



Cytokinetics Announces Receipt of Breakthrough Therapy Designation from FDA for Aficamten

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FDA Granted Designation for the Treatment of Symptomatic Obstructive Hypertrophic Cardiomyopathy Based on Results of REDWOOD-HCM

SOUTH SAN FRANCISCO, Dec. 09, 2021 (GLOBE NEWSWIRE) -- Cytokinetics, Incorporated (Nasdaq: CYTK) today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation for *aficamten* for the treatment of symptomatic obstructive hypertrophic cardiomyopathy (oHCM). *Aficamten* is a next-generation cardiac myosin inhibitor in development for the potential treatment of HCM.

Breakthrough Therapy Designation is granted by FDA to expedite the development and review of drugs that are intended to treat a serious condition when preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapies on a clinically significant endpoint. A drug that receives Breakthrough Therapy Designation is eligible for all Fast Track designation features, intensive guidance on an efficient drug development program, and organizational commitment involving senior managers.

"We are pleased that the FDA has granted Breakthrough Therapy Designation for *aficamten* for the treatment of symptomatic oHCM, a disease associated with myriad symptoms and functional limitations," said Fady I. Malik, M.D., Ph.D., Cytokinetics' Executive Vice President of Research & Development. "The results of REDWOOD-HCM met our high expectations supporting the potential role of *aficamten* as an innovative therapy to address the hypercontractility that underlies oHCM. With start-up activities underway in SEQUOIA-HCM, our Phase 3 clinical trial of *aficamten*, we look forward to its further development and engaging with FDA as may hopefully benefit patients whose lives are severely impacted by this disease."

About REDWOOD-HCM

The Breakthrough Therapy Designation for *aficamten* was based on results from Cohorts 1 and 2 of REDWOOD-HCM (Randomized Evaluation of Dosing With CK-274 in Obstructive Outflow Disease in HCM), the Phase 2 clinical trial of *aficamten* in patients with symptomatic oHCM, which were presented at the Heart Failure Society of America (HFSA) Annual Scientific Meeting in September 2021. The results showed that treatment with *aficamten* for 10 weeks resulted in statistically significant reductions from baseline compared to placebo in the average resting left ventricular outflow tract pressure gradient (LVOT-G) and the average post-Valsalva LVOT-G. A large majority of patients treated with *aficamten* achieved the target goal of treatment, defined as resting gradient <30 mmHg and post-Valsalva gradient <50 mmHg at Week 10, compared to placebo. Patients treated with *aficamten* also saw improvements in heart failure symptoms and reductions in NT-proBNP, a biomarker of cardiac wall stress. Treatment with *aficamten* in REDWOOD-HCM was generally well tolerated and the incidence of adverse events on *aficamten* was similar to that of placebo. No serious adverse events were attributed to *aficamten*, and no treatment interruptions occurred on *aficamten*.

About Aficamten

Aficamten is an investigational selective, small molecule cardiac myosin inhibitor discovered following an extensive chemical optimization program that was conducted with careful attention to therapeutic index and pharmacokinetic properties and as may translate into next-in-class potential in clinical development. *Aficamten* was designed to reduce the number of active actin-myosin cross bridges during each cardiac cycle and consequently suppress the myocardial hypercontractility that is associated with hypertrophic cardiomyopathy (HCM). In preclinical models, *aficamten* reduced myocardial contractility by binding directly to cardiac myosin at a distinct and selective allosteric binding site, thereby preventing myosin from entering a force producing state. The development program for *aficamten* is assessing its potential as a treatment that improves exercise capacity and relieves symptoms in patients with HCM as well as its long-term effects on cardiac structure and function. Cytokinetics is currently conducting start-up activities for SEQUOIA-HCM (Safety, Efficacy, and Quantitative Understanding of Obstruction Impact of *Aficamten* in HCM), the Phase 3 clinical trial of *aficamten* in patients with symptomatic oHCM.

About Hypertrophic Cardiomyopathy

Hypertrophic cardiomyopathy (HCM) is a disease in which the heart muscle (myocardium) becomes abnormally thick (hypertrophied). The thickening of cardiac muscle leads to the inside of the left ventricle becoming smaller and stiffer, and thus the ventricle becomes less able to relax and fill with blood. This ultimately limits the heart's pumping function, resulting in symptoms including chest pain, dizziness, shortness of breath, or fainting during physical activity. A subset of patients with HCM are at high risk of progressive disease which can lead to atrial fibrillation, stroke and death due to arrhythmias. There are no FDA approved medical treatments that directly address the hypercontractility that underlies HCM.

About Cytokinetics

Cytokinetics is a late-stage biopharmaceutical company focused on discovering, developing and commercializing first-in-class muscle activators and next-in-class muscle inhibitors as potential treatments for debilitating diseases in which muscle performance is compromised. As a leader in muscle biology and the mechanics of muscle performance, the company is developing small molecule drug candidates specifically engineered to impact muscle function and contractility. Cytokinetics has communicated its objective to submit a U.S. NDA for *omecamtiv mecarbil*, its novel cardiac muscle activator, following positive results from GALACTIC-HF, a large, international Phase 3 clinical trial in patients with heart failure. Cytokinetics is conducting METEORIC-HF, a second Phase 3 clinical trial of *omecamtiv mecarbil*. Cytokinetics is also developing *aficamten*, a next-generation cardiac myosin inhibitor, for the potential treatment of hypertrophic cardiomyopathies (HCM). The company has announced positive results from Cohorts 1 and 2 in REDWOOD-HCM, a Phase 2 clinical trial of *aficamten* in patients with obstructive HCM. Cytokinetics is conducting start-up activities for SEQUOIA-HCM, the Phase 3 clinical trial of *aficamten* in patients with obstructive HCM. Cytokinetics is also developing *reldesemtiv*, a fast skeletal muscle troponin activator, currently the subject of COURAGE-ALS, a Phase 3 clinical trial in patients with ALS. Cytokinetics continues its over 20-year history of pioneering innovation in muscle biology and related pharmacology focused to diseases of muscle dysfunction and conditions of muscle weakness.

For additional information about Cytokinetics, visit www.cytokinetics.com and follow us on [Twitter](#), [LinkedIn](#), [Facebook](#) and [YouTube](#).

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Forward-Looking Statements

This press release contains forward-looking statements for purposes of the Private Securities Litigation Reform Act of 1995 (the "Act"). Cytokinetics disclaims any intent or obligation to update these forward-looking statements and claims the protection of the Act's Safe Harbor for forward-looking statements. Examples of such statements include, but are not limited to: statements relating to the timing and design of Cytokinetics' Phase 3 clinical trial of *aficamten*; the potential benefits of *aficamten*; Cytokinetics' ability to fully enroll SEQUOIA-HCM; the likelihood and timeframes within which Cytokinetics may obtain regulatory approval from the FDA or any other regulatory agency for *aficamten*; Cytokinetics' research and development activities; and the properties and potential benefits of Cytokinetics' drug candidates. Such statements are based on management's current expectations, but actual results may differ materially due to various risks and uncertainties, including, but not limited to, potential difficulties or delays in the development, testing, regulatory approvals for trial commencement, progression or product sale or manufacturing, or production of Cytokinetics' drug candidates that could slow or prevent clinical development or product approval; patient enrollment for or conduct of clinical trials may be difficult or delayed; Cytokinetics' drug candidates may have adverse side effects or inadequate therapeutic efficacy; the FDA or foreign regulatory agencies may delay or limit Cytokinetics' ability to conduct clinical trials; Cytokinetics may be unable to obtain or maintain patent or trade secret protection for its intellectual property; standards of care may change, rendering Cytokinetics' drug candidates obsolete; competitive products or alternative therapies may be developed by others for the treatment of indications Cytokinetics' drug candidates and potential drug candidates may target; and risks and uncertainties relating to the timing and receipt of payments from its partners, including milestones and royalties on future potential product sales under Cytokinetics' collaboration agreements with such partners. For further information regarding these and other risks related to Cytokinetics' business, investors should consult Cytokinetics' filings with the Securities and Exchange Commission, particularly under the caption "Risk Factors" in Cytokinetics' latest Quarterly Report on Form 10-Q.

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