



## Cytokinetics Joins Global Movement to Raise Awareness for Rare Diseases

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**SOUTH SAN FRANCISCO, CA, February 27, 2015** - Cytokinetics, Incorporated (Nasdaq: CYTK) announced today that it is joining forces on February 28 with patients and health care advocates in the United States and globally to raise awareness for Rare Disease Day®. Rare Disease Day is dedicated to elevating public understanding of rare diseases and calling attention to the special challenges faced by patients with rare diseases and the support community around them.

"Cytokinetics is proud to stand alongside patients and caregivers representing the rare disease community," stated Robert I. Blum, Cytokinetics' President & Chief Executive Officer. "We applaud the tireless work of NORD and EURORDIS, two outstanding organizations that perform extraordinary work every day in support of education, advocacy, research and the care of patients suffering from rare diseases. Cytokinetics is dedicated to the discovery and development of novel therapeutics that modulate muscle function for the treatment of severe illnesses and conditions, including rare neuromuscular diseases."

Rare Disease Day, which takes place every year on the last day of February, was established in Europe in 2008 by the European Organisation for Rare Diseases (EURORDIS), the organization representing rare disease patients in Europe, and is now observed in more than 80 nations. In the United States, Rare Disease Day is sponsored by the National Organization for Rare Disorders (NORD), a leading independent, non-profit organization committed to the identification, treatment, and cure of rare diseases. According to EURORDIS, a disease or disorder is defined as rare in Europe when it affects less than 1 in 2000. Rare diseases may affect 30 million European Union citizens. According to the National Institutes of Health (NIH), a disease is rare if it affects fewer than 200,000 people. Nearly 1 in 10 Americans live with a rare disease-affecting 30 million people - and two-thirds of these patients are children. There are more than 7,000 rare diseases and only approximately 450 FDA-approved medical treatments. For more information about Rare Disease Day in the U.S., go to [www.rarediseaseday.us](http://www.rarediseaseday.us). For information about global activities, go to [www.rarediseaseday.org](http://www.rarediseaseday.org). To search for information about rare diseases, visit NORD's website, [www.rarediseases.org](http://www.rarediseases.org).

Cytokinetics is developing two drug candidates for the potential treatment of rare diseases. The company is developing *tirasemtiv*, a fast skeletal troponin muscle activator, as a potential treatment for patients with amyotrophic lateral sclerosis (ALS). In addition, the company is collaborating with Astellas Pharma Inc. to develop CK-2127107, a structurally distinct fast skeletal troponin muscle activator, as a potential treatment for patients with spinal muscular atrophy (SMA).

### About Amyotrophic Lateral Sclerosis

Amyotrophic lateral sclerosis (ALS) 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 patients with ALS, resulting in a high unmet need for new therapeutic options to address the symptoms and modify disease progression.

### About Spinal Muscular Atrophy

Spinal Muscular Atrophy (SMA) is a severe neuromuscular disease that occurs in 1 in every 6000 to 10,000 live births each year and is one of the most common 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 based on time 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 varies from Type I patients, who have the worst prognosis and a life expectancy of generally less than 2 years from birth to Type IV patients, who have a normal life span but with gradual weakness in proximal muscles of the extremities 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

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 is developing *tirasemtiv*, a fast skeletal muscle activator, as a potential treatment for amyotrophic lateral sclerosis (ALS). *Tirasemtiv* 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 ALS. Cytokinetics is collaborating with Amgen Inc. to develop *omecamtiv mecarbil*, a cardiac muscle activator, for the potential treatment of heart failure. Cytokinetics is collaborating with Astellas Pharma Inc. to develop CK-2127107, a skeletal muscle activator, for the potential treatment of spinal muscular atrophy. Amgen holds an exclusive license worldwide to develop and commercialize *omecamtiv mecarbil* and Astellas holds an exclusive license worldwide to develop and commercialize CK-2127107. Both licenses are subject to Cytokinetics' specified development and commercialization participation rights. 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 <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' research and development activities; the potential size of markets for *tirasemtiv* and CK-2127107; and the properties and potential benefits of *tirasemtiv*, CK-2127107 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: further clinical development of *tirasemtiv* in ALS patients 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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