



Cytokinetics Announces Top-Line Results From BENEFIT-ALS

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Company Summarizes Initial Data From Phase IIb Clinical Trial of Tirasemtiv

No Effect on Primary Efficacy Endpoint; Mixed Effects on Secondary Endpoints

SOUTH SAN FRANCISCO, CA, April 25, 2014 - Cytokinetics, Incorporated (Nasdaq: CYTK) announced today the top-line results of BENEFIT-ALS (Blinded Evaluation of Neuromuscular Effects and Functional Improvement with *Tirasemtiv* in ALS). Detailed results will be presented during the 66th Annual Meeting of the American Academy of Neurology (AAN) on Tuesday, April 29th at the Pennsylvania Convention Center in Philadelphia, PA.

BENEFIT-ALS did not achieve its primary efficacy endpoint, the mean change from baseline in the ALS Functional Rating Scale in its revised form (ALSFRS-R) on *tirasemtiv* versus placebo (-2.98 points in the *tirasemtiv* group versus -2.40 points in the placebo group, $p = 0.11$). Secondary efficacy analyses of the effect of *tirasemtiv* on respiratory function and other measures of skeletal muscle function produced mixed results.

"Patients with ALS desperately need new therapeutic alternatives to slow the course of their disease and loss of function. We stand with the ALS community in our disappointment that BENEFIT-ALS did not achieve its primary efficacy endpoint," stated Robert I. Blum, Cytokinetics' President and Chief Executive Officer. "The results from BENEFIT-ALS are just now becoming available to our team at Cytokinetics and will be shared in more detail with the broader scientific and medical community focused to research in ALS in the next few days. Understanding these results will require significant further review. Once we have fully evaluated the data from BENEFIT-ALS, we expect to determine whether there is a potential development path forward for *tirasemtiv* for the potential treatment of ALS and what may be the appropriate next steps."

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.

About BENEFIT-ALS

BENEFIT-ALS was a Phase IIb, multi-national, double-blind, randomized, placebo-controlled, clinical trial which was designed to evaluate the safety, tolerability and efficacy of *tirasemtiv* in patients with amyotrophic lateral sclerosis (ALS). BENEFIT-ALS enrolled 711 patients in 73 centers in 8 countries. Patients enrolled in BENEFIT-ALS began treatment with open-label *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 occurred monthly during double-blind treatment; patients also returned 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 compared the mean change from baseline in the ALS Functional Rating Scale in its revised form (ALSFRS-R) on *tirasemtiv* versus placebo. Secondary endpoints evaluated measures of respiratory performance and other measures of skeletal muscle function and fatigability.

About Amyotrophic Lateral Sclerosis (ALS)

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.

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' research and development activities, including the conduct, design, and results of clinical trials, the anticipated timing for the availability of clinical trial results and planned presentations of such results, evaluations of clinical trial results, and the significance and utility of clinical trial results; the further development of *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, the results of BENEFIT-ALS may not support further clinical development of *tirasemtiv*; further clinical development of *tirasemtiv* in ALS patients, if supported by the BENEFIT-ALS*

data, will require significant additional funding, and Cytokinetics 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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