Rafael Pharmaceuticals Crosses Enrollment of 150 Patients in Pivotal Phase 3 Trial (ARMADA 2000) of CPI-613® (Devimistat) for Relapsed or Refractory Acute Myeloid Leukemia (AML)

Company meets enrollment milestones to support research and development of a new and novel treatment for hard-to-treat cancer

CRANBURY, N.J., March 30, 2021 (GLOBE NEWSWIRE) -- Rafael Pharmaceuticals, Inc. ("Rafael" or the "Company"), a leader in the growing field of cancer metabolism-based therapeutics, today announced that it has crossed the enrollment of 150 patients in its Phase 3 clinical trial for patients with relapsed or refractory acute myeloid leukemia (AML) (ARMADA 2000). The multicenter, open-label, randomized pivotal trial is assessing the efficacy and safety of Rafael's lead compound CPI-613® (devimistat) in combination with high dose cytarabine and mitoxantrone (CHAM) compared to high dose cytarabine and mitoxantrone (HAM) therapy in older patients. The announcement comes on the heels of the Company's achievement in receiving FDA fast-track designation for CPI-613® (devimistat) in the treatment of AML, at the end of 2020.

"Every enrollment is an opportunity for us to hope for the future of this therapy for very hard-to-treat cancers," said **Sanjeev Luther**, President and CEO of Rafael Pharmaceuticals. "The pace at which we are enrolling patients demonstrates the need for more effective AML treatments, and we have so much appreciation for the caregivers, patients, primary investigators and Rafael employees who contributed to help us surpass this important milestone."

There are currently no standard therapy for AML patients who suffer a relapse, and the prognosis for older patients is grim.

"The need for new and novel treatments in AML continues to grow alongside the estimated incidence rate," said Richard Larson, M.D., Director of the Hematologic Malignancies Program at the University of Chicago Medical Center and a principal investigator on the Phase 3 clinical trial. "As patients face a diagnosis with relapsed or refractory AML, the potential of these treatments is incredibly important."

"Having Dr. Larson and other leading clinicians contributing to the enrollment growth of this trial is immensely helpful," said Timothy S. Pardee, M.D., Ph.D., Co-Chief Medical Officer of the Company. "Ultimately, it is these milestones that will shepherd us towards finding treatment options for patients who need them most."

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, 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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Rafael Media Contact:
Diana Cleinmark
rafael@antennagroup.com
(408) 713-1216

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