Idera Pharmaceuticals Announces Name Change to Aceragen, Inc. and Provides Near-Term Strategic Outlook

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Company completes preferred stock conversion, implements reverse stock split, and regains Nasdaq compliance

Company positioned for success with late-stage rare disease portfolio and multiple clinical milestones in 2023

DURHAM, N.C. and EXTON, Pa., Jan. 17, 2023 (GLOBE NEWSWIRE) -- Idera Pharmaceuticals, Inc. ("Idera") (Nasdaq: IDRA), a clinical-stage biopharmaceutical company committed to transforming the care of people living with rare pulmonary and rheumatic diseases, today announced the Company has changed its name and symbol to Aceragen, Inc. ("Aceragen," the "Company," "we," "us," or "our") and "ACGN". Additionally, the Company's stockholders approved the conversion into common stock of the Series Z preferred shares resulting from the previously announced merger with Aceragen and authorized a reverse split of common stock. The Company's Board of Directors has approved the reverse stock split at a ratio of 1-for-17 shares. As a result of these changes, which will be effective upon the market open on Wednesday, January 18, 2023, the Company will be in compliance with all applicable Nasdaq listing standards. Nasdaq has issued an approval letter confirming Aceragen's listing.

"We are excited to have completed the transformation of Aceragen via our merger with Idera and the subsequent adjustments to our stock and Nasdaq listing. We believe this transition strengthens our portfolio of late-stage clinical assets in cystic fibrosis and Farber disease and aligns with our goal of delivering important therapies for people living with rare diseases," stated John Taylor, Aceragen's Chief Executive Officer. "During the course of this year, we anticipate the achievement of significant clinical milestones that include two Phase 2 data read-outs for ACG-701, as well as lifting of the clinical hold and advancing toward the initiation of our Phase 2/3 trial in Farber disease for ACG-801."

"With the positive result of the stockholder vote behind us, I share in John's excitement and optimism for Aceragen and look forward to the progress that this team will make for patients in need," added Vincent J. Milano, Chair of Aceragen's Board of Directors.

Clinical Development Overview
Aceragen has a portfolio of late-stage clinical assets in cystic fibrosis and Farber disease with clinical milestones anticipated in 2023.

ACG-701 for Acute Pulmonary Exacerbations in Cystic Fibrosis
ACG-701 is a proprietary oral, loading dose formulation of sodium fusidate being developed as a treatment for acute pulmonary exacerbations ("PEx") associated with cystic fibrosis ("CF"), a major factor driving lung function decline in people living with CF. Sodium fusidate has an established clinical efficacy and safety profile from more than 50 years of use in other countries, including as part of CF PEx treatment guidelines in the United Kingdom and Australia. Despite this, the compound has never been approved by the FDA and represents a new and potentially powerful approach in the United States to address the infection, inflammation, and enhanced mucin expression that are hallmark features of CF PEx.

A Phase 2 trial of ACG-701 in CF PEx (the REPRIEVE study), a randomized double-blinded, placebo-controlled study, was initiated in December 2022 at clinical sites in the United States in collaboration with the CF Foundation's Therapeutic Development Network (TDN). The CF Foundation has also provided funding of $3.5 million in support of the study. If approved, ACG-701 would represent the first product in the United States indicated for the treatment of newly diagnosed CF PEx patients. Data from the REPRIEVE study is expected in 2H 2023. The FDA has granted Orphan Drug Designation, Fast Track and Qualified Infectious Disease Product (QIDP) status to ACG-701 for CF PEx.

ACG-701 for Melioidosis
Aceragen has also executed an ~$50 million development partnership with the Department of Defense's Defense Threat Reduction Agency ("DTRA") to investigate ACG-701 as a potential medical countermeasure for melioidosis, a life-threatening infection caused by the *B. pseudomallei* pathogen. This program is centered around a Phase 2 trial, the TERRA study (NCT05105035), which is a randomized double-blind, placebo-controlled trial conducted in hospitalized melioidosis patients. TERRA was initiated in May 2022 and continues to actively enroll patients with an independent DMC review planned during Q1 2023 and a data read-out expected in 2H 2023.

ACG-801 for Farber Disease
ACG-801, recombinant human acid ceramidase, is an investigational biologic in development to be the first-ever enzyme replacement therapy for the treatment of Farber disease, a progressive, severe, and life-threatening lysosomal storage disorder that is caused by the monogenic deficiency of acid ceramidase. The biochemical hallmark of Farber disease is the loss of acid ceramidase enzyme activity leading to abnormal accumulation of ceramide, profound macrophage-driven inflammation and multi-organ disease affecting bone and joints, cartilage, the immune system, central nervous system, and the lungs. Complications of the disease are life threatening, with many patients dying in the first years of life. There are no ceramide-targeted medications currently available that can alter the disease natural history.

The Company expects to initiate the ADVANCE clinical study for ACG-801 in Farber disease, a randomized, double-blind, placebo-controlled, first-in-human study, in the first quarter of 2024 with data expected in the first quarter of 2025. Due to the ultra-rare nature of Farber disease, if successful, this study has the potential to support registration of the product. The FDA has granted Orphan Drug, Fast Track, and Rare Pediatric Disease designations for ACG-801. Rare pediatric disease designation permits priority review voucher eligibility, upon FDA marketing authorization.

Clinical Milestone Summary

- ACG-701 - REPRIEVE study in CF PEx, data expected 2H 2023
- ACG-701 – TERRA study in melioidosis, data expected 2H 2023
- ACG-801 – ADVANCE study in Farber disease, initiation expected in Q1 2024

Projected cash available is expected to provide the Company with capital runway into Q3'2023.

About Aceragen, Inc.
Aceragen is a clinical-stage biopharmaceutical company committed to transforming the care of people living with rare pulmonary and rheumatic diseases. Our portfolio includes late-stage programs based on well-established biological principles that we are developing to be innovative therapeutics capable of addressing the unmet medical needs of individuals living with rare diseases. To learn more about us and our programs, please visit Aceragen.com.

Forward-Looking Statements
This press release contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. All statements, other than statements of historical fact, included or incorporated in this press release, including, without limitation, statements regarding the Company's new development opportunities, clinical trials and studies, product designation and/or status, financial position, funding for continued operations, cash reserves, projected costs, prospects, clinical trials, plans, expectations, strategies, projections and objectives of management, are forward-looking statements. The words "believes," "anticipates," "estimates," "plans," "expects," "intends," "may," "could," "should," "potential," "likely," "projects," "continue," "will," “schedule,” and "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. These forward-looking statements are predictions based on our current expectations and projections about future events and various assumptions. We cannot guarantee that we will achieve the plans, intentions, or expectations disclosed in our forward-looking statements and you should not place undue reliance on our forward-looking statements. These forward-looking statements involve known and unknown risks, uncertainties, and other factors, which may be beyond our control, and which may cause our actual results, performance, or achievements to differ materially from future results, performance, or achievements expressed or implied by such forward-looking statements. There are a number of important factors that could cause our actual results to differ materially from those indicated or implied by its forward-looking statements including, without limitation: whether we will be able to successfully integrate the acquired operations; whether our cash resources will be sufficient to fund continuing operations; and newly acquired operations,; whether our products will advance into or through the clinical trial process when anticipated or at all or warrant submission for regulatory approval; whether such products will receive approval from the U.S. Food and Drug Administration or equivalent foreign regulatory agencies; whether our products receive approval, they will be successfully distributed and marketed; and whether our collaborations will be successful. All forward-looking statements included in this press release are made as of the date hereof and are expressly qualified in their entirety by this cautionary notice, including, without limitation, those risks and uncertainties described in the Company’s Annual Report on Form 10-K for the year ended December 31, 2021, and otherwise in the Company’s subsequent filings and reports filed with Securities and Exchange Commission. The Company does not assume any obligation to update any forward-looking statements and it disclaims any intention or obligation to update or revise any forward-looking statement, whether as a result of new information, future events, or otherwise, except as may be required by law.

Please direct questions to:

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