

Cytokinetics Announces Results from First-Time-In-Humans Clinical Trial of CK-2127107 at Conference of the Society on Sarcopenia, Cachexia and Wasting Disorders

December 10, 2013 7:30 AM ET

CK-2127107 Appears Well-Tolerated with Linear Dose-Proportional Pharmacokinetics in Phase I Clinical Trial

South San Francisco, CA, December 10, 2013 - Cytokinetics, Incorporated (Nasdaq: CYTK) announced today that the results from a first-time-in-humans clinical trial of CK-2127107 in healthy male volunteers were presented during a Late Breaking Clinical Trials session at the 7th International Conference of the Society on Sarcopenia, Cachexia and Wasting Disorders in Kobe, Japan. Cytokinetics, in collaboration with Astellas Pharma Inc. ("Astellas," Tokyo: 4503), is developing CK-2127107, a novel small molecule activator of the fast skeletal muscle troponin complex, for potential application in non-neuromuscular diseases and medical conditions associated with muscle weakness.

Presentations at the 7th International Conference of the Society of Sarcopenia, Cachexia and Wasting Disorders

During a session titled, "Late Breaking Clinical Trials and Biomarker Research," Fady I. Malik, MD, PhD, FACC presented results from the first-time-in-human clinical trial of CK-2127107, known as CY 5011, included in his presentation titled: "Fast Skeletal Muscle Troponin Activators and their Application to Disease - Clinical Update."

This Phase I clinical trial was a double-blind, randomized, placebo-controlled study designed to assess the safety, tolerability, and pharmacokinetics of single ascending oral doses of CK-2127107 administered to healthy adult males in a three period, escalating dose, crossover design. The primary objective of this trial was to evaluate the safety and tolerability of single doses of CK-2127107 administered orally to healthy male volunteers. The secondary objective was to evaluate the pharmacokinetic profile of single doses of CK-2127107. Planned single doses of CK-2127107 up to 4000 mg, the highest dose administered in this trial, were well-tolerated without an emerging pattern of adverse events; therefore, a maximum tolerated dose could not be defined. The pharmacokinetic profile of CK-2127107 was linear and dose-proportional across the dose range studied, with a mean terminal half-life compatible with once or twice daily dosing.

"We are pleased with the results from this first-time-in-humans clinical trial of CK-2127107, which suggest an encouraging safety and pharmacokinetic profile for this novel activator of skeletal muscle," stated Fady Malik, MD, PhD, FACC, Cytokinetics' Senior Vice President of Research and Early Development. "These results will inform our plans for the further clinical development of this drug candidate."

During an earlier session titled, "Drug Treatment Options and Results Update," Dr. Malik also presented the pre-clinical rationale and therapeutic hypotheses that supported the progression of two fast skeletal muscle activators, CK-2127107 and *tirasemtiv*, into human clinical trials. In this presentation, Dr. Malik highlighted pre-clinical research that demonstrated that fast skeletal muscle troponin activators amplify the response to motor neuron input, increase muscle power and improve muscle fatigability. In addition, the presentation highlighted the results of studies related to fast skeletal muscle troponin activators in preclinical disease models of amyotrophic lateral sclerosis and heart failure.

Cytokinetics and Astellas Collaboration

Under the collaboration, Cytokinetics has exclusively licensed to Astellas the rights to co-develop and commercialize CK-2127107, a fast skeletal troponin activator, for potential application in non-neuromuscular indications. Cytokinetics will be primarily responsible for the conduct of Phase I clinical trials and certain Phase II readiness activities for CK-2127107 and Astellas will be primarily responsible for the conduct of subsequent development and commercialization activities for CK-2127107. Cytokinetics and Astellas are jointly conducting research in the area of skeletal muscle activation. Astellas will have exclusive rights to develop and commercialize other fast skeletal troponin activators in non-neuromuscular indications and to develop and commercialize other novel mechanism skeletal muscle activators in all indications, subject to certain Cytokinetics' development and commercialization rights.

Background on Skeletal Muscle Activators

Skeletal muscle contractility is driven by the sarcomere, the fundamental unit of skeletal muscle contraction. It is a highly ordered cytoskeletal structure composed of several key proteins. The first, skeletal muscle myosin, is the cytoskeletal motor protein that converts chemical energy into mechanical force through its interaction with a second protein, actin. A set of regulatory proteins, which includes tropomyosin and several types of troponin, make the actin-myosin interaction dependent on changes in intracellular calcium levels. In non-clinical models, CK-2127107 slows the rate of calcium release from the regulatory troponin complex of fast skeletal muscle fibers, which sensitizes the sarcomere to calcium, leading to an increase in skeletal muscle contractility. CK-2127107 and other skeletal sarcomere activators have demonstrated pharmacological activity that may lead to new therapeutic options for diseases associated with aging and muscle wasting.

The clinical effects of muscle wasting, fatigue and loss of mobility can range from decreased quality of life to life-threatening complications. By directly improving skeletal muscle function, a small molecule activator of the skeletal sarcomere may potentially enhance physical performance and quality of life in patients with conditions marked by muscle weakness and fatigue.

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 Cytokinetics' and its partners' research and development activities, including the conduct, design and results of clinical trials, the significance and utility of preclinical data and clinical trial results, and the properties and potential benefits of Cytokinetics' skeletal muscle activators, including CK-2127107, and other drug candidates; and the expected roles of Cytokinetics and Astellas under their collaboration. 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; Astellas' and Amgen's decisions with respect to the design, initiation,*

conduct, timing and continuation of development activities for CK-2127107 and omecamtiv mecarbil, 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.

Contact:

*Joanna L. Goldstein
Manager, Corporate Communications & Marketing
(650) 624-3000*

HUG#1748780