



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

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SOUTH SAN FRANCISCO, Calif., Feb. 26, 2021 (GLOBE NEWSWIRE) -- Cytokinetics, Incorporated (Nasdaq: CYTK) today announced that on February 28 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 awareness and public understanding of rare diseases. The initiative's key message of "Rare is many, rare is strong, rare is proud," spotlights the more than 300 million people worldwide living with a rare disease, and the awareness efforts focused on bringing them more equitable access to diagnosis, treatment, care and social opportunity.

"As the world navigates the ongoing COVID-19 pandemic, people living with rare diseases need our support, now more than ever, and we are especially proud to join with national and global organizations to shine a light on Rare Disease Day," said Robert I. Blum, Cytokinetics' President and Chief Executive Officer. "We gain tremendous inspiration from people overcoming the daily challenges of devastating rare diseases and remain committed to the development of potential medicines that arise from our leadership and expertise in muscle biology research."

Cytokinetics is developing CK-3773274 (CK-274), a next-generation cardiac myosin inhibitor, for the potential treatment of hypertrophic cardiomyopathy (HCM). Cytokinetics is conducting REDWOOD-HCM (Randomized Evaluation of Dosing With CK-274 in Obstructive Outflow Disease in HCM), a Phase 2 clinical trial of CK-274 in patients with obstructive HCM (oHCM). Results are expected in mid-2021, with the goal of starting a Phase 3 clinical trial by year end. CK-274 was recently granted orphan drug designation for the treatment of symptomatic HCM by the U.S. Food and Drug Administration (FDA).

Cytokinetics is also developing *rel-desemtiv*, a fast skeletal muscle troponin activator, for the potential treatment of amyotrophic lateral sclerosis (ALS) and spinal muscular atrophy (SMA). This year, the company is preparing for COURAGE-ALS, the planned Phase 3 clinical trial of *rel-desemtiv* in patients with ALS. *Rel-desemtiv* has been granted orphan drug designation for the treatment of ALS by the FDA and the European Medicines Agency (EMA). *Rel-desemtiv* was also granted orphan drug designation for the treatment of SMA by the FDA and EMA.

### 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 HCM

Hypertrophic cardiomyopathy (HCM) is a disease in which the heart muscle (myocardium) becomes abnormally thick (hypertrophied). The thickening of cardiac muscle leads to the inside of the left ventricle becoming smaller and stiffer, and thus the ventricle becomes less able to relax and fill with blood. This ultimately limits the heart's pumping function, resulting in symptoms including chest pain, dizziness, shortness of breath, or fainting during physical activity. A subset of patients with HCM are at high risk of progressive disease which can lead to atrial fibrillation, stroke and death due to arrhythmias. There are no FDA approved medical treatments that directly address the hypercontractility that underlies HCM.

### 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 preparing for regulatory interactions for *omecamtiv mecarbil*, its novel cardiac muscle activator, following positive results from GALACTIC-HF, a large, international Phase 3 clinical trial in patients with heart failure. Cytokinetics is conducting METEORIC-HF, a second Phase 3 clinical trial of *omecamtiv mecarbil*. Cytokinetics is also developing CK-274, a next-generation cardiac myosin inhibitor, for the potential treatment of hypertrophic cardiomyopathies (HCM). Cytokinetics is conducting REDWOOD-HCM, a Phase 2 clinical trial of CK-274 in patients with obstructive HCM. Cytokinetics is also developing *rel-desemtiv*, a fast skeletal muscle troponin activator for the potential treatment of ALS and other neuromuscular indications following conduct of FORTITUDE-ALS and other Phase 2 clinical trials. The company is

preparing for the potential advancement of *reldesemtiv* to a Phase 3 clinical trial in ALS. 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](http://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 Cytokinetics' and its partners' research and development activities of Cytokinetics' product 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 the risks related to Cytokinetics' business outlined in 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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