

Cytokinetics Announces Publication of Results from Phase II Trial of Tirasemtiv in Patients with Myasthenia Gravis

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Data from Evidence of Effect Trial Provide Support for Novel Mechanism of Action in Neuromuscular Diseases

South San Francisco, CA, March 25, 2015 - Cytokinetics, Incorporated (Nasdaq: CYTK) announced the publication of a manuscript relating to its fast skeletal muscle troponin activator *tirasemtiv* in the journal *Neurotherapeutics*. This publication summarizes results from a Phase IIa "Evidence of Effect" or hypothesis-generating clinical trial which evaluated *tirasemtiv* in patients with generalized myasthenia gravis (MG). *Tirasemtiv* is the lead drug candidate from Cytokinetics' skeletal muscle contractility program and is being developed as a potential treatment for amyotrophic lateral sclerosis (ALS).

"We are pleased to share additional clinical data relating to *tirasemtiv* in patients with generalized myasthenia gravis," stated Andrew A. Wolff, MD, FACC, Cytokinetics' Senior Vice President and Chief Medical Officer. "We believe that effects observed on the Quantitative Myasthenia Gravis score and on vital capacity following administration of a single dose of *tirasemtiv* support the evaluation of skeletal muscle activation in patients with neuromuscular disorders including ALS. We are preparing to initiate a Phase III clinical development program to evaluate the effects of *tirasemtiv* on measures of respiratory function and other measures of skeletal muscle performance in patients with ALS."

The publication, titled "A Double-Blinded, Randomized, Placebo-Controlled Trial to Evaluate Efficacy, Safety, and Tolerability of Single Doses of *Tirasemtiv* in Patients with Acetylcholine Receptor-Binding Antibody-Positive Myasthenia Gravis," appeared online in the March edition of the journal *Neurotherapeutics*. The primary objective of this early-stage clinical study was to evaluate the effects of single 250 mg and 500 mg doses of *tirasemtiv* versus placebo on measures of skeletal muscle function and fatigability in patients with generalized MG and persistent muscle weakness. The secondary objectives of the study were to evaluate and characterize the relationship, if any, between the doses and plasma concentrations of *tirasemtiv* and its pharmacodynamic effects, and to evaluate the safety and tolerability of *tirasemtiv* administered as single doses to patients with MG. The authors concluded that 6 hours after dosing, *tirasemtiv* produced dose-related improvements from baseline in the Quantitative MG (QMG) score (slope: -0.49 QMG point per 250 mg administered; $p=0.02$; lower scores indicate better function) and in percent predicted forced vital capacity (slope: 2.2 % increase per

250 mg administered; $p=0.04$). The QMG improved by >3 points in twice as many patients after 500 mg *tirasemtiv* than after placebo. Both doses of *tirasemtiv* were well tolerated; there were no premature terminations or serious adverse events. The results of this study suggest that *tirasemtiv* may improve muscle function in patients with MG and support further development of *tirasemtiv* in neuromuscular diseases.

About *Tirasemtiv*

Tirasemtiv, a novel skeletal muscle activator, is the lead drug candidate from Cytokinetics' skeletal muscle contractility program. *Tirasemtiv* selectively activates the fast skeletal muscle troponin complex by increasing its sensitivity to calcium and, in preclinical studies and early clinical trials, demonstrated increases in skeletal muscle force in response to neuronal input and delays in the onset and reductions in the degree of muscle fatigue. Cytokinetics is developing *tirasemtiv*, a fast skeletal muscle activator, as a potential treatment for ALS. Cytokinetics conducted BENEFIT-ALS, a Phase IIb, multi-national, double-blind, randomized, placebo-controlled, clinical trial designed to evaluate the safety, tolerability and efficacy of *tirasemtiv* in patients with ALS. BENEFIT-ALS enrolled 711 patients from 73 centers in 8 countries. Following review of results from BENEFIT-ALS and initial regulatory interactions in both the United States and Europe, the company is preparing to advance *tirasemtiv* to a Phase III clinical development program that is designed to potentially confirm and extend results from BENEFIT-ALS. Objectives of the Phase III program will include measures of respiratory function after longer duration treatment in patients with ALS, including effects on Slow Vital Capacity.

About Myasthenia Gravis

Myasthenia gravis is a progressive, chronic neuromuscular disease that commonly strikes people between the ages of 40 and 70 and afflicts between 50,000 and 85,000 people in the United States. Approximately 13,600 new cases of myasthenia gravis are diagnosed each year. Myasthenia gravis is an autoimmune disease in which the immune system attacks the junction between nerve and muscle, targeting either the muscle cell's acetylcholine receptor (which receives signals from the associated nerve cell) or the muscle-specific kinase, a protein that helps to organize acetylcholine receptors on the muscle cell. The cause of myasthenia gravis is unclear. Researchers suspect viruses or bacteria may trigger the autoimmune response; the thymus gland also may play a role in the disease. Symptoms include fatigue and weakness of voluntary muscles, including partial paralysis of eye movements, double vision, droopy eyelids, and weakness and fatigue in neck and jaw regions. This weakness fluctuates daily but tends to progress over the course of a few years, especially as may be untreated.

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 is developing *tirasemtiv*, a fast skeletal muscle activator, as a potential treatment for amyotrophic lateral sclerosis (ALS). *Tirasemtiv* 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 ALS. Cytokinetics is collaborating with Amgen Inc. to develop *omecamtiv mecarbil*, a cardiac muscle activator, for the potential treatment of heart failure. Cytokinetics is collaborating with Astellas Pharma Inc. to develop CK-2127107, a fast skeletal muscle activator, for the potential treatment of spinal muscular atrophy. Amgen holds an exclusive license worldwide to develop and commercialize *omecamtiv mecarbil* and Astellas holds an exclusive license worldwide to develop and commercialize CK-2127107. Both licenses are subject to Cytokinetics' specified development and commercialization participation rights. 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 <http://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 initiation, conduct, design, and results of clinical trials, the significance and utility of clinical trial results, the expected availability of clinical trial results, and the use of effects on respiratory function, including slow vital capacity, as a Phase III clinical trial endpoint for tirasemtiv; potential markets for tirasemtiv; 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 further clinical development of tirasemtiv in ALS patients which will require significant additional funding, and Cytokinetics may be unable to obtain such additional funding on acceptable terms, if at all; the FDA and/or other regulatory authorities may not accept effects on slow vital capacity as a clinical endpoint to support registration of tirasemtiv for the treatment of ALS; 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 and Astellas' decisions with respect to the design, initiation, conduct, timing and continuation of development activities for omecamtiv mecarbil and CK-2127107, respectively; 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.

Funding: Cytokinetics received \$3.4 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. Cytokinetics incurred \$4.7 million in research and development expenses associated with its MG program; 73% of the program's funding was from the 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.

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