PRESS RELEASE

AnaCardio Reports Strong Phase 2a Topline Results for AC01 in patients with heart failure and reduced ejection fraction (HFrEF); Paving the Way for Rapid Advancement to Phase 2b

December 10, 2025, Stockholm, Sweden – AnaCardio, a clinical-stage biopharmaceutical company developing novel contractile agents for heart failure, today announces strong topline results from the Phase 2a portion of its GOAL-HF1 clinical study program evaluating AC01 in patients with heart failure and reduced ejection fraction (HFrEF). AC01 demonstrated a favorable safety and tolerability profile and exploratory efficacy assessments showed encouraging, consistent signals of rapid and sustained improvements in hemodynamics, and cardiac structure and function over 28 days of treatment. These findings clearly justify advancing AC01 into a Phase 2b study and reinforce the company's confidence in its continued clinical development.

The Phase 2a part of GOAL-HF1 was designed in collaboration with a panel of leading heart failure experts and enrolled 26 patients across 13 European heart failure centers, randomized to receive 1 mg or 3 mg AC01, or placebo twice daily for 28 days.

Key findings

- AC01 demonstrated a favorable safety and tolerability profile, with no treatment-emergent adverse events leading to discontinuation, no drug-related serious adverse events and no evidence of ischemia, new onset of sustained arrhythmias, morphological or conduction abnormalities, or clinically relevant effects of AC01 on blood pressure.
- Although the study was not powered for placebo-controlled efficacy evaluation, exploratory findings showed consistent rapid and sustained improvements across hemodynamics, and cardiac structure and function.
- AC01 pharmacokinetics were predictable and dose-proportional, and target engagement was confirmed by a dose-dependent increase in growth hormone release, consistent with previous AC01 data.

Patrik Strömberg, CEO of AnaCardio, commented: *"These Phase 2a results strengthen our conviction in AC01's potential to transform the treatment landscape for heart failure. The consistency across safety, tolerability, and markers of cardiac function improvements gives us strong confidence to rapidly progress AC01 into late-stage development. We believe AC01 is emerging as a highly differentiated, first-in-class therapy with the potential to deliver meaningful improvements in cardiac function for patients who need better treatment options."*

Lars Lund, Founder of AnaCardio and Professor at Karolinska Institutet, added: *"The consistency we now observe from preclinical studies through Phase 1b and into Phase 2a strengthens our belief that AC01 has the potential to become a transformative therapy for HFrEF and to address one of the highest unmet medical needs in cardiovascular medicine. This alignment across stages of development is compelling and reinforces our commitment to advancing AC01 with confidence into the next phase of clinical development."*

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Riccardo Braglia, Executive Chairman of Helsinn, added: *"We are extremely pleased to see AC01, which is exclusively licensed from Helsinn, deliver such compelling clinical signals. These results reinforce the strength of the underlying science and validate our decision to partner with AnaCardio on its continued development. Their focused execution and clinical expertise are clearly accelerating the program's progress. We believe AC01 has the potential to redefine how contractility is safely enhanced in heart failure, and we are excited to see the strong momentum AnaCardio is building."*

Rationale for continued development

AnaCardio is developing AC01 as the first oral contractile agent designed to improve cardiac output without the safety concerns associated with conventional inotropes. AC01's mode of action — calcium sensitization through ghrelin receptor agonism — aims to increase cardiac contractility while minimizing arrhythmic and ischemic risks.

Results from Phase 1b and now Phase 2a together highlight:

- A favourable and consistent safety and tolerability profile across all evaluated dose levels
- Robust evidence of target engagement, aligned with the proposed mechanism of action
- Encouraging functional signals indicative of improved myocardial performance

These findings support AC01 as a high-potential, unique therapeutic candidate that could address a major unmet need in chronic HFrEF.

Next steps — advancing to Phase 2b

The Phase 2a results provide a unified dataset across safety, pharmacokinetics, biomarkers, and exploratory functional outcomes. Based on these findings, AnaCardio will advance AC01 into Phase 2b, a larger and longer-term clinical study designed to more rigorously evaluate efficacy and patient benefit. Preparations are underway and the company anticipates initiating the study mid-2026. AnaCardio believes AC01 is emerging as a highly promising first-in-class therapeutic candidate with the potential to significantly improve the treatment landscape for heart failure.

With promising Phase 2a results and a clear path forward, AnaCardio is entering its next stage of clinical development with strong momentum and growing confidence in AC01's transformative potential.

About the GOAL-HF1 HFrEF study

GOAL-HF1 is a Phase 1b/2a multicenter, randomized, double-blind, placebo-controlled study evaluating the safety, tolerability, pharmacokinetics, and pharmacodynamics of AC01 in patients with heart failure and reduced ejection fraction (HFrEF). All patients had NYHA class II-III heart failure with reduced ejection fraction, an ICD for primary prevention and optimized guideline-directed medical therapy.

- Phase 1b consisted of a multiple-dose escalation in 32 patients across four sequential dose cohorts (0.3–3 mg), each treated orally twice daily for 7 days.
- In Phase 2a, 26 patients were equally randomized into one of three parallel treatment arms and treated twice daily with 1 or 3 mg AC01, or placebo for 28 days.

More information about the study is available at www.clinicaltrials.gov (NCT05642507).

About AnaCardio

AnaCardio AB is a privately held clinical stage biopharmaceutical company developing novel drugs to treat heart failure. AnaCardio was founded based on ground-breaking research from Karolinska Institutet showing improved contractility of the heart muscle through a unique and differentiated mechanism. The Company's lead program AC01, a first-in-class calcium sensitizing inotrope, is an oral ghrelin mimetic small-molecule, which was in-licensed from Helsinn and is now being developed as a contractile agent in heart failure patients. AnaCardio holds the exclusive global rights from Helsinn to clinically develop AC01 for heart failure. The company has raised over USD 40 million to date. AnaCardio is based in Stockholm, Sweden.

Find more information about AnaCardio at www.anacardio.com and follow us on LinkedIn.

About Helsinn

Helsinn is a global pharmaceutical company that builds, manufactures, launches, and commercializes products to improve the quality of life for patients with cancer and chronic diseases, with a focus on supportive care, oncology and dermato-oncology. Headquartered in Lugano, Switzerland, Helsinn has direct commercial operations in the U.S., manufacturing operations in Ireland, offices in China and a network of trusted partners enabling a commercial presence in 90 countries.

Established in 1976, Helsinn is a third-generation family-owned company with broad pharmaceutical and technical expertise. For nearly half a century, Helsinn has been operating with integrity, passion and quality. The company continuously fosters innovation for its patients and embraces sustainable growth as a core element of its strategic vision.

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