# Cytokinetics Announces Results From BENEFIT-ALS Will Be Presented at the 66th Annual Meeting of the American Academy of Neurology

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***South San Francisco, CA, April 22, 2014-*** Cytokinetics, Incorporated (Nasdaq: CYTK) announced today that results from BENEFIT-ALS (**B**linded **E**valuation of **N**euromuscular **E**ffects and **F**unctional **I**mprovement with ***T***irasemtiv in **AL**S) will be presented during a Platform Session scheduled at the 66th Annual Meeting of the American Academy of Neurology (AAN) to be held April 26 - May 3, 2014 at the Pennsylvania Convention Center in Philadelphia, PA. Cytokinetics also announced that additional poster presentations related to *tirasemtiv* and ALS will be presented at AAN.

**Platform Session Presentation at the 66th Annual Meeting of the American Academy of Neurology**

Title: The Effect of *Tirasemtiv* on Functional Status in Patients with ALS
Presenter: Jeremy M. Shefner, M.D., Ph.D
Date: Tuesday, April 29, 2014
Presentation Time: 4:15 PM (Eastern Time)
Session: S16.005 - 5LB.001 - Platform Session: Neuromuscular and Clinical Neurophysiology

**About *Tirasemtiv* and BENEFIT-ALS**

*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, demonstrated increases in skeletal muscle force in response to neuronal input and delays in the onset and reductions in the degree of muscle fatigue. In previously conducted Phase IIa clinical trials in patients with amyotrophic lateral sclerosis (ALS), *tirasemtiv* appeared generally well-tolerated, and demonstrated encouraging trends to improvement in patients' functional abilities and increases in measures of respiratory and skeletal muscle strength and endurance.

BENEFIT-ALS is an international, double-blind, randomized, placebo-controlled, Phase IIb clinical trial which was designed to evaluate the safety, tolerability and potential efficacy of *tirasemtiv* 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 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 compares the mean change from baseline in the ALS Functional Rating Scale in its revised form (ALSFRS-R) on *tirasemtiv* versus placebo. Secondary endpoints include Maximum Voluntary Ventilation (MVV) and other measures of respiratory and skeletal muscle function and fatigability.

**Additional Poster Presentations at the 66th Annual Meeting of the American Academy of Neurology**

Title: Fast Skeletal Muscle Troponin Activator *Tirasemtiv* Increases Muscle Function and Performance in the B6SJL SOD1G93A ALS Mouse Model
Presenter: Fady I. Malik, M.D., Ph.D., F.A.C.C.
Date: Monday, April 28, 2014
Presentation Time: 3:00 PM (Eastern Time)
Session: P1.081 - Poster Session I: Anterior Horn Cell Disease: Pathogenesis and Pathology
Poster on Display: 3:00 PM - 6:30 PM (Eastern Time)

Title: *Tirasemtiv* Amplifies Skeletal Muscle Response to Nerve Activation in Humans
Presenter: Fady I. Malik, M.D., Ph.D., F.A.C.C.
Date: Wednesday, April 30, 2014
Presentation Time: 7:30 AM (Eastern Time)
Session: P4.077 - Poster Session IV: ALS: Trials and Biomarkers
Poster on Display: 7:30 AM - 11:00 AM (Eastern Time)

Title: Profile of Medical Care Costs in Patients with Amyotrophic Lateral Sclerosis in Medicare Program and Under Commercial Insurance
Presenter: Lisa Meng, Ph.D.
Date: Thursday, May 1, 2014
Presentation Time: 3:00 PM (Eastern Time)
Session: P7.102 - Poster Session VII: Neuromuscular Health Services/Outcomes Research
Poster on Display: 3:00 PM - 6:30 PM (Eastern Time)

**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, 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, Cytokinetics anticipates that it will be required to conduct at least one confirmatory Phase III clinical trial of tirasemtiv in ALS patients if supported by the BENEFIT-ALS data, 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' filings with the Securities and Exchange Commission.*

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