Rafael Pharmaceuticals Announces Successful Completion of Dose Escalation with No Dose-Limiting Toxicity (DLT) in First Cohort of APOLLO 613 Phase 1/2 Clinical Trial of CPI-613® (Devimistat) in Patients with Relapsed Clear Cell Sarcoma

Company Will Open Additional Sites and Has Begun Enrolling Patients in the Second Cohort of the Trial

CRANBURY, N.J., Jan. 06, 2022 (GLOBE NEWSWIRE) -- Rafael Pharmaceuticals, Inc. ("Rafael" or the "Company"), a company focused on the growing field of cancer metabolism-based therapeutics, announced the completion of the first cohort of dose escalation with no dose-limiting toxicity (DLT) in the APOLLO 613 Phase 1/2 clinical trial of CPI-613® (devimistat) in combination with hydroxychloroquine in patients with relapsed clear cell sarcoma. Rafael is also launching additional sites for the trial at Seattle Children's and Atrium Health Wake Forest Baptist. Existing sites already enrolling patients include City of Hope in Duarte, California, Cleveland Clinic Children's, University of Michigan's Rogel Cancer Center and Vanderbilt University Medical Center.

"The success of our first cohort of dose escalation increases our optimism that devimistat may help address the gaps for efficacious treatment methods for relapsed clear cell sarcoma," said Sanjeev Luther, President and CEO of Rafael Pharmaceuticals. "There is a significant unmet need in treatments for clear cell sarcoma and other rare cancers, and as such, patients and physicians in the rare disease community are incredibly hopeful of devimistat's capabilities."

Clear cell sarcoma is very difficult to diagnose, and therefore, is often discovered in late stages. The disease often spreads to other parts of the body quickly and has a high relapse rate. The estimated 5-year survival rate for clear cell sarcoma can be anywhere between 30% and 67%.

"Enrolling patients into our second cohort of dose escalation so soon after establishing safety in the first cohort demonstrates the dire need and demand for effective treatments for rare sarcomas," said Rashmi Chugh, M.D., co-principal investigator on the trial and clinical professor at the University of Michigan Health. "We are hopeful that devimistat will produce favorable outcomes for those suffering from relapsed clear cell sarcoma."

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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