

Rain Oncology Announces Topline Results from Phase 3 MANTRA Trial of Milademetan for the Treatment of Dedifferentiated Liposarcoma

- The Phase 3 MANTRA trial did not meet the primary endpoint of progression free survival vs.
 standard of care –
- The median PFS was 3.6 months for milademetan versus 2.2 months for trabectedin with a
 hazard ratio of 0.89 –

- Rain to host a conference call at 8:30 a.m. ET -

NEWARK, Calif., May 22, 2023 – Rain Oncology Inc. (NasdaqGS: RAIN), (Rain), a late-stage company developing precision oncology therapeutics with its lead product candidate, milademetan, an oral, small molecule inhibitor of the MDM2-p53 complex that reactivates p53, today announced its topline pivotal Phase 3 MANTRA data. The trial, evaluating the efficacy, safety, and tolerability of milademetan in patients with dedifferentiated (DD) liposarcoma (LPS), did not meet its primary endpoint of progression free survival (PFS) by blinded independent central review compared to the standard of care, trabectedin.

The median PFS for milademetan was 3.6 months vs 2.2 months for trabectedin, with a hazard ratio of 0.89, p=0.53. The most common treatment emergent adverse events (TEAEs) in the milademetan arm included nausea, thrombocytopenia, anemia, vomiting and neutropenia. The most common Grade 3/4 TEAEs were thrombocytopenia (39.5%), neutropenia (25.5%) and anemia (18.6%). Dose reductions in the milademetan arm were 44.2% vs 29.1% in the trabectedin arm. Discontinuation in the milademetan arm due to AEs were 11.6% vs 19.0% for trabectedin. Based upon these topline data, Rain does not expect to pursue further development of milademetan in DD LPS. Rain hopes to present the MANTRA data in an upcoming medical conference.

"We are very disappointed in the outcome of the MANTRA trial, as the results did not closely mirror prior clinical results in patients with DD LPS," said Avanish Vellanki, co-founder and chief executive officer of Rain. "We are truly saddened we will not likely be able to offer patients new treatment options for this challenging disease. However, the quality and robustness of the global MANTRA trial reflects an unambiguous data set. Rain's mission remains to advance science, and therefore we will further evaluate the totality of the MANTRA data to support the scientific and medical community in the hope we can aid others in finding new strategies for patients with DD LPS. Based on the MANTRA topline results, we will also re-evaluate the path forward for milademetan. We continue to believe that reactivating p53 is an important avenue to pursue as part of a treatment strategy across cancer. I would like to extend our sincerest



gratitude to the patients and clinicians who participated in the trial as well as our dedicated team."

Phase 3 MANTRA Topline Data Results:

- The median PFS was 3.6 months with milademetan versus 2.2 months for trabectedin, with a hazard ratio of 0.89 (95% CI [0.61 to 1.29]; p=0.53) based on 115 events
- Most common TEAEs in the milademetan arm included nausea, thrombocytopenia, anemia, vomiting and neutropenia
- The most common Grade 3/4 TEAEs in the milademetan arm were thrombocytopenia (39.5%), neutropenia (25.5%) and anemia (18.6%)
- Dose reductions in the milademetan arm were 44.2% vs 29.1% in the trabectedin arm
- Discontinuations in the milademetan arm due to AEs were 11.6% vs 19.0% for trabectedin
- Treatment emergent SAEs in the milademetan arm were 36.0% vs 48.1% in the trabectedin arm

The Rain management team will host a conference call today at 8:30 a.m. ET to discuss the topline data. The dial-in number for the conference call is 877-704-4453 for domestic participants and 201-389-0920 for international participants, with Conference ID: 13739033. A live webcast of the conference call can be accessed at the "Events" page on the Rain website, or by clicking here. A replay will be available shortly after conclusion of the event.

About MANTRA Trial

The MANTRA trial, a randomized, multicenter, open-label, Phase 3 registrational study, was designed to evaluate the safety and efficacy of milademetan compared to trabectedin, a current standard of care, in patients with unresectable or metastatic DD LPS with or without a WD LPS component that has progressed on one or more prior systemic therapies, including at least one anthracycline-based therapy. 175 patients were enrolled and randomized in a 1:1 ratio to receive milademetan or trabectedin. The primary objective of the trial was to compare PFS by blinded independent central review between the milademetan treatment arm and the trabectedin control arm. Secondary endpoints included overall survival, PFS by investigator assessment, objective response rate, duration of response, disease control rate, safety and patient reported outcomes.

About Milademetan

Milademetan (also known as RAIN-32) is an oral small molecule inhibitor of the MDM2-p53 complex that reactivates p53. Milademetan has demonstrated antitumor activity in an MDM2-amplified subtype of liposarcoma (LPS) and other solid tumors in a Phase 1 clinical trial, supported by a rationally designed dosing schedule to mitigate safety concerns and widen the potential therapeutic window of inhibition of the MDM2-p53 complex. Rain has completed its



Phase 3 trial of milademetan (MANTRA) in patients with LPS, and is evaluating milademetan in a Phase 2 tumor-agnostic basket trial in certain solid tumors with MDM2 amplification (MANTRA-2). Rain anticipates commencing a Phase 1/2 clinical trial to evaluate the safety, tolerability and efficacy of milademetan in combination with Roche's atezolizumab in patients with loss of cyclin-dependent kinase inhibitor 2A (CDKN2A) and wildtype p53 advanced solid tumors (MANTRA-4), in mid-2023. Milademetan has received Orphan Drug Designation from the U.S. Food and Drug Administration (FDA) for the treatment of LPS.

About Rain Oncology Inc.

Rain Oncology Inc. is a late-stage precision oncology company developing therapies that target oncogenic drivers 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 product candidate, milademetan, is a small molecule, oral inhibitor of the MDM2-p53 complex that reactivates p53.

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 clinical strategy, the therapeutic potential of the reactivation of p53 and a potential path forward for 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, Rain's ability to execute on its strategy; the results of preclinical and clinical studies; the FDA regulatory process; Rain's reliance on third parties to conduct and support its preclinical studies and clinical trials, positive results from a clinical trial may not necessarily be predictive of the results of future or ongoing clinical studies; the effect of public health crises on Rain's clinical trials and business operations, the impact of general economic, health, industrial or political conditions in the United States or internationally, the sufficiency of Rain's capital resources and its ability to raise additional capital, and the other risks described in Rain's Annual Report on Form 10-Q for the quarter ended March 31, 2023 and subsequent 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.



Media Contact

Jordyn Temperato
LifeSci Communications
jtemperato@lifescicomms.com

Investor Contact

Dan Ferry LifeSci Advisors +1.617.430.7576 daniel@lifesciadvisors.com