



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

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SOUTH SAN FRANCISCO, Calif., Feb. 28, 2018 (GLOBE NEWSWIRE) -- Cytokinetics, Incorporated (Nasdaq:CYTK) today announced that it is joining the global initiative to raise awareness of Rare Disease Day®, an international campaign led by the European Organisation for Rare Diseases (EURORDIS) and the National Organization for Rare Disorders (NORD), dedicated to elevating the public understanding of rare diseases. Much like the similar global initiative in 2017, this year's activities will focus attention on the importance of research and will highlight the roles both researchers and patients play in advancing research towards potential treatments for rare diseases.

"Today we stand with EURORDIS and NORD to recognize Rare Disease Day and shine a light on the innovations that power research focused to novel therapies for people living with rare diseases," said Robert I. Blum, Cytokinetics' President and Chief Executive Officer. "In addition to ongoing Phase 2 clinical trials of *reldesemtiv*, our next-generation fast skeletal muscle activator directed to the potential treatment of ALS and SMA, we are also proud to be advancing three new potential drug candidates from our research that may address needs of patients with rare diseases."

Cytokinetics is collaborating with Astellas to develop *reldesemtiv*, a next-generation fast skeletal troponin muscle activator (FSTA), which is the subject of four ongoing clinical trials, including Phase 2 clinical trials directed to the potential treatment of amyotrophic lateral sclerosis (ALS) and spinal muscular atrophy (SMA). Results from both these clinical trials are expected in 2018.

### 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 over 6,000 rare diseases, 25 million Americans are living with a rare disease, but only 5 percent of these diseases have a treatment.

### About ALS

Amyotrophic lateral sclerosis (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 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 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

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* (CK-2127107), a next-generation FSTA. *Reldesemtiv* has been granted orphan drug designation by the FDA for the potential treatment of SMA. *Reldesemtiv* is the subject of three ongoing Phase 2 clinical trials enrolling patients with spinal muscular atrophy, chronic obstructive pulmonary disease and ALS. 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; 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, 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; 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.

**Contact:**

Cytokinetics

Diane Weiser

Vice President, Corporate Communications, Investor Relations

(415) 290-7757



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