

Rafael Pharmaceuticals Announces Ongoing Successful Enrollment and Opening of Additional Sites for Phase 2 Clinical Trial of CPI-613® (Devimistat) in Combination with Gemcitabine and Cisplatin in Patients with Biliary Tract Cancer

CRANBURY, N.J., Jan. 18, 2022 (GLOBE NEWSWIRE) -- [Rafael Pharmaceuticals, Inc.](#) ("Rafael" or the "Company"), a company focused on the growing field of cancer metabolism-based therapeutics, today announced the ongoing successful enrollment rate of its Phase 2 clinical trial for CPI-613® (devimistat) in combination with gemcitabine and cisplatin in patients with biliary tract cancer. The multicenter trial is for patients with locally advanced unresectable or metastatic biliary tract cancer who have had no prior treatment. The trial is currently open through [The University of Michigan Health Rogel Cancer Center](#) as an investigator-sponsored trial. The trial is also open at [Fred Hutchinson Cancer Research Center](#) and [UT Southwestern Simmons Cancer Center](#). Rafael is also launching additional sites for the trial at [Northwestern University Lurie Cancer Center](#), [UH Seidman Cancer Center](#), [The University of Arizona Cancer Center](#), [University of Wisconsin Carbone Cancer Center](#), [Vanderbilt-Ingram Cancer Center](#), [Atlantic Health System Cancer Care](#), and [Allegheny Health Network Cancer Institute](#).

"We are pleased that our Phase 2 trial has continued to see a positive enrollment rate, demonstrating devimistat's ability to serve as a catalyst for hope and potential relief to families affected by this harrowing condition," said Sanjeev Luther, President and CEO of Rafael Pharmaceuticals. "We are happy to report a continuation of safety within the trial, as the wellbeing of our patients is our top priority."

"Biliary tract cancer is considered a very rare and aggressive form of cancer, with fewer than 20,000 U.S. cases per year and a 5-year survival rate of less than 5%," said [Dr. Vaibhav Sahai](#), MBBS, M.S., principal investigator on the trial and medical oncologist at the University of Michigan Rogel Cancer Center. "The research community remains engaged towards identifying devimistat's capabilities in biliary cancer which is being reflected through the strong and continued Phase 2 enrollment rate, and we're hopeful that the opening of our additional sites will allow families and their loved ones affected by biliary tract cancer an effective treatment option."

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 side effects. Combination with devimistat represents a diverse range of opportunities to substantially improve patients' benefit in many different cancers. The U.S. Food and Drug Administration (FDA) 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 European Medicines Agency (EMA) has granted orphan drug designation to devimistat for pancreatic cancer, acute myeloid leukemia and Burkitt's lymphoma.

About Rafael Pharmaceuticals, Inc.

Rafael Pharmaceuticals is focused on the growing field of cancer metabolism. The company is developing a new, first-in-class category of metabolic oncology therapeutic candidates that are designed to attack hard-to-treat cancers by targeting the metabolic processes that these cancers need to survive, grow and proliferate. Rafael Pharmaceuticals' lead compound, CPI-613® (devimistat), is an investigational anti-cancer agent that is being evaluated in ongoing and completed Phase 1, 2 and 3 clinical trials. 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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