

Westminster, CO, April 26, 2017 – ARCA biopharma, Inc. (Nasdaq: ABIO), a biopharmaceutical company applying a precision medicine approach to developing genetically-targeted therapies for cardiovascular diseases, today announced that the 200th patient has been randomized into the GENETIC-AF clinical trial evaluating GencaroTM (bucindolol hydrochloride) as potentially the first genetically-targeted treatment for atrial fibrillation (AF).

"The enrollment strategies we've implemented have increased the rate of patient enrollment. We'd like to thank the investigators and enrolled 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 CEO. "With the Phase 2B portion of the trial nearing completion, we are focused on the upcoming interim efficacy analysis that will determine the next steps of this adaptive, seamless design trial. We expect the outcome of this interim efficacy analysis in September 2017."

GENETIC-AF Clinical Trial

GENETIC-AF is an adaptive, seamless design Phase 2B/3, multi-center, randomized, doubleblind, 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 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.

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, GencaroTM (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 <u>www.arcabio.com</u>.

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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