Rafael Pharmaceuticals Announces the Successful Completion of Phase 1b Clinical Trial and Initiates Phase 2 for CPI-613® (devimistat) in Combination with Gemcitabine and Cisplatin in Patients with Biliary Tract Cancer

CRANBURY, N.J., Aug. 25, 2021 (GLOBE NEWSWIRE) -- -- Rafael Pharmaceuticals, Inc. ("Rafael" or the "Company"), a leader in the growing field of cancer metabolism-based therapeutics, today announced the successful completion of a Phase 1b clinical trial of CPI-613® (devimistat) in combination with gemcitabine and cisplatin in patients with biliary tract cancer. In collaboration with Michigan Medicine, the Phase 1b study consisted of a multicenter trial of gemcitabine and cisplatin with devimistat as first-line therapy for patients with locally advanced unresectable or metastatic biliary tract cancer who have had no prior treatment.

"We are extremely excited and gratified to have completed Phase 1b and to be able to advance to the next phase (Phase 2)," said Sanjeev Luther, President and CEO of Rafael Pharmaceuticals. "Advanced biliary tract cancer is a rare and aggressive cancer with a 5-year survival rate of less than 5%, and the continuation of the trial aligns with our mission to help patients with significant unmet medical needs."

"The Phase 1b part of the trial has successfully identified the recommended Phase 2 dose of CPI-613 when given in combination with gemcitabine and cisplatin in this patient population. The 2:1 randomized Phase 2 part of the trial has already started enrolling patients and will now determine the efficacy of devimistat at this maximum tolerated dose in combination with gemcitabine and cisplatin as compared with the combination chemotherapy alone" said Dr. Vaibhav Sahai, MBBS, M.S., primary investigator and medical oncologist at the University of Michigan Rogel Cancer Center in Ann Arbor, Michigan. An estimated 68 to 78 patients will be enrolled in the study (NCT04203160).

About CPI-613® (devimistat)

CPI-613® (devimistat) is a first-in-class clinical lead compound of Rafael, which targets enzymes that are involved in cancer cell energy metabolism and are located in the mitochondria of cancer cells. Devimistat is designed to target the mitochondrial tricarboxylic acid (TCA) cycle, a process essential to tumor cell multiplication and survival, selectively in cancer cells. Devimistat substantially increases cellular stress and the sensitivity of cancer cells to a diverse range of chemotherapeutic agents. This synergy allows for potential combinations of devimistat with lower doses of these generally toxic drugs to be more effective with lower patient's side effects. Combination with devimistat represent a diverse range of opportunities to substantially improve patient's benefit in many different cancers. The U.S. Food and Drug Administration (FDA) has given Rafael approval to initiate pivotal Phase 3 clinical trials in pancreatic cancer (AVENGER 500) and acute myeloid leukemia (ARMADA 2000) and has designated devimistat as an orphan drug for the treatment of pancreatic cancer, acute myeloid leukemia, myelodysplastic syndrome, peripheral T-cell lymphoma, Soft Tissue Sarcoma, Burkitt's lymphoma and biliary tract cancer. The EMA has granted orphan drug designation to devimistat for pancreatic cancer and acute myeloid leukemia.

About Rafael Pharmaceuticals, Inc.

Rafael Pharmaceuticals is a leader in the growing field of cancer metabolism. The company is developing a new, first-in-class category of metabolic oncology therapeutics that attack hard-to-treat cancers by targeting the metabolic processes the disease needs to survive, grow and proliferate. Rafael Pharmaceuticals' lead compound, CPI-613® (devimistat), is a highly selective, well-tolerated and effective anti-cancer agent that is being evaluated in ongoing and completed Phase 1, 2 and 3 clinical trials. Devimistat has been granted orphan drug status by the FDA for the treatment of pancreatic cancer, acute myeloid leukemia (AML), myelodysplastic syndrome (MDS) and Burkitt's, peripheral T-cell lymphomas and soft tissue sarcoma and biliary tract cancer. The Company's investors include Rafael Holdings, Inc. (NYSE: RFL). On June 21, 2021, we announced that we have entered into a merger agreement with Rafael Holdings, Inc., to create a publicly traded late-stage clinical oncology company focused on cancer metabolism-based therapeutics. For more information, please visit www.rafaelpharma.com.

Safe Harbor Statement

This press release contains forward-looking statements. These statements relate to future events or the company's future financial performance. In some cases, you can identify forward-looking statements by terminology such as "may", "will", "should", "expect", "plan", "anticipate", "believe", "estimate", "predict", "potential" or "continue", the negative of such terms, or other comparable terminology. These statements are only predictions. Actual events or results may differ materially from those in the forward-looking statements as a result of various important factors. Although we believe that the expectations reflected in the forward-looking statements are reasonable, such statements should not be regarded as a representation by the company, or any other person, that such forward-looking statements will be achieved. The business and operations of the company are subject to substantial risks which increase the uncertainty inherent in forward-looking statements. We undertake no duty to update any of the forward-looking statements, whether as a result of new information, future events or otherwise.

In light of the foregoing, readers are cautioned not to place undue reliance on such forward-looking statements.

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