



## Cytokinetics Announces Presentation of Data From Phase 2 Clinical Study of Reldesemtiv in Patients With Spinal Muscular Atrophy at the 2018 Annual Cure SMA Conference

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SOUTH SAN FRANCISCO, Calif., April 24, 2018 (GLOBE NEWSWIRE) -- Cytokinetics, Incorporated (Nasdaq:CYTK) today announced that data from the Phase 2 clinical study of *rel-desemtiv* in patients with spinal muscular atrophy (SMA) will be presented by John Day, M.D., Ph.D., Professor of Neurology and Pediatrics (Genetics), Stanford University Medical Center, in an oral presentation at the 2018 Annual Cure SMA Conference in Dallas on June 16, 2018.

The clinical study is designed to assess the effect of *rel-desemtiv*, a next-generation fast skeletal muscle troponin activator (FSTA), on multiple measures of muscle function in both ambulatory and non-ambulatory patients with SMA. In collaboration with Astellas Pharma Inc. (TSE:4503) ("Astellas"), Cytokinetics is developing *rel-desemtiv* as a potential treatment for people living with SMA and certain other debilitating diseases and conditions associated with skeletal muscle weakness and/or fatigue.

"We are evaluating multiple pharmacodynamic measures in this hypothesis-generating study intended to inform next steps in the development of *rel-desemtiv*," said Fady Malik, M.D., Ph.D., Cytokinetics' Executive Vice President of Research and Development. "We look forward to these data in adolescents and adults with types II, III and IV SMA, a population in need of new therapies to address impaired muscle function and weakness despite the promise of gene-directed therapies."

### Clinical Study Design

The primary objective of this Phase 2, double-blind, randomized, placebo-controlled clinical study is to determine the potential pharmacodynamic effects of a suspension formulation of *rel-desemtiv* following multiple oral doses in patients with Type II, Type III, or Type IV SMA. Secondary objectives are to evaluate the safety, tolerability and pharmacokinetics of *rel-desemtiv*. There is no single primary endpoint in this hypothesis-generating study.

The study enrolled 70 patients, 39 in Cohort 1 and 31 in Cohort 2. Ambulatory (Type III or Type IV) and non-ambulatory (Type II or Type III) patients 12 years of age and older were randomized 2:1 to receive *rel-desemtiv* or placebo dosed twice daily for eight weeks, stratified by ambulatory versus non-ambulatory status. The first cohort of patients received 150 mg of *rel-desemtiv* or placebo and the second cohort of patients received 450 mg of *rel-desemtiv* or placebo. Enrollment in this study was stopped short of the targeted 72 patients after blinded analyses of variability for the change from baseline of several of efficacy measures demonstrated that the study has sufficient statistical power to detect clinically relevant differences versus placebo in efficacy endpoints.

Multiple assessments of skeletal muscle function and fatigability are being performed in the study, including respiratory assessments, upper limb strength and functionality for non-ambulatory patients, as well as six-minute walk and timed-up-and-go for ambulatory patients. Patients enrolled in the second cohort will also be assessed with the SMA Health Index, a patient reported outcome measure. Additional information regarding the study can be found at [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

### About Reldesemtiv

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. Skeletal muscle myosin is the motor protein that converts chemical energy into mechanical force through its interaction with 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. *Reldesemtiv*, a next-generation fast skeletal muscle troponin activator (FSTA) arising from Cytokinetics' skeletal muscle contractility program, 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. *Reldesemtiv* has demonstrated pharmacological activity that may lead to new therapeutic options for diseases associated with muscle weakness and fatigue. In non-clinical models of SMA, a skeletal muscle activator has demonstrated increases in submaximal skeletal muscle force and power in response to neuronal input and delays in the onset and reductions in the degree of muscle fatigue. *Reldesemtiv* has been the subject of five completed Phase 1 clinical trials in healthy volunteers, which evaluated the safety, tolerability, bioavailability, pharmacokinetics and pharmacodynamics of the drug candidate. In addition to the Phase 2 clinical trial in patients with SMA, Cytokinetics is collaborating with Astellas on the conduct of a Phase 2 clinical trial in patients with amyotrophic lateral sclerosis (ALS) and chronic obstructive pulmonary disease (COPD) as well as a Phase 1b clinical trial of *rel-desemtiv* in elderly adults with limited mobility.

### About SMA

SMA is a severe, genetic neuromuscular disease that leads to debilitating muscle function and progressive, often fatal, muscle weakness. It occurs in 1 in 6,000 to 10,000 live births each year and is one of the most common potentially fatal genetic disorders. Spinal muscular atrophy manifests in various degrees of severity as progressive muscle weakness resulting in respiratory and mobility impairment. There are four types of SMA, named for age of initial onset of muscle weakness and related symptoms: Type I (Infantile), Type II (Intermediate), Type III (Juvenile) and Type IV (Adult onset). Life expectancy and disease severity vary by type of SMA. Type I patients have the worst prognosis, with a life expectancy of no more than 2 years; Type IV patients may have a normal life span but eventually suffer gradual weakness in the proximal muscles of the extremities, eventually resulting in mobility issues. Few treatment options exist for these patients, resulting in a high unmet need for new therapeutic options to address symptoms and modify disease progression.

### About Cytokinetics and Astellas Collaboration

In 2013, Astellas and Cytokinetics formed a partnership focused on the research, development, and commercialization of skeletal muscle activators. The primary objective of the collaboration is to advance novel therapies for diseases and medical conditions associated with muscle impairment and weakness. Under the collaboration, Cytokinetics exclusively licensed to Astellas rights to co-develop and potentially co-commercialize *rel-desemtiv*, a fast skeletal muscle troponin activator (FSTA), in non-neuromuscular indications. In 2014, Astellas and Cytokinetics agreed to expand the collaboration to include certain neuromuscular indications, including SMA, and to advance *rel-desemtiv* into Phase 2 clinical development, initially in SMA. Under the agreement as further amended in 2016, Astellas has exclusive rights to co-develop and commercialize *rel-desemtiv* and other FSTAs in non-neuromuscular indications and certain neuromuscular indications (including SMA and ALS) and other novel mechanism skeletal muscle

activators in all indications, subject to certain Cytokinetics' development and commercialization rights; Cytokinetics may co-promote and conduct certain commercial activities in North America and Europe under agreed scenarios.

## About Cytokinetics

Cytokinetics is a late-stage biopharmaceutical company focused on discovering, developing and commercializing first-in-class muscle activators as potential treatments for debilitating diseases in which muscle performance is compromised and/or declining. As a leader in muscle biology and the mechanics of muscle performance, the company is developing small molecule drug candidates specifically engineered to increase muscle function and contractility. Cytokinetics is collaborating with Amgen Inc. ("Amgen") to develop *omecamtiv mecarbil*, a novel cardiac muscle activator. *Omecamtiv mecarbil* is the subject of GALACTIC-HF, an international Phase 3 clinical trial in patients with heart failure. Amgen holds an exclusive worldwide license to develop and commercialize *omecamtiv mecarbil* with a sublicense held by Servier for commercialization in Europe and certain other countries. Cytokinetics is collaborating with Astellas Pharma Inc. ("Astellas") to develop *reldesemtiv*, a next-generation FSTA. *Reldesemtiv* has been granted orphan drug designation by the FDA for the potential treatment of spinal muscular atrophy. *Reldesemtiv* is the subject of three ongoing Phase 2 clinical trials enrolling patients with spinal muscular atrophy, chronic obstructive pulmonary disease and amyotrophic lateral sclerosis. Astellas is also conducting a Phase 1b clinical trial of *reldesemtiv* in elderly adults with limited mobility. Astellas holds an exclusive worldwide license to develop and commercialize *reldesemtiv*. Licenses held by Amgen and Astellas are subject to Cytokinetics' specified co-development and co-commercialization rights. Cytokinetics continues its 20-year history of innovation with three new muscle biology directed compounds advancing from research to development in 2018. For additional information about Cytokinetics, visit [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 Cytokinetics' and its partners' research and development activities, including the Phase 2 clinical study of *reldesemtiv* in patients with SMA; the design, results, significance and utility of preclinical study results; 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, 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; Astellas' decisions with respect to the design, initiation, conduct, timing and continuation of development activities for *reldesemtiv*; Cytokinetics may incur unanticipated research and development and other costs or be unable to obtain additional financing necessary to conduct development of its products; 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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