

# Cytokinetics' President & CEO Robert Blum Receives 2014 Lou Gehrig Iron Horse Award From ALS Therapy Development Institute

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**SOUTH SAN FRANCISCO, CA, November 10, 2014** - Cytokinetics, Incorporated (Nasdaq: CYTK) announced today that the Company's President and CEO, Robert Blum, received the 2014 Lou Gehrig Iron Horse Award from ALS Therapy Development Institute (ALS.net) at the Fourth Annual A White Coat Affair Gala on Saturday, November 8, 2014.

The award is the organization's top honor named after Lou Gehrig, who suffered from amyotrophic lateral sclerosis (ALS) and earned the nickname "Iron Horse" for his prowess, durability and character as a Hall of Fame baseball player for the New York Yankees. Lou Gehrig's strength and endurance enabled him to play a then-record 2,130 consecutive games. He retired from baseball in 1939 after being diagnosed with ALS. Gehrig passed away two years later due to complications associated with his disease and his name has become synonymous with ALS and the battle for hope for new treatment options.

"I am proud to accept the 2014 Lou Gehrig Iron Horse Award on behalf of my colleagues at Cytokinetics," stated Robert Blum. "This award is a tribute to our employees all of whom are steadfastly committed to the fight against ALS. We are pleased to be recognized by the Institute as our two organizations share commitment to the development of novel mechanism treatments for patients living with ALS. Inspired by patients with ALS like Lou Gehrig who portray everyday courage, Cytokinetics employees similarly demonstrate conviction and dedication every day in the fight against this grievous disease."

Cytokinetics, a clinical stage biopharmaceutical company, is developing *tirasemtiv*, a fast skeletal muscle activator, as a potential treatment for ALS. *Tirasemtiv* is the subject of a Phase II clinical trials program and 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. In BENEFIT-ALS, a recently completed Phase IIb trial, treatment with *tirasemtiv* resulted in a statistically significant and potentially clinically meaningful reduction in the decline of SVC, a measure of the strength of the skeletal muscles responsible for breathing that has been shown to be an important predictor of disease progression and survival in prior trials of patients with ALS.

# **About Amyotrophic Lateral Sclerosis**

Amyotrophic lateral sclerosis 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 patients with ALS, resulting in a high unmet need for new therapeutic options to address the symptoms and modify disease progression.

# About Tirasemtiv

Tirasemtiv selectively activates the fast skeletal muscle troponin complex by increasing its sensitivity to calcium and, in preclinical studies and early clinical trials, increased skeletal muscle force in response to neuronal input and delayed the onset and reduced the degree of muscle fatigue. BENEFIT-ALS (Blinded Evaluation of Neuromuscular Effects and Functional Improvement with Tirasemtiv in ALS) was a Phase IIb, multi-national, double-blind, randomized, placebo-controlled, clinical trial designed to evaluate the safety, tolerability and efficacy of tirasemtiv in patients with ALS. BENEFIT-ALS enrolled 711 patients from 73 centers in 8 countries; 605 patients were subsequently randomized 1:1 to double-blind treatment with either tirasemtiv or placebo for 12 weeks. The primary outcome measure, the ALS Functional Rating Scale in its revised form (ALSFRS-R), and secondary outcome measures of respiratory performance and other measures of skeletal muscle function and fatigability were assessed after 4, 8, and 12 weeks of double-blind treatment, and again at 1 and 4 weeks after the last dose of double-blind treatment. In BENEFIT-ALS, the change from baseline to the average of the ALSFRS-R total scores obtained after 8 and 12 weeks of double-blind treatment was not statistically different between the treatment groups. Treatment with tirasemtiv resulted in a statistically significant and potentially clinically meaningful slowing of the rate of decline of SVC versus placebo; the reduction from baseline in SVC was statistically significantly smaller on tirasemtiv versus placebo at each time point. The difference in the reduction from baseline in SVC in patients treated with tirasemtiv versus those on placebo persisted for at least four weeks following the last dose of double-blind medication.

# About ALS Therapy Development Institute (ALS.net)

The ALS Therapy Development Institute (ALS.net) and its scientists actively discover and develop treatments for ALS. ALS.net is the world's first and largest nonprofit biotech focused 100 percent on ALS research. Led by ALS patients and their families, the charity understands the urgent need to slow and stop this horrible disease. ALS.net, based in Cambridge, MA, has served as one of the leaders in sharing data and information with academic and ALS research organizations, patients and their families. For more information, visit <a href="https://www.als.net">www.als.net</a>.

# **About Cytokinetics**

Cytokinetics is a clinical-stage biopharmaceutical company focused on the discovery and development of novel small molecule therapeutics that modulate muscle function for the potential treatment of serious diseases and medical conditions. Cytokinetics' lead drug candidate from its cardiac muscle contractility program, *omecamtiv mecarbil*, is in Phase II clinical development for the potential treatment of heart failure. Amgen Inc. holds an exclusive license worldwide to develop and commercialize *omecamtiv mecarbil* and related compounds, subject to Cytokinetics' specified development and commercialization participation rights. Cytokinetics is independently developing *tirasemtiv*, a fast skeletal muscle activator, as a potential treatment for diseases and medical conditions associated with neuromuscular dysfunction. *Tirasemtiv* is the subject of a Phase II clinical trials program and 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 amyotrophic lateral sclerosis (ALS). Cytokinetics is collaborating with Astellas Pharma Inc. to develop CK-2127107, a skeletal muscle activator structurally distinct from *tirasemtiv*, for non-neuromuscular indications. All of these drug candidates have arisen from Cytokinetics' muscle biology focused research activities and are directed towards the cytoskeleton. The cytoskeleton is a complex biological infrastructure that plays a fundamental role within every human cell. Additional information about Cytokinetics can be obtained at <a href="https://www.cytokinetics.com">www.cytokinetics.com</a>.

#### **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' research and development activities, including the potential significance and utility of the results from BENEFIT-ALS and other studies of tirasemtiv; the potential size of markets for tirasemtiv; and the properties and potential benefits of tirasemtiv 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 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 trials 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.

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