



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

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SOUTH SAN FRANCISCO, Calif., Feb. 28, 2017 (GLOBE NEWSWIRE) -- Cytokinetics, Inc. (Nasdaq:CYTK) today announced that it is joining the global initiative 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, Research, calls attention to the importance of scientific research to better understand rare diseases and to develop innovative treatments to better patients' lives.

"We are proud to stand together with EURORDIS and NORD and to honor people living with rare diseases. We are especially inspired by this year's theme highlighting the promise of innovative research," said Robert I. Blum, Cytokinetics' President and Chief Executive Officer. "This is truly an exciting year for us as we expect results from late-stage clinical trials of our novel mechanism drug candidates in patients with ALS and SMA, both rare diseases urgently in need of new therapies."

Cytokinetics is developing drug candidates for the potential treatment of rare diseases. *Tirasemtiv*, a fast skeletal troponin activator (FSTA), is being evaluated in a Phase 3 clinical trial, VITALITY-ALS, as a potential treatment for amyotrophic lateral sclerosis (ALS), and CK-2127107, a next-generation FSTA, is being evaluated in a Phase 2 clinical trial as a potential treatment for spinal muscular atrophy (SMA), in collaboration with Astellas. Data from VITALITY-ALS are expected in Q4 2017 and data from the Phase 2 trial of CK-2127107 are expected in the second half of 2017.

### 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 European Organisation for Rare Diseases (EURORDIS), 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), in the US, a rare disease is defined as one that affects fewer than 200,000 people. With nearly 7,000 rare diseases, 25 million Americans are living with a rare disease, but only 5 percent of these diseases have a treatment.

### About ALS

ALS is a progressive neurodegenerative disease that afflicts approximately 30,000 people in the United States and a comparable number of patients in Europe. Approximately 6,000 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 approximately 10 percent 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 therapies to address functional deficits and disease progression.

### 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. SMA 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 troponin activator (FSTA). *Tirasemtiv* is the subject of VITALITY-ALS, an international Phase 3 clinical trial in patients with 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. Cytokinetics is preparing for the potential commercialization of *tirasemtiv* in North America and Europe and has granted an option to Astellas Pharma Inc. for development and commercialization in other countries. Cytokinetics is collaborating with Astellas to develop CK-2127107, a next-generation fast skeletal muscle activator. CK-2127107 is the subject of two ongoing Phase 2 clinical trials enrolling patients with spinal muscular atrophy and chronic obstructive pulmonary disease. Cytokinetics is collaborating with Amgen Inc. 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. Astellas holds an exclusive worldwide license to develop and commercialize CK-2127107. Licenses held by Amgen and Astellas are subject to Cytokinetics' specified co-development and co-commercialization rights. For additional information about Cytokinetics, visit <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 conduct, design, enrollment, progress and timing of results of the VITALITY-ALS Phase 3 clinical trial of *tirasemtiv* in patients with ALS and 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 *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; 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 FDA 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; standards of care may change, rendering Cytokinetics' drug candidates obsolete; and 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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