Cytokinetics Announces Presentation of Phase IIA Clinical Trial Data of Tirasemtiv in Patients with Myasthenia Gravis

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Positive Results from Evidence of Effect Trial Inform Translation of Novel Mechanism of Action

South San Francisco, CA, March 21, 2013 - Cytokinetics, Incorporated (Nasdaq: CYTK) announced the presentation yesterday of positive data from a completed Phase IIa "Evidence of Effect" clinical trial of *tirasemtiv* in patients with generalized myasthenia gravis (MG) during the Emerging Science Program at the 65th Annual Meeting of the American Academy of Neurology. *Tirasemtiv,* the lead drug candidate from the company's 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. *Tirasemtiv* is being evaluated as a potential treatment for amyotrophic lateral sclerosis (ALS) in BENEFIT-ALS (**B**linded **E**valuation of **N**euromuscular **E**ffects and **F**unctional **I**mprovement with ***T****irasemtiv* in **ALS**), an international Phase IIb clinical trial that is now enrolling patients.

Emerging Science Presentation at the 65th Annual Meeting of the American Academy of Neurology

A presentation titled "A Study to Evaluate Efficacy, Safety and Tolerability of Single Doses of *Tirasemtiv* in Patients with Myasthenia Gravis" was made by Donald B. Sanders, MD, FAAN, Professor, Division of Neurology and Founder of the Duke Myasthenia Gravis Clinic and Chair. The presentation summarized the results of a double-blind, randomized, three-period crossover, placebo-controlled, pharmacokinetic and pharmacodynamic study of *tirasemtiv* in patients with generalized MG. In this trial, also known as CY 4023, patients received single, oral, double-blind doses of placebo, 250 mg of *tirasemtiv*, and 500 mg of *tirasemtiv* in random order and approximately one week apart. The main objectives of this trial were to assess the effects of *tirasemtiv* on various measures of skeletal muscle strength and fatigue including measures of pulmonary function. Since CY 4023 was a hypothesis-generating trial, no single primary efficacy endpoint was pre-specified.

At six hours after dosing in CY 4023, improvements (i.e., decreases) in the Quantitative MG score (QMG) were related to the dose of *tirasemtiv*. Decreases in the QMG score were dose related (-0.49 QMG points per 250 mg; p = 0.02). At six hours after the 500 mg dose of *tirasemtiv*, the QMG score decreased by 0.99 points (p = 0.02). In a responder analysis, twice as many patients (n = 12) improved by 3 points or more six hours after dosing with 500 mg of *tirasemtiv* than six hours after placebo (p = 0.098). The QMG is a validated index of disease severity that is often employed as a primary endpoint in clinical trials of patients with MG. In addition, the percent predicted forced vital capacity (FVC) increased relative to placebo at six hours after both the 250 mg and 500 mg doses of *tirasemtiv* (7.0 ± 2.1%, p = 0.0012 and 4.5 ± 2.1%, p = 0.034, respectively). Increases in percent predicted FVC were also dose-related (2.2% per 250mg, p = 0.043).

The authors concluded that both the 250 mg and 500 mg single oral doses of *tirasemtiv* studied in this Phase IIa clinical trial were well-tolerated by the 32 patients enrolled. No premature terminations and no serious adverse events were reported in this trial. The most commonly reported adverse event in this trial was dizziness, which was mild in all but one case that was classified as moderate.

Cytokinetics has been awarded $3.5 million in grants (Award Number RC3NS070670) from the National Institute of Neurological Disorders and Stroke (NINDS) to fund research and development of *tirasemtiv* in MG. Through December 31, 2012, Cytokinetics has incurred $4.6 million in research and development expenses associated with its MG program, and has received $3.2 million or 70% of the program's funding from NINDS. The content of this press release is solely the responsibility of Cytokinetics and does not necessarily represent the official views of the NINDS or the National Institutes of Health.

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 (ALSFRS-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 this trial at 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 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*, a skeletal muscle activator, as a potential treatment for diseases and 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, 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. 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.

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