Aceragen Announces Acquisition of Arrevus, Expands Late-Stage Rare Disease Pipeline

Combination advances strategy of building multi-product commercial rare disease company

Portfolio targeting Farber disease, Cystic Fibrosis, and orphan infectious diseases

RALEIGH-DURHAM, NC – October 28, 2021 – Aceragen, Inc., a biopharmaceutical company focused on advancing transformational therapeutics for rare and orphan diseases, today announced the acquisition of Arrevus, Inc., a clinical-stage biopharmaceutical company developing therapies for orphan infectious diseases with high unmet need. The transaction adds ARV-1801 (now ACG-721), an oral, small molecule treatment in late-stage development for indications including cystic fibrosis, as well as several preclinical programs with potential application to other rare diseases. Arrevus' President and CEO, Carl Kraus, MD will join Aceragen as Chief Medical Officer.

"The acquisition of Arrevus represents a major step forward for Aceragen in executing on its stated goal of building a multi-product rare disease company that aspires to commercialize its own products," said John Taylor, President and CEO, Aceragen. "Adding Arrevus' clinical and regulatory capabilities along with its lead program in late-stage development, advances our transition to a diversified, rare-disease-focused biotech company with an amazing growth trajectory. We don't see many other companies in the rare and orphan disease space that have the same combination of superior talent and a late-stage portfolio of targeted, high-value assets."

Strategic Rationale

Aceragen intends to launch advanced clinical studies for both ACG-801 and ACG-721 in rare and orphan indications with overlapping commercial footprints. Specifically, the addition of ACG-721 creates a foundation in the cystic fibrosis field on which to investigate ACG-801's complimentary mechanism.

- ACG-801 is an investigational form of recombinant human acid ceramidase (rhAC) designed to address the enzyme deficiency which is the cause of Farber disease.
    - Registrational trial for Farber disease is planned for the second half of 2022
- ACG-721 is intended for the treatment of cystic fibrosis pulmonary exacerbations and other orphan infectious diseases.
    - Phase 2/3 trial for cystic fibrosis pulmonary exacerbations expected to begin in the second half of 2022
    - Phase 2 trial for additional orphan indication expected to begin the first half of 2022
    - 600 patient treatment safety database already established
- Arrevus team adds deep scientific knowledge of the infectious disease field and significantly strengthens clinical and regulatory expertise
- Deal is expected to be accretive for Aceragen

"I'm really proud of Arrevus' record of achievement in bringing forward our lead candidate and in establishing development efforts and collaborations for our earlier programs," said Dr. Kraus. "My team is excited to join Aceragen. Together we can realize the full potential of the combined portfolio for maximum impact on patient needs."

About Aceragen

Aceragen is a biopharmaceutical company developing innovative therapeutics for rare and orphan diseases. The Company is advancing ACG-801 (rhAC) as an investigational enzyme replacement therapy for the treatment of patients with Farber disease and potentially other diseases associated with the dysregulation of ceramide metabolism, including cystic fibrosis. Its other development lead is ACG-721, an oral small molecule candidate for cystic fibrosis pulmonary exacerbations and related orphan infectious diseases.

For more information, please visit www.aceragen.com.

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