A New Phase 1 Study Began to Evaluate Rafael Pharmaceuticals' Lead Compound CPI-613®? (devimistat) for Patients With Pancreatic Cancer in Japan

CRANBURY, N.J., Oct. 20, 2020 (GLOBE NEWSWIRE) -- Rafael Pharmaceuticals, Inc. ("Rafael" or the "Company"), a leader in the growing field of cancer metabolism-based therapeutics announced today its partner Ono Pharmaceutical Co., Ltd. ("Ono"), a pharmaceutical company committed to creating innovative medicines, has begun a Phase 1 study in Japan for patients with pancreatic cancer. The multicenter, open-label study is evaluating the efficacy and safety of Rafael's lead compound, CPI-613®? (devimistat), labeled as ONO-7912 in Japan, in combination with modified FOLFIRINOX (mFFX) in patients with pancreatic cancer refractory or intolerance to chemotherapy including gemcitabine. This announcement comes on the heels of Rafael reaching its target enrollment of 500 patients in its global Phase 3 trial.

"After achieving the milestone of enrolling 500 patients in our global Phase 3 trial, we believe the natural next step is to work with Ono Pharmaceutical to bring the treatment to the Japanese market," said Sanjeev Luther, President and Chief Executive Officer of Rafael Pharmaceuticals. "Sadly, in Japan, patients with pancreatic cancer have the lowest survival rates. We are hopeful that bringing our trial to this market will allow us to help bridge the gap in survival rates and help more people around the globe."

In June 2019, the Company announced an out-licensing agreement with Japan-based Ono Pharmaceutical to further develop and commercialize devimistat. Ono will work with Rafael in supporting the trial through lending its deep expertise in the region.

"We continue to see the incidence and death rates of pancreatic cancer increase worldwide," said Philip A. Philip, M.D.,Ph.D., F.R.C.P., Professor of Oncology at the Barbara Ann Karmanos Cancer Institute at Wayne State University, and principal investigator of the global Phase 3 trial. "While pancreatic cancer remains a hard-to-treat disease, we refuse to believe it is an impossible feat. We are eager to bring hope to those living in Japan and continue to remain hopeful around positive outcomes for the trial."

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 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 represents 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 and Burkitt's lymphoma. 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 and peripheral T-cell lymphomas. The Company's investors include Rafael Holdings, Inc. (NYSE AMERICAN: RFL). 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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10/20/2020 9:00:00 AM