



Cytokinetics Provides Update on BENEFIT-ALS

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South San Francisco, CA, July 8, 2013 - Cytokinetics, Incorporated (Nasdaq: [CYTK](#)) announced today an update on the conduct of BENEFIT-ALS (Blinded Evaluation of Neuromuscular Effects and Functional Improvement with *Tirasemtiv* in 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 amyotrophic lateral sclerosis (ALS).

BENEFIT-ALS is currently designed to enroll up to 500 patients with ALS. To date, over 450 patients have been enrolled in this study. The primary analysis of BENEFIT-ALS will compare the mean change from baseline in the ALS Functional Rating Scale in its revised form, or ALSFRS-R (a clinically validated instrument designed to measure disease progression and changes in functional status), in patients receiving *tirasemtiv* versus those receiving placebo.

Cytokinetics was recently informed by its data management vendor that a programming error in the electronic data capture system controlling study drug assignment caused 58 patients initially randomized to and treated with *tirasemtiv* to receive placebo instead at a certain study visit and for the remainder of the study. No patients randomized to placebo were dispensed incorrect treatment. Cytokinetics and all clinical trial site personnel remain blinded to the specific patients affected by the error.

Since the error was detected, the company has taken steps to ensure that no further incorrect study drug assignments have occurred and the programming error in the electronic data capture system controlling study drug assignment has been corrected. In addition, the company recently convened an *ad hoc* meeting of the study's Data Safety Monitoring Board (DSMB) to assess whether the error in dispensing study drug had impacted the safety of the 58 affected patients. After review of the relevant safety data from BENEFIT-ALS, the DSMB reported no concerns regarding patient safety.

Cytokinetics is in communication with regulatory authorities regarding how best to respond to the error in drug assignment in order to preserve the intended scientific value of BENEFIT-ALS. The company continues to enroll patients in the study under the current protocol and may amend the protocol to allow increased enrollment. Following further communications with regulatory authorities, Cytokinetics expects to provide updated guidance relating to the conduct of BENEFIT-ALS, which may include revisions to the timing of publicly available results from the study as well as to the projected costs of the study.

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, a debilitating disease of neuromuscular impairment in which treatment with *tirasemtiv* produced potentially clinically relevant pharmacodynamic effects in Phase II trials.

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.

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 Cytokinetics' discussions with regulatory authorities and the outcomes of those discussions; potential amendments to the protocol for BENEFIT-ALS, including potential changes in enrollment limits; the provision of further guidance with respect to the timing of publicly available results from BENEFIT-ALS and the projected costs of the study; the design, enrollment, conduct and results of BENEFIT-ALS; the effectiveness of steps taken to prevent further occurrences of incorrect study drug assignment; 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: if the BENEFIT-ALS protocol is amended to increase enrollment, it will result in increased time to enroll and complete the trial and increased costs to conduct the trial, and Cytokinetics may not be able to enroll patients in the trial above the current 500-patient limit until such protocol amendment is implemented; 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; 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' most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission.*

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