



## **ARCA BIOPHARMA ANNOUNCES SECOND QUARTER 2017 FINANCIAL RESULTS AND PROVIDES BUSINESS UPDATE**

### **Outcome of GENETIC-AF Data and Safety Monitoring Board Phase 2B Interim Efficacy Analysis and Recommendation Anticipated in August 2017**

*Westminster, CO, August 3, 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 June 30, 2017, and provided a business update.

“We continue to make progress in the GENETIC-AF clinical trial with more than 250 patients now randomized,” commented Dr. Michael Bristow, ARCA’s President and Chief Executive Officer. “We are awaiting the outcome of the ongoing DSMB interim efficacy analysis that will recommend the next steps for this adaptive, seamless design trial – transitioning to Phase 3 and enrolling an additional 370 patients; completing Phase 2B with approximately 250 patients; or, stopping immediately for futility. We expect the outcome of this important clinical milestone in August.”

### **Second Quarter 2017 Summary Financial Results**

**Cash, cash equivalents and marketable securities** totaled \$16.0 million as of June 30, 2017, compared to \$23.5 million as of December 31, 2016. ARCA had approximately 10.1 million outstanding shares of common stock as of June 30, 2017. Subsequent to June 30, 2017, the Company sold an aggregate of 1,634,158 shares of common stock through its existing “at the market offering”, raising net proceeds of approximately \$3.9 million. As of July 25, 2017, ARCA has sold all shares available under the prospectus to its registration statement on Form S-3 (No. 333-217459) and does not currently anticipate making any additional sales under this facility. 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 three months ended June 30, 2017 totaled \$4.5 million compared to \$2.9 million for the corresponding period of 2016, an increase of approximately \$1.6 million. R&D expense for the six months ended June 30, 2017 totaled \$7.8 million compared to \$5.5 million for the corresponding period of 2016, an increase of approximately \$2.2 million. The increase in R&D expense in the three and six month periods ended June 30, 2017 was due primarily to the increased expense of ARCA’s 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 June 30, 2017 were \$1.1 million compared to \$1.0 million for the corresponding period in 2016, a net increase of approximately \$23,000. G&A expenses totaled \$2.2 million for the six months ended June 30, 2017 as compared to \$2.1 million for the corresponding period in 2016, a net increase of approximately \$84,000. The increase for the three and six month periods is comprised primarily of higher consulting costs and professional fees. These increases are partially offset by decreased non-cash, stock-based compensation expense in 2017, as compared to the corresponding periods 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 June 30, 2017 were \$5.6 million compared to \$3.9 million for the corresponding period in 2016. Total operating expenses for the six months ended June 30, 2017 were \$9.9 million compared to \$7.6 million for the corresponding period in 2016. The increase in total operating for the three and six month periods 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 \$5.5 million, or \$0.59 per share, for the second quarter of 2017 compared to \$3.9 million, or \$0.43 per share, for the second quarter of 2016. Net loss for the six months ended June 30, 2017 was \$9.9 million, or \$1.07 per share, compared to \$7.5 million, or \$0.83 per share, for the corresponding period in 2016.

## **GENETIC-AF Clinical Trial**

GENETIC-AF is an adaptive, seamless design Phase 2B/3, multi-center, randomized, double-blind, superiority clinical 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 GENETIC-AF trial design has been reviewed by the FDA. The trial is currently enrolling patients in the United States, Canada and Europe.

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

The GENETIC-AF Data and Safety Monitoring Board (DSMB) is performing a pre-specified interim analysis of unblinded efficacy data. The primary 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

primary analysis method will generate predictive probability of success (PPoS) values that will be compared to prespecified PPoS boundaries constructed from Bayesian statistical modeling. Prospectively defined PPoS ranges 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, 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, if the PPoS results fall below the boundary for completion as a Phase 2 trial.

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. The Company expects to announce the DSMB's recommendation based on this interim analysis in August 2017. The unblinded statistical data available to the DSMB will not be disclosed to ARCA or the public.

### **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<sup>TM</sup> (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. The Gencaro development program has been granted Fast Track designation by the U.S. Food and Drug Administration (FDA). 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, the potential that the data from the interim analysis will support a recommendation that the GENETIC-AF trial transition to Phase 3 or the completion of Phase 2, 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.*

**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)  
(unaudited)

	<u>June 30, 2017</u>	<u>December 31, 2016</u>
Cash, cash equivalents & marketable securities	\$16,007	\$23,515
Working capital	\$14,136	\$19,049
Total assets	\$17,296	\$24,629
Total stockholders' equity	\$14,747	\$22,194

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

	<b>Three Months Ended</b>		<b>Six Months Ended</b>	
	<b>June 30,</b>		<b>June 30,</b>	
	<u>2017</u>	<u>2016</u>	<u>2017</u>	<u>2016</u>
(in thousands, except share and per share amounts)				
<b>Costs and expenses:</b>				
Research and development	\$ 4,508	\$ 2,913	\$ 7,754	\$ 5,507
General and administrative	1,051	1,028	2,186	2,102
Total costs and expenses	5,559	3,941	9,940	7,609
Loss from operations	(5,559)	(3,941)	(9,940)	(7,609)
Interest and other income	39	47	84	68
Interest expense	(2)	—	(4)	—
Net loss	<u>\$ (5,522)</u>	<u>\$ (3,894)</u>	<u>\$ (9,860)</u>	<u>\$ (7,541)</u>
Change in unrealized loss on marketable securities	4	7	14	13
Comprehensive loss	<u>\$ (5,518)</u>	<u>\$ (3,887)</u>	<u>\$ (9,846)</u>	<u>\$ (7,528)</u>
<b>Net loss per share:</b>				
Basic and diluted	\$ (0.59)	\$ (0.43)	\$ (1.07)	\$ (0.83)
<b>Weighted average shares outstanding:</b>				
Basic and diluted	9,324,822	9,065,922	9,210,186	9,059,554