

Rafael Pharmaceuticals Announces First Patient Enrolled in APOLLO 613 Phase 1/2 Clinical Trial of CPI-613® (Devimistat) in Patients with Relapsed Clear Cell Sarcoma

New Trial Sites Open at Cleveland Clinic Children's and University of Michigan

CRANBURY, N.J., Nov. 11, 2021 (GLOBE NEWSWIRE) -- [Rafael Pharmaceuticals, Inc.](#) ("Rafael" or the "Company"), a company focused on the growing field of cancer metabolism-based therapeutics, announced that the first patient has been enrolled in the APOLLO 613 Phase 1/2 clinical trial of CPI-613® (devimistat) in combination with hydroxychloroquine in patients with clear cell sarcoma at [City of Hope](#) in Duarte, California. Two additional sites have also been opened for enrollment in the trial at [Cleveland Clinic Children's](#) and [University of Michigan's Rogel Cancer Center](#).

"With the aggressive nature of clear cell sarcoma, finding a treatment for this rare cancer is a priority of ours," said Sanjeev Luther, President and CEO of Rafael Pharmaceuticals. "Enrolling a patient so soon after we opened the trial demonstrates the dire need for a treatment and the hope that devimistat brings to the rare cancer community. We opened two additional sites in order to increase access to the trial for patients in need."

Clear cell sarcoma is very difficult to diagnose, and the average age of diagnosis is 25 years old. By the time the disease is diagnosed, it is often in advanced stages when the cancer is aggressive and hard to treat.

"Clear cell sarcoma has the propensity to spread quickly within the body, and our current available medical therapies are not consistently beneficial to patients," said Rashmi Chugh, M.D., co-principal investigator from the University of Michigan. "Finding effective treatments for the disease is critical, and we are hopeful that devimistat's mechanism of action will be effective in clear cell sarcoma patients."

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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