



Regenacy Pharmaceuticals Announces Collaboration with the Charcot-Marie-Tooth Association (CMTA) to Advance Ricolinostat for the Treatment of Hereditary Neuropathy

*-- Collaboration to explore role of HDAC6 in multiple forms of CMT disease --
-- Company appoints Dr. David Herrmann to Scientific Advisory Board --*

Waltham, Mass., August 2, 2018 – [Regenacy Pharmaceuticals, LLC](#), a clinical-stage biopharmaceutical company developing breakthrough treatments for diabetic and other peripheral neuropathies, today announced a collaboration with the Charcot-Marie-Tooth Association (CMTA), a registered non-profit organization serving the hereditary neuropathy patient community, to validate the role of HDAC6 in multiple forms of Charcot-Marie-Tooth (CMT) disease and evaluate the efficacy of ricolinostat, a selective HDAC6 inhibitor, in animal models. In addition, the company announced the appointment of David Herrmann, M.B.B.Ch., to its Scientific Advisory Board. Dr. Herrmann is Chief of the Neuromuscular Division & Director of the Peripheral Neuropathy Clinics and Cutaneous Innervation Laboratory in the Department of Neurology at the University of Rochester, and a member of the STAR (Strategy to Accelerate Research) advisory board for the CMTA.

“We are thrilled to have such a substantial collaboration to broaden our programs for ricolinostat into inherited forms of neuropathy where there is a tremendous unmet need,” said Matt Jarpe, Ph.D., Vice President of R&D of Regenacy Pharmaceuticals. “This alliance with the Charcot-Marie-Tooth Association will enable us to expand our understanding of the role of HDAC6 in neuropathic diseases and lead us closer to initiating clinical trials in CMT disease.”

CMT disease is a progressive and degenerative nerve disease that usually appears in adolescence or early adulthood. Symptoms can include muscle weakness, decreased muscle size, foot-drop, foot bone abnormalities, fatigue, balance problems, neuropathic and/or musculoskeletal pain, loss of feeling in the hands and feet and loss of coordination in the limbs. There are no FDA approved treatments to stop or reverse the loss of nerve function in CMT. Based on the terms of the collaboration, Regenacy has an opportunity to expand on the groundbreaking [work](#) of Dr. Ludo van den Bosch at University of Leuven and Dr. Andrew Grierson at University of Sheffield to show the role of HDAC6 in several forms of CMT Type 2. The collaboration will focus on evaluating the efficacy of ricolinostat in animal models of CMT to support initiation of clinical trials. This relationship is taking advantage of the extensive suite of expert preclinical testing capabilities the CMTA has assembled and makes available to groups such as Regenacy that want to evaluate therapeutic potential in a CMT disorder.

“In parallel to announcing our collaboration with CMTA, we are pleased to welcome Dr. David Herrmann to our Scientific Advisory Board,” added Dr. Jarpe. “Dr. Herrmann brings to Regenacy relevant and valuable experience in clinical outcome measures in peripheral neuropathies, particularly CMT disease. We are looking forward to his contributions and proven strategic expertise as we continue to develop ricolinostat for the treatment of CMT.”

Dr. Herrmann is currently Professor of Neurology, Pathology and Laboratory Medicine at the University of Rochester. He established one of the first cutaneous innervation laboratories in the United States at University of Rochester for diagnosis of small fiber neuropathy. In addition to his role on the STAR advisory

board for the CMTA, Dr. Herrmann is a member of the Inherited Neuropathies Consortium. He is currently Principal Investigator in Rochester for the NIH sponsored Inherited Neuropathy Consortium Rare Disease Clinical Research Center. Dr. Herrmann received his medical degree from The University of Witwatersrand Medical School.

“I am personally passionate about inherited neuropathies for which there is an urgency to develop treatments,” said Dr. Herrmann. “Current treatment options for Charcot-Marie-Tooth disease, and related disorders, are very limited so each potential new therapy can make a critical difference for patients in need. I am excited to collaborate with the highly regarded Regenacy team, who are leaders in the field and are committed to bringing forward new treatment options in various types of peripheral neuropathy.”

About Charcot-Marie-Tooth Association (CMTA)

The Charcot-Marie-Tooth Association is a leading patient led nonprofit organization dedicated to finding a cure for CMT. The CMTA’s Strategy to Accelerate Research (STAR) program brings top researchers together with pharmaceutical and biotechnology partners to accelerate scientific breakthroughs. The CMTA also offers community services including 70 local branches, Camp Footprint exclusively for teens with CMT, and patient conferences. To learn more about the CMTA, please visit www.cmtausa.org.

About HDAC6 Inhibition

HDAC6 is an enzyme that regulates mitochondrial function and intracellular transport along microtubules. Peripheral nerves, due to their length, are uniquely sensitive to metabolic and chemical influences that disrupt intracellular transport, leading to debilitating pain, numbness and loss of muscle control, partially caused by mitochondrial dysfunction. Recent studies in animal models of diabetic, chemotherapy induced and inherited peripheral neuropathy, have implicated HDAC6 as a promising therapeutic target to restore nerve function in the periphery and significantly alleviate the symptoms of pain, motor function loss and numbness.

About Regenacy

Regenacy Pharmaceuticals, LLC is a clinical-stage biopharmaceutical company regenerating biological function by protein acetylation for the treatment of diabetic and other peripheral neuropathies and other chronic conditions. The company’s selective inhibition technology provides superior safety profiles and potential enhanced efficacy compared to non-selective HDAC inhibitors. Regenacy’s programs selectively inhibit histone deacetylase 6 (HDAC6) to restore normal intracellular protein and organelle transport in peripheral neuropathies, and a platform of selective HDACs 1 and 2 inhibitors that have potential to treat major blood diseases such as sickle cell disease, β -thalassemia, leukemia, and cognitive dysfunction in neurological disorders. www.regenacy.com

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