Cytokinetics Joins Global Initiative to Recognize International Rare Disease Day

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SOUTH SAN FRANCISCO, Calif., Feb. 28, 2020 (GLOBE NEWSWIRE) -- Cytokinetics, Incorporated (Nasdaq: CYTK) today announced that on February 29 it is joining the European Organisation for Rare Diseases (EURORDIS) and the National Organization for Rare Disorders (NORD) to recognize Rare Disease Day®, an international campaign elevating the public understanding of rare diseases. This year's theme for Rare Disease Day, "Rare is Many. Rare is Strong. Rare is Proud," represents a shift in the global campaign to increase equity for the rare disease community and to reframe perceptions of what it means to be "rare." The campaign aims to build awareness of the importance of equitable access to diagnosis, treatment and care.

"We are pleased to again voice our support for this year's Rare Disease Day campaign as equity for all patients with rare diseases is consistent with our culture and company's core values," said Robert I. Blum, Cytokinetics' President and Chief Executive Officer. "We honor the courageous and inspirational patients who battle rare diseases every day, and we remain committed to developing potential medicines for devastating diseases of muscle dysfunction and weakness."

Cytokinetics is developing *reldesemtiv*, a fast skeletal muscle troponin activator, for the potential treatment of amyotrophic lateral sclerosis (ALS) and spinal muscular atrophy (SMA). Results from FORTITUDE-ALS (**F**unctional **O**utcomes in a **R**andomized **T**rial of **I**nvestigational **T**reatment with CK-2127107 to **U**nderstand **D**ecline in **E**ndpoints – in **ALS**), were presented in 2019 and, while statistical significance for the primary endpoint was not met, the study demonstrated that patients on all dose groups of *reldesemtiv* declined less than patients on placebo for slow vital capacity, a measure of respiratory function, and ALSFRS-R, a measure of disease progression, with larger and clinically meaningful differences emerging over time. This year, the company will engage with regulatory and reimbursement authorities to prepare for a potential Phase 3 clinical trial and registration program for *reldesemtiv* in patients with ALS. The U.S. Food and Drug Administration (FDA) has granted orphan drug designation to *reldesemtiv* for the treatment of amyotrophic lateral sclerosis (ALS). Previously *reldesemtiv* was granted orphan drug designation for the treatment of spinal muscular atrophy (SMA) by the FDA and by the European Medicines Agency.

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 20,000 people in the United States and a comparable number of patients in Europe. Approximately 5,000 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

Spinal muscular atrophy (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 1 (Infantile), Type 2 (Intermediate), Type 3 (Juvenile) and Type 4 (Adult onset). Of the prevalent population, approximately 80% of the patients are characterized as Type 2 and Type 3. Life expectancy and disease severity vary by type of SMA. Type 1 patients have the worst prognosis, with a life expectancy of no more than two years unless treated with SMN-directed therapies; Type 2 patients have delayed motor milestones with the most advanced milestone normally achieved being sitting unsupported; Type 3 patients can usually stand and walk but have increasingly limited mobility as their abilities regress as they age; Type 4 patients may have a normal life span but eventually suffer gradual weakness in the proximal muscles of the extremities, eventually resulting in mobility issues. With the recent introduction of SMN-directed therapies, it is expected that patients may live longer, but will still have a significant need to address ongoing disabilities related to respiration and mobility.

About Cytokinetics

Cytokinetics is a late-stage biopharmaceutical company focused on discovering, developing and commercializing first-in-class muscle activators and next-in-class muscle inhibitors 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 impact 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 an international clinical trials program in patients with heart failure including GALACTIC-HF and METEORIC-HF. 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 fast skeletal muscle troponin activator (FSTA). Astellas currently holds an exclusive worldwide license to develop and commercialize *reldesemtiv*. Licenses held by Amgen and Astellas are subject to specified co-development and co-commercialization rights of Cytokinetics. Cytokinetics is also developing CK-274, a novel cardiac myosin inhibitor that company scientists discovered independent of its collaborations, for the potential treatment of hypertrophic cardiomyopathies. Cytokinetics continues its over 20-year history of pioneering innovation in muscle biology and related pharmacology focused to diseases of muscle dysfunction and conditions of muscle weakness.

For additional information about Cytokinetics, visit www.cytokinetics.com and follow us on Twitter, LinkedIn, Facebook and YouTube.

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 the potential benefits of *reldesemtiv*, 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.

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