Rafael Pharmaceuticals Receives FDA Fast Track Designation for CPI-613® (devimistat) for the Treatment of Acute Myeloid Leukemia (AML)

CRANBURY, N.J., Dec. 15, 2020 (GLOBE NEWSWIRE) -- Rafael Pharmaceuticals, Inc. ("Rafael" or the "Company"), a leader in the growing field of cancer metabolism-based therapeutics, announced today that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for the Company's lead compound, CPI-613® (devimistat), for the treatment of acute myeloid leukemia (AML).

"Receiving Fast Track designation, especially during a pandemic that has created significant challenges for many trials across the globe, is a testament to the dedicated work of the Rafael team," said **Sanjeev Luther**, President and CEO of Rafael. "We would not be here without the support of the FDA, our doctors, our patients, and all who are invested in the hope of finding a successful treatment for this hard-to-treat cancer."

While one of the most common types of leukemia in adults, AML accounts for only 1% of all cancers.

"This designation underscores the pressing need to find new ways to combat this aggressive disease," said **Jorge Cortes**, M.D., Director of the Georgia Cancer Center at Augusta University and principal investigator on the Phase 3 clinical trial. "It brings hope not only to clinicians, but to patients who hear that they have been diagnosed."

"We would not be here today without our principal investigator Jorge Cortes, M.D., the FDA, and leadership at Rafael who remain focused on patient care," said **Timothy S. Pardee**, M.D., Ph.D., Co-Chief Medical Officer of the Company. "We are a community coming together to fight a common enemy, and I believe we are gaining ground in this battle every day."

This announcement comes on the heels of the Company receiving Fast Track designation for devimistat for the treatment of metastatic pancreatic cancer, in November. The company has continued to reach milestones throughout the year, including the recent Orphan Drug Designation for the treatment of soft tissue sarcoma for devimistat, and the initiation of a Phase 2 clinical trial of devimistat in combination with hydroxychloroquine in patients with clear cell sarcoma of soft tissue.

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, Burkitt's lymphoma and soft tissue sarcoma. 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. 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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