Cytokinetics Announces Presentation Providing Update on BENEFIT-ALS at International Symposium on ALS/MND

December 8, 2013 9:30 AM ET

Interim, Blinded, Aggregate Data on Patient Enrollment, Baseline Demographics, Dose Escalation and Tolerability Presented; Final Results Expected in Second Quarter of 2014

South San Francisco, CA, December 8, 2013 - Cytokinetics, Incorporated (Nasdaq: CYTK) announced today that a platform presentation of data from BENEFIT-ALS (**B**linded **E**valuation of **N**euromuscular **E**ffects and **F**unctional **I**mprovement with ***T****irasemtiv* in **ALS**) was given at the 24th International Symposium on ALS/MND on December 7, 2013 in Milan, Italy. The clinical trial design was explained and interim, double-blind, aggregate data on patient enrollment, baseline demographics, dose escalation, and tolerability were presented. BENEFIT-ALS is evaluating *tirasemtiv*, a novel mechanism skeletal muscle activator, as a potential treatment for patients with amyotrophic lateral sclerosis (ALS).

Presentation at the 24th International Symposium of ALS/MND

A presentation titled, "The Effect of *Tirasemtiv* on Functional Status in Patients with ALS" was given by Jeremy M. Shefner, M.D., Ph.D., Professor and Chair, Department of Neurology at the Upstate Medical University, State University of New York and Lead Investigator for BENEFIT-ALS. Dr. Shefner provided an update on BENEFIT-ALS and shared interim, double-blind, aggregate data (i.e., data from the two treatment groups, *tirasemtiv* versus placebo, were not separated) from the ongoing clinical trial. He reported that BENEFIT-ALS recently completed enrollment with a total of 711 patients. He also concluded that the open-label, lead-in phase with *tirasemtiv* appears to maintain the study blind and to reduce dropouts following randomization. Dr. Shefner reported that, to date, the majority of patients randomized in BENEFIT-ALS are titrated to the target dose of *tirasemtiv* (250 mg twice daily) or matching placebo and complete the trial at that dose. He reported that dizziness, the most commonly reported adverse effect associated with *tirasemtiv* in prior Phase IIa clinical trials, has been the most frequently reported adverse event in BENEFIT-ALS to date and has generally resolved during continuous treatment with median duration of six to seven days.

Dr. Shefner described the study drug assignment error that had been reported in July, and explained the response to that error, which is intended to maintain the original statistical power of the trial and preserve the statistical integrity of the primary analysis of efficacy. He concluded that changes in the baseline demographics of patients observed over time during the conduct of BENEFIT-ALS support the decision to exclude from the primary efficacy analysis all of the 156 patients randomized concurrently (to *tirasemtiv* or placebo) in blocks that included any of the 58 patients subjected to the study drug assignment error. Dr. Shefner also shared interim, double-blind, aggregate data from novel functional assessments employed in BENEFIT-ALS and remarked on the potential of this clinical trial to provide useful information regarding the possible effects of *tirasemtiv* on parameters of skeletal muscle strength and fatigue, including specific measures of pulmonary function, that may be relevant to activities of daily living in patients with ALS. Dr. Shefner also reported that the recent completion of patient enrollment in BENEFIT-ALS may enable the results of this clinical trial to be reported during the second quarter of 2014.

"I am pleased to have an opportunity to provide an update on BENEFIT-ALS at the largest international gathering of physicians, patients and caregivers representing the ALS community," stated Dr. Shefner. "This clinical trial appears to be proceeding well in line with our objectives. I expect that it will provide important information regarding this novel drug candidate that offers potential to impact measures of skeletal muscle and overall patient function that we know are relevant to the natural history of this progressive disease."

About BENEFIT-ALS

BENEFIT-ALS is a Phase IIb, multinational, double-blind, randomized, placebo-controlled clinical trial designed to

evaluate the safety, tolerability and potential efficacy of *tirasemtiv*, a fast skeletal muscle troponin activator, in patients with ALS. Patients enrolled in BENEFIT-ALS began treatment with open-label dosing of *tirasemtiv* at 125 mg twice daily. Patients who tolerated this open-label treatment for one week were randomized to receive 12 weeks of double-blind treatment with twice-daily oral ascending doses of *tirasemtiv* or placebo, beginning at 125 mg twice daily and increasing weekly up to 250 mg twice daily (or a dummy dose titration with placebo). Clinical assessments occur monthly during double-blind treatment; patients also return for follow-up evaluations at one and four weeks after their final dose of double-blind study medication. 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 skeletal muscle function and fatigability.

Development Status of *Tirasemtiv*

Tirasemtiv (formerly CK-2017357) 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, which increases skeletal muscle force in response to neuronal input and delays the onset and reduces the degree of muscle fatigue. *Tirasemtiv* is the subject of a Phase II clinical trials development program and has been granted orphan drug designation and fast track status by the United States Food and Drug Administration and orphan medicinal product designation by the European Medicines Agency for the potential treatment of ALS. In three previously completed Phase IIa clinical trials, *tirasemtiv* appeared to be generally well-tolerated; in addition, encouraging trends toward improvement in patients' functional status and increases in respiratory function parameters and other measures of skeletal muscle strength and endurance were observed.

Background on Amyotrophic Lateral Sclerosis

Amyotrophic lateral sclerosis is a progressive neurodegenerative disease that afflicts approximately 25,000 people in the United States and a comparable number of patients in Europe. Approximately 5,600 new cases of ALS are diagnosed each year in the United States. The average life expectancy of an ALS patient is approximately three to five years after diagnosis and only 10% of patients survive for more than 10 years. Death is usually due to respiratory failure because of diminished strength in the skeletal muscles responsible for breathing. Few treatment options exist for these patients, resulting in a high unmet need for new therapeutic options to address the symptoms and modify the disease progression of this grievous illness.

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 to develop and commercialize *omecamtiv mecarbil* and related compounds, subject to Cytokinetics' specified development and commercialization participation rights. Cytokinetics is independently developing *tirasemtiv*, a fast skeletal muscle activator, as a potential treatment for diseases and medical conditions associated with 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 (ALS). Cytokinetics is collaborating with Astellas Pharma Inc. to develop CK-2127107, a skeletal muscle activator structurally distinct from *tirasemtiv*, for non-neuromuscular indications. 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 planned presentations; Cytokinetics' and its partners' research and development activities, including the conduct, design, progress and results of clinical trials; the expected timing for the availability of, and the significance and utility of, clinical trial results; the objectives of the protocol amendment for BENEFIT-ALS in response to the drug assignment error to maintain the originally intended statistical power of the trial and preserve the statistical integrity of the primary analysis of efficacy; the properties and potential benefits of tirasemtiv and Cytokinetics' other drug candidates, including the potential benefits of tirasemtiv in treating patients with ALS; and the potential market for tirasemtiv. 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 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.

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