



Rain Therapeutics Initiates Phase 3 MANTRA Clinical Trial of Milademetan for De-differentiated Liposarcoma and Provides Patient Update from Prior Clinical Program

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The randomized, multi-center, open-label Phase 3 trial will evaluate the efficacy and safety of milademetan in patients with de-differentiated (DD) liposarcoma (LPS)

Multiple patients with liposarcoma have demonstrated long-term therapy with milademetan monotherapy now exceeding 4 years

NEWARK, Calif., July 20, 2021 (GLOBE NEWSWIRE) -- Rain Therapeutics Inc., a clinical-stage company developing precision oncology therapeutics, today announced that the first patient has been randomized in the multicenter, open-label, Phase 3 registrational study (MANTRA) evaluating milademetan (RAIN-32), an oral mouse double minute 2 (MDM2) inhibitor, for the treatment of DD LPS.

"The start of our Phase 3 MANTRA study evaluating milademetan marks an important step forward in addressing a high unmet need for patients with DD LPS," said Richard Bryce, MBChB, Chief Medical Officer at Rain Therapeutics. "We are proud to have advanced milademetan into a pivotal study less than 12 months after acquiring the program, and believe it has the potential to be the best-in-class MDM2 inhibitor."

The MANTRA trial, a randomized, multicenter, open-label, Phase 3 registrational study, is designed to evaluate the safety and efficacy of RAIN-32 compared to trabectedin, a current standard of care, in patients with unresectable or metastatic DD LPS with or without a well-differentiated (WD) LPS component that has progressed on one or more prior systemic therapies, including at least one anthracycline-based therapy. Approximately 160 patients are expected to be randomized in a 1:1 ratio to receive milademetan or trabectedin. The primary objective of the trial is to compare progression-free survival (PFS) by blinded independent review between the milademetan treatment arm and the trabectedin control arm. Secondary endpoints include overall survival, PFS by investigator assessment, objective response rate, duration of response, disease control rate, safety and patient reported outcomes.

The Company also provided an update on patients continuing to receive RAIN-32 monotherapy from the previously concluded Phase 1 dose escalation and expansion study. As of July 1, 2021, three WD/DD LPS patients received therapy with milademetan monotherapy for greater than 51 months. Two of these patients continue to receive therapy with durations now at 51 and 57 months without disease progression, and an additional patient received therapy for greater than 59 months before discontinuation in the second quarter of 2021. This highlights the potential for a favorable milademetan long-term tolerability and safety profile.

About Well-Differentiated/Dedifferentiated Liposarcoma

Liposarcoma is a rare cancer originating from fat cells located in the soft tissues of the body. It is a malignant cancer that can spread to other parts of the body. Well-differentiated LPS is less aggressive and tends to be a large painless mass found in deeper tissues. Dedifferentiated (DD) LPS is more aggressive, arising from WD LPS, and is usually found in tissue behind the abdominal area or the extremities. WD/DD LPS are the most frequent subtypes of LPS and share common genomic abnormalities, predominately MDM2 gene amplification. The incidence of LPS is estimated at approximately 3,000 patients annually in the U.S. for which there are few effective treatment options.

About RAIN-32

Milademetan (RAIN-32) is a small molecule, oral inhibitor of MDM2, which is oncogenic in numerous cancers. Milademetan has already demonstrated meaningful antitumor activity in an MDM2-amplified subtype of LPS and other solid tumors in a Phase 1 clinical trial, validating a rationally-designed dosing schedule to mitigate safety concerns and widen the therapeutic window of MDM2 inhibition. In addition to the ongoing Phase 3 clinical trial evaluating milademetan in patients with LPS, Rain Therapeutics anticipates commencing a Phase 2 tumor-agnostic basket trial in certain solid tumors in the second half of 2021 and a Phase 2 trial in intimal sarcoma by early 2022. Milademetan has received U.S. Food and Drug Administration Orphan Drug Designation for patients with LPS.

About Rain Therapeutics Inc.

Rain Therapeutics Inc. is a clinical-stage precision oncology company developing therapies that target oncogenic drivers for which it is able to genetically select patients it believes will most likely benefit. This approach includes using a tumor-agnostic strategy to select patients based on their tumors' underlying genetics rather than histology. Rain's lead product candidate, milademetan (RAIN-32), is a small molecule, oral inhibitor of MDM2, which is oncogenic in numerous cancers. In addition to milademetan, Rain is also developing a preclinical program that is focused on inducing synthetic lethality in cancer cells by inhibiting RAD52. For more information, visit www.rainthera.com.

Forward Looking Statements

Statements contained in this press release regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements include statements regarding, among other things, Rain's ongoing and planned studies for RAIN-32 (milademetan). Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Words such as "plans," "will," "anticipates," "goal," "potential," "expects" and similar expressions are intended to identify forward-looking statements. These forward-looking statements are based upon Rain's current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, which include, without limitation, risks and uncertainties associated with Rain's business in general and limited operating history, difficulty enrolling patients in our clinical trials given the relatively small LPS patient

population, Rain's reliance on third parties to conduct and support its preclinical studies and clinical trials, and the other risks described in Rain's filings with the Securities and Exchange Commission. All forward-looking statements contained in this press release speak only as of the date on which they were made and are based on management's assumptions and estimates as of such date. Rain undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made, except as required by law.

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