Arena Pharmaceuticals Reports Positive Long-Term Data from the Ongoing Open-Label Extension of the Phase 2 Trial Evaluating Ralinepag for Treatment of Pulmonary Arterial Hypertension

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- Ralinepag demonstrated durable, long-term improvements in both PVR and 6MWD
- Favorable long-term tolerability profile demonstrated

SAN DIEGO, Oct. 2, 2018 /PRNewswire/ -- Arena Pharmaceuticals, Inc. (Nasdaq: ARNA) today announced positive data from a planned interim analysis of the ongoing open-label extension of the Phase 2 trial of its investigational drug candidate ralinepag, a next-generation, oral, selective and potent prostacyclin receptor agonist in development for the treatment of pulmonary arterial hypertension (PAH).

Open-Label Extension of Phase 2 Trial Design
This is an open-label extension study evaluating the long-term safety, tolerability and efficacy of ralinepag in 45 patients (85% of study completers) who completed the Phase 2 randomized study. In the extension study, patients originally randomized to ralinepag continued on active therapy (N=30); patients randomized to placebo switched to ralinepag (N=15). Key efficacy measurements include pulmonary vascular resistance (PVR) and 6-minute walk distance (6MWD).

Key Efficacy and Safety Measurements
Patients who continued on ralinepag in the open-label extension had a median treatment duration of 1.8 years (range 1.2-3.4 years) at the time of right heart catheterization (RHC). In these patients, sustained improvements from baseline in the original study were observed for PVR (219 dyn*s*cm $^{-5}$ median reduction, $p = 0.002$) and 6MWD (49.8 meters mean improvement; $p = 0.003$). Patients switching from placebo to active drug had a median ralinepag treatment duration of 1.4 years (range 0.9-2.3 years) at the time of RHC. In these patients, a similar magnitude of improvement was observed for PVR (214 dyn*s*cm $^{-5}$ median reduction, $p = 0.206$) and 6MWD (69.8 meters mean improvement; $p = 0.010$). In both groups, these long-term changes in PVR and 6MWD were observed in a population where the majority of patients were already receiving dual combination PAH background therapy.

Adverse events (AEs) observed in this extension study were consistent with the known profile of prostacyclin therapies for the management of PAH, with headache and nausea being the most commonly reported. Among patients who continued ralinepag in the open-label extension, the incidence rate of AEs was lower relative to the randomized Phase 2 study, suggesting that AEs related to tolerability are reduced after initial drug titration.

"We are pleased with the long-term safety, tolerability and efficacy that ralinepag has demonstrated in the open-label extension of our Phase 2 trial. This is the first time an oral prostacyclin has shown durable, long-term improvements on hemodynamic and functional measures. Patients continuing ralinepag from the original study clearly maintained improvements in PVR and 6MWD. Those patients switching from placebo to ralinepag in this extension trial demonstrated a similar magnitude of effect on PVR and 6MWD, although the smaller sample size limits some of the statistical comparisons. These data reinforce our belief that PAH patients can truly benefit from ralinepag's improved receptor potency and extended pharmacokinetics," said Preston Klassen, MD, MHS, Executive Vice President, Research and Development and Chief Medical Officer of Arena. "Ralinepag has the potential to offer the pharmacokinetic and pharmacodynamic advantages of continuously infused IV prostacyclin with the ease of a once-daily oral tablet."

"It is encouraging to gain additional insight into the long-term safety and efficacy of ralinepag. I look forward to seeing data from the Phase 3 ADVANCE program and the effect that ralinepag may have when added to PAH standard of care," said Vallerie McLaughlin, M.D., Kim A. Eagle MD Endowed Professor of Cardiovascular Medicine at the University of Michigan and Director of the Pulmonary Hypertension Program[1]. "These data validate progress towards bringing a new therapeutic option to patients suffering from PAH, a devastating disease with significant unmet medical need despite available treatments."

About Ralinepag
Ralinepag (APD811) is a next-generation, oral, selective potent, once-daily IP receptor agonist intended for the treatment of pulmonary arterial hypertension (PAH). Arena discovered and developed this drug candidate internally. Ralinepag's potency on vasodilation, inhibition of proliferation of vascular smooth muscle cells, and inhibition of platelet aggregation, combined with an extended half-life, support its application as a potentially best-in-class agent for the treatment of PAH.

Ralinepag is an investigational compound that is not approved for any use in any country.

About Arena Pharmaceuticals
Arena Pharmaceuticals is focused on delivering novel, transformational medicines with optimized pharmacology and pharmacokinetics to patients globally. Arena's proprietary pipeline includes multiple potentially first- or best-in-class programs with broad clinical utility. The most advanced investigational clinical programs are ralinepag (APD811), in a Phase 3 program for pulmonary arterial hypertension (PAH), and etrasimod (APD334), expected to commence a Phase 3 program for ulcerative colitis (UC) and a program in Crohn's disease (CD), and which has potential utility for a broad range of immune and inflammatory conditions. Arena is also evaluating olorinab (APD371) for the treatment of gastrointestinal pain, as well as other drug candidates in earlier research and development stages.

In addition, Arena has several collaborations including with Everest Medicines Limited (ralinepag and etrasimod in Greater China and select Asian countries), Axovant Sciences GmbH (nelotanserin - Phase 2), Boehringer Ingelheim International GmbH (undisclosed target - preclinical), Outpost Medicine, LLC (undisclosed target – preclinical), and Eisai Co., Ltd. and Eisai Inc. (BELVIQ® - marketed product).

Forward-Looking Statements
Certain statements in this press release are forward-looking statements that involve a number of risks and uncertainties. These forward-looking statements may be identified by introductory words such as "in development for," "evaluating," "suggesting," "belief," "can," "potential," "look forward to," "may," "progress towards," "intended for," "potentially," "focused on," "expected," "evaluating," or words of similar meaning, or by the fact that they do not relate strictly to historical or current facts. Such forward-looking statements include, without limitation, statements regarding the intention and plan to progress ralinepag's development; the potential for reduced adverse events related to tolerability after initial drug titration; ralinepag's potential, including the potential for its potency and pharmacokinetics to offer benefits to patients, its potential to offer the pharmacokinetic and pharmacodynamic advantages of continuously infused IV prostacyclin with the ease of a once-daily oral tablet, and its potential to bring a new therapeutic option to patients suffering with unmet medical need; the potential of Arena's compounds in its pipeline, including to be first- or best-in-class programs and their utility; and Arena's focus, goals, strategy, clinical programs and collaborations. For such statements, Arena claims the protection of the Private Securities Litigation Reform Act of 1995. Actual events or results may differ materially from Arena's expectations. Factors that could cause actual results to differ materially from the forward-looking statements include the following: the announced data are based on an interim analysis of certain key measurements, and such interim data or analysis may change following a more comprehensive review of the data, and such interim data or analysis may not accurately reflect the final results of the study; the reported-on trial was not a placebo-controlled study; results of clinical trials and other studies are subject to different interpretations and may not be predictive of future results; nonclinical and clinical data are voluminous and detailed, and regulatory agencies may interpret or weigh the importance of data differently and reach different conclusions than Arena or others, request additional information, have additional recommendations or change their guidance or requirements before or after approval; the timing and outcome of research, development and regulatory review is uncertain; we expect to need additional funds to advance all of our programs, and you and others may not agree with the manner we allocate our resources; our drug candidates may not advance in development or be approved for marketing; clinical trials and other studies may not proceed at the time or in the manner expected or at all; enrolling patients in our ongoing and intended clinical trials is competitive and challenging; unexpected or unfavorable new data; risks related to developing and commercializing drugs; risks related to relying on partners and other third parties; Arena's and third parties' intellectual property rights; and satisfactory resolution of litigation or other disagreements with others. Additional factors that could cause actual results to differ materially from those stated or implied by Arena's forward-looking statements are disclosed in Arena's filings with the Securities and Exchange Commission (SEC), including but not limited to Arena's most recent Annual Report on Form 10-K and Quarterly Report on Form 10-Q. These forward-looking statements represent Arena's judgment as of the time of this release. Arena disclaims any intent or obligation to update these forward-looking statements, other than as may be required under applicable law.

[1] Dr. McLaughlin receives consulting fees from Arena.

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