



Rain Therapeutics Presents Data on Milademetan (RAIN-32) at the 2021 AACR-NCI-EORTC Virtual International Conference on Molecular Targets and Cancer Therapeutics

October 7, 2021

Presentations highlight potential of the oral MDM2 inhibitor, milademetan, in advanced cancers with MDM2 amplification, GATA3 mutant ER-positive breast cancer, and Merkel cell carcinoma

NEWARK, Calif., Oct. 07, 2021 (GLOBE NEWSWIRE) -- Rain Therapeutics Inc., (NasdaqGS: RAIN), ("Rain"), a late-stage company developing precision oncology therapeutics, today announced data on its oral mouse double minute 2 (MDM2) inhibitor, milademetan, presented at the 2021 AACR-NCI-EORTC Virtual International Conference on Molecular Targets and Cancer Therapeutics held virtually October 7-10, 2021.

Three poster presentations detailed: 1) *in vivo* and *in vitro* activity of milademetan using a rationally-derived MDM2 copy number threshold as a predictive biomarker for patient selection, 2) *in vivo* and *in vitro* activity of milademetan in Merkel cell carcinoma (MCC) models and 3) results demonstrating milademetan induces synthetic lethality of (ER)-positive breast tumors with GATA3 mutations.

Key findings from Rain's presentations include:

- Milademetan demonstrated anti-tumor activity in patient-derived organoid models and xenograft models consisting of genetically selected tumors, representing many different histologies, using MDM2 gene amplification (amp) and wild-type (WT) p53 as selection criteria.
 - Robust induction of p53 target genes including MIC-1, p21 and PUMA was observed following milademetan treatment indicating re-activation of p53 by milademetan.
- Milademetan inhibited MCC cell lines in patient-derived xenograft models that lack TP53 mutations, representing the majority of MCC cases, for which no approved targeted therapies are available.
 - MCC has low rates of p53 mutation and MDM2 dependence in MCC is driven by polyoma virus-induced MDM2 expression in the majority of cases.
- Milademetan inhibited proliferation of GATA3 frameshift (fs) mutant ER+ breast cancers, demonstrating significant activity and the potential to treat ER+, GATA3 mutant breast cancers that have been unresponsive to the current standard of care.
 - GATA3 fs mutations are mutually exclusive of p53 mutations in ER+ breast cancer and are associated with higher expression of MDM2 and other genes in the MDM2/p53 axis associated with p53 inactivation.

"These data further support the potential of milademetan as a targeted therapy in genetically pre-defined cancer patients beyond our lead indication in liposarcoma," said Robert Doebele, M.D., Ph.D., co-founder, president and chief scientific officer of Rain Therapeutics. "These therapeutic opportunities represent areas of unmet need where the tumors may exhibit MDM2 dependency and for which milademetan therapy alone or in combination may lead to clinically meaningful outcomes."

Copies of each poster will be available by visiting the ["Events & Presentations"](#) section of the Rain website.

About Milademetan

Milademetan 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 late-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, 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.

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. 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" 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 the risks and uncertainties 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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