



Cytokinetics Announces Grant From Families with Spinal Muscular Atrophy for Preclinical Development of Tirasemtiv

April 4, 2013 11:30 AM EDT

Award to Support Testing of *Tirasemtiv* in Mouse Models of Spinal Muscular Atrophy

South San Francisco, CA, and Elk Grove Village, IL, April 4, 2013 - Cytokinetics, Incorporated (Nasdaq: CYTK) and Families of Spinal Muscular Atrophy (FSMA) announced the award of a grant from FSMA to Cytokinetics to support preclinical research on muscle function in a mouse model of spinal muscular atrophy (SMA) to be conducted with the company's fast skeletal muscle troponin activator, *tirasemtiv*. Financial details of the grant were not disclosed.

Tirasemtiv, the lead drug candidate from Cytokinetics' skeletal muscle contractility program, selectively activates the fast skeletal muscle troponin complex by increasing its sensitivity to calcium, thereby increasing skeletal muscle force in response to neuronal input and delaying the onset and reducing the degree of muscle fatigue. Cytokinetics is evaluating *tirasemtiv* as a potential treatment for amyotrophic lateral sclerosis (ALS) in BENEFIT-ALS (Blinded Evaluation of Neuromuscular Effects and Functional Improvement with *Tirasemtiv* in ALS), an international Phase IIb clinical trial that is now enrolling patients.

The objective of this new funded preclinical research is to examine whether *tirasemtiv* can improve muscle function in mouse models of SMA. Cytokinetics will examine the effects of *tirasemtiv* on leg and respiratory muscle function and the effects of *tirasemtiv* to reduce fatigue and improve muscle strength during exercise.

"There remains a significant unmet medical need for a novel therapy that can improve muscle function, including respiratory muscle function, in patients with SMA," stated Jill Jarecki, Ph.D., Research Director of Families of Spinal Muscular Atrophy. "If *tirasemtiv* can improve muscle function in mouse models of SMA, it may also ameliorate muscle weakness in patients with SMA and thereby has the potential to improve quality of life for patients affected by this disease."

"We are pleased to be the recipient of this grant funding from Families of Spinal Muscular Atrophy which will enable us to investigate the potential of *tirasemtiv* to address some of the unmet needs of this grievous disease," stated Jeffrey Jasper, Ph.D., Cytokinetics' Head of Pharmacology. "We look forward to working with Families of Spinal Muscular Atrophy on this important project which may inform Cytokinetics' plans for future clinical development activities of *tirasemtiv*."

Development Status of *Tirasemtiv*

Tirasemtiv (formerly CK-2017357) is currently being evaluated in BENEFIT-ALS, an international, double-blind, randomized, placebo-controlled, Phase IIb clinical trial designed to evaluate the safety, tolerability and potential efficacy of this novel drug candidate in patients with ALS. BENEFIT-ALS is designed to enroll approximately 400 patients who will first complete one week of treatment with open-label *tirasemtiv* at 125 mg twice daily. Following completion of the open-label period, patients will be randomized to receive 12 weeks of double-blind treatment with twice-daily oral ascending doses of *tirasemtiv* beginning at 125 mg twice daily and increasing weekly up to 250 mg twice daily or a dummy dose titration with placebo. Clinical assessments will take place monthly during the course of treatment; patients will also participate in follow-up evaluations one and four weeks after their final dose. The primary efficacy analysis of BENEFIT-ALS will compare the mean change from baseline in the ALS Functional Rating Scale in its revised form (ALSFERS-R) on *tirasemtiv* versus placebo. Secondary endpoints will include Maximum Voluntary Ventilation (MVV) and other measures of respiratory and skeletal muscle function. Patients taking *riluzole* at the time of enrollment and who are randomized to receive *tirasemtiv* will receive *riluzole* at a reduced dose of 50 mg daily. Cytokinetics plans to conduct BENEFIT-ALS in over 70 sites across the United States, Canada, and several European countries.

Data from prior Phase IIa clinical trials of *tirasemtiv* in patients with ALS were presented at the 2012 American Academy of Neurology Annual Meeting and the 2010 International Symposium on ALS and Motor Neurone Diseases.

About Families of Spinal Muscular Atrophy

Families of Spinal Muscular Atrophy is the world's leader focused on funding SMA research to develop a treatment and cure for the disease. The successful results and progress that the organization has delivered, from basic research to drug discovery to clinical trials, provide real hope for families and patients impacted by the disease. The charity has invested over \$55 million in research and has been involved in funding half of all the ongoing novel drug programs for SMA. Families of SMA is a nonprofit 501(c)3 organization, with 31 Chapters and 90,000 members and supporters throughout the United States. The organization's work has produced major discoveries, including identification of the underlying cause and a back-up gene for the disease, which provides a clearly defined target for disease altering therapies. The organization is also dedicated to supporting SMA families through networking, information and services and to improving care for all SMA patients.

About Cytokinetics

Cytokinetics is a clinical-stage biopharmaceutical company focused on the discovery and development of novel small molecule therapeutics that modulate muscle function for the potential treatment of serious diseases and medical conditions. Cytokinetics' lead drug candidate from its cardiac muscle contractility program, *omecamtiv mecarbil*, is in Phase II clinical development for the potential treatment of heart failure. Amgen Inc. holds an exclusive license worldwide (excluding Japan) to develop and commercialize *omecamtiv mecarbil* and related compounds, subject to Cytokinetics' specified development and commercialization participation rights. Cytokinetics is independently developing *tirasemtiv* and CK-2127107, both fast skeletal muscle activators, as potential treatments for diseases and medical conditions associated with aging, muscle wasting or neuromuscular dysfunction. *Tirasemtiv* is currently the subject of a Phase II clinical trials program and has been granted orphan drug designation and fast track status by the U.S. Food and Drug Administration and orphan medicinal product designation by the European Medicines Agency for the potential treatment of amyotrophic lateral sclerosis, a debilitating disease of neuromuscular impairment in which treatment with *tirasemtiv* produced potentially clinically relevant pharmacodynamic effects in Phase II trials. All of these drug candidates have arisen from Cytokinetics' muscle biology focused research

activities and are directed towards the cytoskeleton. The cytoskeleton is a complex biological infrastructure that plays a fundamental role within every human cell. Additional information about Cytokinetics can be obtained at www.cytokinetics.com.

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 Cytokinetics' and its partners' research and development activities, including the conduct of preclinical research, the conduct, design and results of clinical trials, the significance and utility of clinical trial results, and the properties and potential benefits of tirasemtiv and Cytokinetics' other drug candidates, including the potential utility of tirasemtiv as a treatment of SMA. 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, Cytokinetics anticipates that it will be required to conduct at least one confirmatory Phase III clinical trial of tirasemtiv in ALS patients which will require significant additional funding, and it may be unable to obtain such additional funding on acceptable terms, if at all; 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, including risks that current and past results of clinical trials or preclinical studies may not be indicative of future clinical trials results, 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 U.S. Food and Drug Administration or foreign regulatory agencies may delay or limit Cytokinetics' or its partners' ability to conduct clinical trials, and Cytokinetics may be unable to obtain or maintain patent or trade secret protection for its intellectual property; Amgen's decisions with respect to the design, initiation, conduct, timing and continuation of development activities for omecamtiv mecarbil; Cytokinetics may incur unanticipated research and development and other costs or be unable to obtain additional financing necessary to conduct development of its products; Cytokinetics may be unable to enter into future collaboration agreements for its drug candidates and programs on acceptable terms, if at all; 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.

Contact:

Joanna L. Goldstein
Manager, Corporate Communications & Marketing
(650) 624-3000

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