



**ARCA BIOPHARMA REPORTS TOPLINE PHASE 2B RESULTS
FOR GENETIC-AF CLINICAL TRIAL**

**Gencaro Demonstrates Comparable Efficacy to Active Control and
Trend for Potential Gencaro Superiority in US Patient Cohort**

**ARCA Anticipates Meeting with the U.S. FDA in the Second Quarter of 2018
to Review Phase 2 Data and Phase 3 Development Plan**

Management to Host Conference Call & Webcast Today at 9:00 am ET

Westminster, CO, February 26, 2018 – [ARCA biopharma, Inc.](#) (Nasdaq: ABIO), a clinical-stage biopharmaceutical company applying a precision medicine approach to developing genetically-targeted therapies for cardiovascular diseases, today announced clinical results from [GENETIC-AF](#), a Phase 2B, double-blind, superiority clinical trial evaluating Gencaro™ (bucindolol hydrochloride) as a genetically-targeted treatment for atrial fibrillation (AF) in patients with heart failure and reduced left ventricular ejection fraction (HFrEF). In all patients, Gencaro demonstrated a similar treatment benefit compared to the active control, metoprolol succinate (TOPROL-XL). In U.S. patients (127 of 267 total patients), a trend for potential superior benefit in favor of Gencaro (approximately 30% risk reduction over TOPROL-XL), was observed for the primary endpoint of time to recurrence of AF. Additionally, in U.S. patients, Gencaro demonstrated a trend for potential superior benefit in favor of Gencaro (approximately 51% risk reduction over TOPROL-XL) in a subset of patients who underwent continuous heart rhythm monitoring with Medtronic implanted devices. Safety data indicated that Gencaro was generally safe and well-tolerated in the AF/heart failure (HF) population investigated with a safety profile similar to TOPROL-XL.

GENETIC-AF enrolled 267 patients from the United States, Canada and Europe. The primary analysis was conducted to evaluate the evidence of safety and superior efficacy of Gencaro versus an active control with demonstrated effectiveness and safety in this patient population TOPROL-XL. The primary endpoint of the trial was time to recurrent AF, atrial flutter (AFL) or all-cause mortality (ACM). The trial was not powered to conventional significance for this endpoint and utilized Bayesian statistical modeling of predictive probability of success (PPoS) of the primary endpoint to estimate outcome if the trial had enrolled 620 patients with 330 primary events.

In all patients, Gencaro demonstrated a similar treatment benefit compared to the active control, TOPROL-XL (143 total events, hazard ratio of 1.01 (95% confidence interval: 0.71, 1.42), which was associated with a PPoS of 14%. In the U.S. patient cohort of 127 patients (approximately 50% of all patients and events), a trend for potential superior benefit in favor of Gencaro over TOPROL-XL was observed (73 events, hazard ratio 0.70, [95% confidence interval: 0.41, 1.19]), with a PPoS

of 61%, which was greater than the prespecified criteria set by the company to proceed to Phase 3 development. The Company believes the difference in treatment effects between the overall and U.S. patient cohorts was primarily due to results in two non-U.S. countries exhibiting hazard ratios >1.0. The differences between patients enrolled at these sites versus the U.S. and other country cohorts are being investigated.

“The U.S. data support our pre-trial assumptions and provide contemporary information to potentially design Phase 3 development of Gencaro,” commented Dr. Debra Marshall, Senior Vice-President of Medical Affairs. “I would like to thank our clinical investigators, as well as the patients and their families, for their participation in this study.”

A subgroup of patients underwent continuous (24/7) heart rhythm monitoring via Medtronic implanted loop recorders or other Medtronic implanted therapeutic devices (e.g., ICDs, CRTs) to evaluate daily AF burden. AF burden is defined as the amount of time per day a patient experiences AF. A prespecified time-to-first event analysis was conducted using a total AF burden of at least 6 hours per day to define an event of AF recurrence. In this analysis, hazard ratios of 0.75 (0.43, 1.32) and 0.49 (0.24, 1.04) were observed in the overall (n=69) and U.S. patient (n=42) cohorts, respectively.

Gencaro was generally safe and well-tolerated, with 84% of patients attaining their target dose compared to 72% of patients receiving TOPROL-XL. The most frequently reported adverse events were similar in both groups and consistent with the known safety profile of the beta-blocker class of drugs. Adverse events assessed as related to study drug by the investigator occurred in 23.8% of patients in the Gencaro group and in 30.1% of patients in the TOPROL-XL group. Of note, adverse events of bradycardia were less frequently reported in the Gencaro group (3.7%) compared to patients receiving TOPROL-XL (12.0%). During the 24-week efficacy follow-up period there were three deaths (ACM) in the TOPROL-XL group and none in the Gencaro group. Three patients died in the long-term treatment extension period after receiving Gencaro for more than a year.

“We are pleased to share these results from our GENETIC-AF clinical trial. It should be emphasized that the control drug in the study, TOPROL-XL, although not FDA approved for this indication, has demonstrated efficacy in preventing AF in HF_{rEF}. While we did observe some regional heterogeneity in effectiveness of Gencaro, we believe the treatment response observed in the U.S. population, which represents approximately half of the overall study population, support continued development of Gencaro as a genetically-targeted treatment for atrial fibrillation,” commented Dr. Michael Bristow, ARCA’s President and CEO. “As in all Phase 2 trials, which in part are conducted to elicit information relevant to the design for Phase 3, elucidation of the reason(s) for regional heterogeneity could prove valuable for Phase 3 trial design.”

ARCA anticipates meeting with the U.S. Food and Drug Administration (FDA) in the second quarter of 2018 to review Gencaro Phase 2 data and potential Phase 3 development plan.

ARCA ended 2017 with cash, cash equivalents and marketable securities totaling \$11.8 million (unaudited) and believes that these funds, with additional net proceeds of approximately \$3.4 million (unaudited) raised in January 2018 through its existing “At-the-Market” offering facility,

will be sufficient to fund its operations, at its projected cost structure, through the end of 2018.

GENETIC-AF Clinical Trial

GENETIC-AF is a Phase 2B, 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 atrial flutter (AF/AFL) in heart failure patients with reduced left ventricular ejection fraction (HFrEF). Eligible patients had 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.

Conference Call and Webcast

ARCA will hold a conference call and live audio webcast today, Monday, February 26, 2018 at 9:00 am Eastern (7:00am Mountain) to discuss results from the GENETIC-AF clinical trial.

Participant Toll-Free Dial-In Number: 1-877-270-2148

Participant International Dial-In Number: 1-412-902-6510

Ask to be joined into the ARCA biopharma call.

Presentation slides to accompany the conference call have been posted to the Presentations page in the investor section of the ARCA website, www.arcabio.com.

Interested parties may access a live audio webcast of the conference call at: <https://services.choruscall.com/links/abio180226.html>. Please connect to the ARCA website several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary. The webcast will be archived for 90 days.

About Gencaro™ (bucindolol hydrochloride)

Gencaro (bucindolol hydrochloride) is an investigational, pharmacologically unique beta-blocker and mild vasodilator being developed for the treatment of AF. Gencaro is considered part of the beta-blocker class of compounds because of its property of blocking both beta-1 and beta-2 receptors in the heart. The blocking of these receptors prevents them from binding with other molecules, primarily the neurotransmitter norepinephrine, which activate these receptors. ARCA believes that Gencaro is well-tolerated in cardiovascular patients because of its mild vasodilator effects. Originally developed by Bristol-Myers Squibb, the active pharmaceutical ingredient in Gencaro, bucindolol hydrochloride, has been tested clinically in approximately 4,500 patients, including over 3,250 patients in eight clinical trials in HFrEF patients. Gencaro was the subject of a Phase 3 HF mortality trial in 2,708 patients, mostly in the United States (the “BEST trial”). The BEST trial included a DNA bank of over 1,000 patients, which was used to evaluate the effect of

genetic variation on patients' response to Gencaro.

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 AF and HFrEF which recently completed a Phase 2B clinical trial. 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 AF 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 FDA. ARCA also plans to develop AB171, a thiol-substituted isosorbide mononitrate, as a potential genetically-targeted treatment for peripheral arterial disease (PAD) and for HF. 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 potential Phase 3 development plans for Gencaro, the expected features and characteristics of Gencaro, including 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, the potential for Gencaro to be the first genetically-targeted AF prevention treatment and the ability of ARCA's financial resources to support its operations through the end of 2018. 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; ARCA may not be able to raise sufficient capital on acceptable terms, or at all, to continue development of Gencaro or to otherwise continue operations in the future; 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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