



## Cytokinetics Provides Additional Update and Revised Guidance For BENEFIT-ALS

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*Protocol Amended to Preserve the Intended Scientific Value of the Clinical Trial*

*Patient Enrollment Expected to Conclude in 2013 and Results Expected in Early 2014*

**South San Francisco, CA, July 23, 2013** -Cytokinetics, Incorporated (Nasdaq: CYTK) announced today an amendment to the protocol for 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). 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 recently announced that it had been 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. Following detection of the error, the company took steps to ensure that no further incorrect study drug assignments occurred and to correct the programming error in the electronic data capture system controlling study drug assignment. In addition, the company 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.

Following interactions with regulatory authorities, Cytokinetics amended the protocol for BENEFIT-ALS to enable increased enrollment to approximately 680 patients and to update the statistical methods section, in both cases with the objective to maintain the originally intended statistical power of the trial. To date, over 500 patients have been enrolled in BENEFIT-ALS. Enrollment is expected to continue as the new protocol amendment becomes effective at participating investigative centers. The company now expects to complete patient enrollment in BENEFIT-ALS during the second half of 2013, with results to be available in early 2014. These changes to BENEFIT-ALS are expected to increase the direct clinical trial costs by approximately \$5 million in 2013 and 2014.

### 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 earlier Phase IIa 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](http://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 the objectives of the protocol amendment for BENEFIT-ALS to maintain the originally intended statistical power and preserve the intended scientific value of the trial; the design, enrollment, conduct and results of BENEFIT-ALS; the expected timing for the completion of enrollment and availability of results from BENEFIT-ALS; the expected additional costs to conduct 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: Cytokinetics may not be able to enroll additional patients in BENEFIT-ALS until the 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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