Rafael Pharmaceuticals Partners with Sara's Cure and SARC for the Launch of Phase 2 Clinical Trial for CPI-613® (devimistat) in Combination with Hydroxychloroquine for Patients with Clear Cell Sarcoma of Soft Tissue

CRANBURY, N.J., Dec. 01, 2020 (GLOBE NEWSWIRE) -- Rafael Pharmaceuticals, Inc. ("Rafael" or the "Company"), a leader in the growing field of cancer metabolism-based therapeutics, today announced it will initiate a Phase 2 clinical trial of CPI-613®(devimistat) in combination with hydroxychloroquine in patients with clear cell sarcoma of soft tissue. In partnership with Sara's Cure and Sarcoma Alliance for Research through Collaboration (SARC), Rafael will begin enrolling patients into the Simon two-stage design trial at sites across the United States.

"Our daughter's battle with clear cell sarcoma was very eye opening around the lack of treatment options for patients with clear cell sarcoma, largely due to the rarity of the disease," said **Lennie Woods**, Founder and Executive Director of Sara's Cure. "When we learned about Rafael's dedication to fighting rare diseases, we immediately connected to discuss if devimistat can potentially be a solution in treating this disease. We are so grateful to have a partner like Rafael that truly cares about helping people like our daughter, and reminds our entire community that we are not alone in this fight."

Woods founded Sara's Cure after Sara, her teenage daughter, was diagnosed with clear cell sarcoma. Traveling to the top hospitals across the country, Woods and her family discovered how difficult it is to treat this disease, and even once it is successfully treated, it often returns. Clear cell sarcoma is most often found in teens and young adults in their 20s.

"We are honored to partner with Lennie and Sara's Cure to fight this disease and launch one of the very few trials for clear cell sarcoma, as part of our continued mission to provide hope for patients with hard-to-treat cancers," said **Sanjeev Luther**, President and CEO of Rafael Pharmaceuticals. "Both Sara's Cure and SARC share our passion in being a voice for those battling this rare disease."

"This study is an outstanding example of the value of collaborative efforts among advocacy, biotech and academia. Recognizing the need to better understand and treat clear cell sarcoma, SARC, as an academic research consortium will leverage the strengths of SARC to assist with this important trial," said **Steven Young**, President and CEO of Sarcoma Alliance for Research Collaboration. SARC has been instrumental in assisting with the study design, correlative studies, identifying key sites to run the trial as well as coordinating with Sara's Cure.

"Clear cell sarcoma most often affects young adults, but it is seen in all ages," said **Robin Jones**, M.D., of the Institute of Cancer Research and principal investigator on the trial. "The opening of this trial not only has the potential to provide a promising treatment option for those affected by this disease, but it sparks the hope that many of us have been waiting for, for a long time."

Being part of this trial gives me hope that we are truly turning a corner in how we treat this childhood-stealing disease," said **Matteo Trucco**, M.D., Director of the Children's Cancer Innovative Therapy Program of the Cleveland Clinic. "I'm honored to be part of this journey."

"Clear cell sarcoma is truly one of the most challenging sarcomas to treat," said **Rashmi Chugh**, M.D., medical oncologist and Professor of Internal Medicine at University of Michigan. "Our hope is that with the initiation of this important study, we will learn some critical lessons about how to successfully treat this aggressive disease."

The Company is deeply appreciative for the many principal investigators who will be part of this critical trial. In the United States, sites to open include Seattle Children's Hospital, Indiana University Melvin and Bren Simon Cancer Center, Cleveland Clinic Taussig Cancer Center and University of Michigan. Additional sites are also planned to be opened in the United Kingdom and Scotland.

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 patients' 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), Burkitt's and 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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