,668)
Interest and other income	45	21
Interest expense	(2)	—
Net loss	<u>\$ (4,338)</u>	<u>\$ (3,647)</u>
Change in unrealized loss on marketable securities	10	6
Comprehensive loss	<u>\$ (4,328)</u>	<u>\$ (3,641)</u>
Net loss per share:		
Basic and diluted	\$ (0.48)	\$ (0.40)
Weighted average shares outstanding:		
Basic and diluted	9,094,276	9,053,186