Rafael Pharmaceuticals Announces APOLLO613 Phase I/II Clinical Trial of CPI-613® (devimistat) in Combination with Hydroxychloroquine in Patients with Relapsed Clear Cell Sarcoma Begins Enrollment at City of Hope in Duarte, Calif.

CRANBURY, N.J., Sept. 27, 2021 (GLOBE NEWSWIRE) -- Rafael Pharmaceuticals, Inc. ("Rafael" or the "Company"), a leader in the growing field of cancer metabolism-based therapeutics, announced that the Phase 1 clinical trial of CPI-613® (devimistat) in combination with hydroxychloroquine in patients with clear cell sarcoma is open for enrollment. The clinical trial will begin enrolling patients at City of Hope in Duarte, California, with other sites across the country to quickly follow.

"Clear cell sarcoma is truly one of the most challenging sarcomas to treat, as it often spreads quickly to other parts of the body and prognosis is generally poor," said Sanjeev Luther, President and CEO of Rafael Pharmaceuticals. "The opening of this trial at City of Hope and several other sites across the country has the potential to grant a population with significant unmet medical needs a promising new treatment option."

"We are optimistic that this trial will provide a potential path forward for individuals suffering from clear cell sarcoma, one of the rarest diseases in the world," said Mark Agulnik M.D., principal investigator from City of Hope.

"This aggressive sarcoma afflicts patients of all ages, especially children and young adults. Currently available standard therapies are limited in their benefit to patients," said Rashmi Chugh, M.D., medical oncologist from the University of Michigan. Matteo Trucco M.D., pediatric oncologist from the Cleveland Clinic added, "This trial is pivotal as we work towards additional treatment options for patients and families affected by this aggressive disease." Chugh and Trucco are both co-principal investigators on this multicenter study.

According to the National Cancer Institute, clear cell sarcoma is so rare, it is relatively unknown how many people suffer from it. Although treatment options are scarce, the increased research around this cancer and potential treatment options signals hope for patients, families, caregivers and doctors.

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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