FDA Grants Rafael Pharmaceuticals Orphan Drug Designation for CPI-613® (Devimistat) for Treatment of Biliary Cancer

Company reaches important milestone to advance potential novel therapy for this subset of gastrointestinal cancers

CRANBURY, N.J., June 29, 2021 -- 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) granted orphan drug designation for CPI-613® (devimistat) for the treatment of biliary cancer.

Biliary cancer, which includes gallbladder cancer and bile duct cancer (which is also known as cholangiocarcinoma), is classified as a rare disease, affecting just 12,000 people in the United States each year, respectively. Biliary cancer appears when cancer cells form in the bile ducts or gallbladder, an essential aspect of the gastrointestinal system.

"Biliary cancer is often identified as an advanced stage cancer and considered aggressive with only modest response to existing treatment options for patients," said Vaibhav Sahai, MBBS, M.S., Associate Professor of Medical Oncology at The University of Michigan Medicine and the principal investigator on the Phase 1b/2 clinical trial of devimistat in combination with gemcitabine and cisplatin for patients with biliary cancer, announced last year. "The orphan drug designation for devimistat showcases the importance of discovering these new treatment options."

"Biliary cancer affects a small subset of cancer patients, but the unique complexities of the disease create an incredible need to investigate and identify effective treatments," said Sanjeev Luther, President and CEO of Rafael Pharmaceuticals. "Therapies developed for hard-to-treat cancers need to consider the unique and complex intricacies of each disease. Having effective treatment options available to patients significantly improves the outlook for patients diagnosed with a rare cancer."

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 lymphoma, peripheral T-cell lymphomas, and soft tissue sarcoma, and now, biliary cancer. With this, devimistat reaches 7 designations in total, making it one of the few compounds to have achieved this milestone. These designations support a future for therapies that focus on cancer metabolism.

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, Soft Tissue Sarcoma, Burkitt's lymphoma and biliary cancer. 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 lymphoma, peripheral T-cell lymphomas, soft tissue sarcoma, and biliary cancer. The Company's investors include Rafael Holdings, Inc. (NYSE: RFL). For more information, please visit www.rafaelpharma.com. On June 21, 2021, we announced that we have entered into a merger agreement with Rafael Holdings, Inc., to create a publicly-traded late-stage clinical oncology company focused on cancer metabolism-based therapeutics. For more information, visit www.rafaelholdings.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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