Rafael Pharmaceuticals Announces Expansion of Phase 2 Trial of CPI-613® (devimistat) for Patients with Relapsed or Refractory Burkitt's Lymphoma/Leukemia to Massachusetts General Hospital in Boston

Cranbury, N.J., Dec. 02, 2019 (GLOBE NEWSWIRE) -- Rafael Pharmaceuticals, Inc. ("Rafael" or the "Company"), a leader in the growing field of cancer metabolism-based therapeutics, today announced the expansion of its Phase 2 clinical trial of CPI-613® (devimistat) for patients with relapsed or refractory Burkitt's lymphoma/leukemia. The clinical trial will begin enrolling patients at Massachusetts General Hospital in Boston starting this month. The trial is currently underway at Memorial Sloan Kettering Cancer Center in New York City and at City of Hope in Duarte, California.

"Burkitt's lymphoma is a rare disease, with approximately 1,200 people in the United States diagnosed annually, so treatment options for these patients are very limited," said Sanjeev Luther, President and CEO of Rafael Pharmaceuticals. "The disease is highly aggressive and has a high rate of relapse. Expanding our clinical trial will increase access and create hope for patients and their loved ones across the country."

In June 2018, devimistat received orphan drug designation for the treatment of relapsed Burkitt's lymphoma from the U.S. Food and Drug Administration (FDA). Orphan drug designation refers to a special status granted by The Orphan Drug Act (ODA) to a drug used to treat a rare disease or condition.

"Currently no definitive second-line therapy exists for patients with relapsed Burkitt's lymphoma," said Ariela Noy, M.D., an oncologist at Memorial Sloan Kettering Cancer Center and principal investigator on the devimistat clinical trial for relapsed Burkitt's lymphoma. "The expansion of the clinical trial across the country will help reach patients that have a clear unmet medical need for additional treatment options."

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 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 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.

Disclosure: Dr. Ariela Noy receives research funding from Rafael Pharmaceuticals, Inc.

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