



## Cytokinetics Joins Global Movement to Recognize International Rare Disease Day

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SOUTH SAN FRANCISCO, Calif., Feb. 29, 2016 (GLOBE NEWSWIRE) – Cytokinetics, Inc. (Nasdaq:CYTK) today announced that it is joining forces with the European Organisation for Rare Diseases (EURORDIS) and the National Organization for Rare Disorders (NORD) to raise awareness of Rare Disease Day®, an international campaign dedicated to elevating the public understanding of rare diseases. This year's theme, "Patient Voice," calls attention to the crucial role that patients, caregivers and health care advocates play in emphasizing the urgent needs of rare diseases to legislators and regulators to effect change and ensure equitable access to life-changing therapies, care and support.

"Patients with rare diseases, as well as their families and caregivers, face unique challenges, and deserve our support today and every day," said Robert I. Blum, Cytokinetics' President and Chief Executive Officer. "We stand in solidarity with these patients, who inspire and motivate us as we collaborate with the ALS and SMA research and clinical communities to advance the development of new medicines."

Cytokinetics is developing two drug candidates for the potential treatment of rare diseases. *Tirasemtiv*, a novel skeletal muscle troponin activator, is being evaluated in a Phase 3 clinical trial, VITALITY-ALS, as a potential treatment for people living with amyotrophic lateral sclerosis (ALS), and CK-2127107, a next-generation fast skeletal muscle activator, is being evaluated in a Phase 2 clinical trial as a potential treatment for people living with spinal muscular atrophy (SMA), in collaboration with Astellas.

### About Rare Disease Day

Rare Disease Day, which takes place every year on the last day in February, was established in Europe in 2008 by 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 the National Institutes of Health (NIH), a rare disease is defined as one that affects fewer than 200,000 people. With nearly 7,000 rare diseases, more than 25 million Americans are living with a rare disease, but less than 5 percent of these diseases have a treatment.

### About ALS

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 these patients, resulting in a high unmet need for new therapeutic options to address the symptoms and to modify the disease progression of this grievous illness.

### About SMA

SMA is a severe neuromuscular disease that occurs in 1 in every 6,000 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, 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 have a normal life span but eventually suffer gradual weakness in the 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 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' lead drug candidate is *tirasemtiv*, a fast skeletal muscle activator, for the potential treatment of 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 retains the right to develop and commercialize *tirasemtiv*. Cytokinetics is collaborating with Amgen Inc. to develop *omecamtiv mecarbil*, a novel cardiac muscle activator, for the potential treatment of heart failure. Cytokinetics is collaborating with Astellas Pharma Inc. to develop CK-2127107, a fast 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. 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 expected revenue and R&D and G&A expenses, the initiation, conduct, design, enrollment, progress, continuation, completion and results of clinical trials, the significance and utility of preclinical study and clinical trial results, the expected availability of clinical trial results, planned interactions with regulatory authorities and the outcomes of such interactions; enrollment in VITALITY-ALS; enrollment and progress of the Phase 2 clinical trial of CK-2127107 in patients with SMA; the significance and utility of preclinical study and clinical trial results; and the properties and potential efficacy and safety profile of CK-2127107; the expected timing of events; 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 further clinical development of *tirasemtiv* in ALS patients which will require significant additional funding, and Cytokinetics may be unable to obtain such additional funding on acceptable terms, if at all; the FDA and/or other regulatory authorities may not accept effects on slow vital capacity as a clinical endpoint to support registration of *tirasemtiv* for the treatment of ALS; 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 trial 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. Forward-looking statements are not guarantees of future performance, and Cytokinetics' actual results of operations, financial condition and liquidity, and the development of the industry in which it operates, may differ materially from the forward-looking statements contained in this press release. Any forward-looking statements that Cytokinetics makes in this press release speak only as of the date of this press release. Cytokinetics assumes no obligation to update its forward-looking statements whether as a result of new information, future events or otherwise, after the date of this press release.*

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