

TRIAL RESULTS – 3rd NOVEMBER 2021

[FAILED TRIAL: AVENGER 500 Ph 3 trial of CPI-613® \(devimistat\) + mFFX in 1L patients with mPDAC did not meet its primary endpoint of OS](#)

“These cancers are incredibly difficult to treat, with few to no effective treatments available, but Rafael took the risk because we will always fight for our patients,” said Sanjeev Luther, President and CEO of Rafael Pharmaceuticals. “While we are disappointed by the outcomes of these well-designed and well-executed studies, we remain committed to furthering our research and development in cancer metabolism for the treatment of hard-to-treat cancers, as our other studies continue. I personally want to express my heartfelt appreciation to the patients, their loved ones, the researchers and principal investigators for their trust and support. I am also incredibly grateful to my team, who works tirelessly for the patients we treat.”

[FAILED TRIAL: TRYbeCA-1 Ph 3 Trial of Eryaspase in Patients with Second-line Advanced Pancreatic Cancer did not meet primary endpoint of OS](#)

“While the results are disappointing, we congratulate the company for a very well managed trial in this difficult disease. With a median survival of 7.5 months, ERYTECH has created a new reference standard for clinical evaluation in second line pancreatic cancer”, said Prof. Pascal Hammel, MD, PhD, gastroenterologist-oncologist at Beaujon Hospital in Paris and co-principal investigator of the TRYbeCA-1 trial.

[SMR 2021: KEYTRUDA® \(pembrolizumab\) results from KEYNOTE-006 and KEYNOTE-716 trials to be presented](#)

“The seven-year data for KEYTRUDA in advanced melanoma is a significant milestone for the field of melanoma research and for patients,” said Dr. Roy Baynes, senior vice president and head of global clinical development, chief medical officer, Merck Research Laboratories. “Just ten years ago, the median survival for patients with metastatic melanoma was less than a year and only 10% of patients could expect to survive more than five years. We are incredibly honored to present these new long-term survival data in advanced disease at this year’s SMR 2021 Congress, as well as updated findings in stage IIB and stage IIC melanoma.”

[FAILED TRIAL: CANOPY-1 Ph 3 study did not meet its primary endpoints of OS and PFS in patients with 1L NSCLC](#)

“CANOPY-1 provides critical insights into the treatment of this devastating disease, and we will continue to analyze the data and conclusions, as well as their potential clinical implications,” said John Tsai, MD, Head of Global Drug Development and Chief Medical Officer, Novartis. “While this trial did not confirm the benefit for all patients we hoped for, we are energized by the overall CANOPY-1 findings as they support our commitment to continue studying canakinumab in lung cancer. We share our gratitude and thanks to the CANOPY-1 study patients and clinical investigators for their partnership.”

[Additional Verzenio® \(abemaciclib\) Ph 3 monarchE Trial Data Published in the Annals of Oncology](#)

- OS data from Verzenio® (abemaciclib) Phase 3 monarchE study were published in a Letter to the Editor in the Annals of Oncology.
- These OS data, while immature, have been published to address questions regarding the recent approval by the U.S. Food and Drug Administration (FDA) in a subgroup of the population studied in the monarchE trial. Patients participating in monarchE continue to be followed over time while overall survival data mature.
- Updated data from the Phase 3 monarchE study were recently disclosed in Annals of Oncology and presented at the ESMO Virtual Plenary.
- On October 12, the FDA approved Verzenio in combination with endocrine therapy (tamoxifen or an aromatase inhibitor) for the adjuvant treatment of adult patients with HR+, HER2-, node-positive, early breast cancer at high risk of recurrence and a Ki-67 score of $\geq 20\%$ as determined by an FDA-approved test. As previously reported, OS was a key secondary outcome measure for the monarchE study and an important component of the FDA review.
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[ASTRO 2021: New Outcomes Data Demonstrating Prolonged Survival for Inoperable Pancreatic Cancer Patients Presented](#)

"Pancreatic cancer is difficult to remove surgically because the tumors often entangle themselves into surrounding blood vessels and tissue in the abdomen, leaving a large number of patients inoperable and further contributing to the deadly nature of the disease," said Dr. Chuong. "We are thrilled to see such an improvement – with long-term survival more than doubling when MRIdian SMART was used to treat this population of patients. In fact, some patients were still alive several years later with excellent quality of life. These results are a significant improvement over historical outcomes from standard CT-guided radiation therapy."

[Initial Data Announced from the First Ph 1a Dose Escalation Trial of NX-2127 in Patients with Relapsed or Refractory B Cell Malignancies](#)

"The initial data from our study are the first proof-of-mechanism of targeted protein degradation in patients with hematologic malignancies," said Arthur T. Sands, M.D., Ph.D., president and chief executive officer of Nurix. "The concept of degrading BTK as a new therapeutic strategy in hematologic cancer has taken an important step forward, and the NX-2127 program has hit an exciting milestone in its clinical development."

[18.1 Month mOS for 41 Evaluable Elderly and Frail Patients With HNSCC in Ph 1 Expansion Evaluating NBTXR3 as a Single Agent Activated by Radiotherapy](#)

"I have held the belief that NBTXR3 could have a real impact for patients with solid tumors since reviewing the proof-of-concept data from the phase II/III in soft tissue sarcoma and throughout my participation in Study 102 Expansion," said study principal investigator Professor Christophe Le Tourneau, senior medical oncologist and head of the Department of Drug Development and Innovation (D3i) at Institut Curie. "This first look at survival data has added to my confidence that NBTXR3 could provide a promising new therapeutic option for the practice. I look forward to leading the upcoming phase III global registration study, and to have the opportunity to evaluate the promise of this innovation in a larger patient population."

[Imfinzi plus chemotherapy significantly improved OS in 1L advanced biliary tract cancer in TOPAZ-1 Ph 3 trial at interim analysis](#)

Do-Youn Oh, MD, PhD, Professor, Division of Medical Oncology, Department of Internal Medicine at Seoul National University Hospital and Seoul National University College of Medicine, and principal investigator in the TOPAZ-1 Phase III trial, said: “Patients with advanced biliary tract cancer are in dire need of new treatments as progress in the 1st-line setting has remained largely stagnant for more than 10 years. TOPAZ-1 is the first Phase III trial to show that adding an immunotherapy to standard chemotherapy delivers a meaningful overall survival benefit for patients in this setting. Today’s exciting results are a major step forward in treating this disease and represent new hope for our patients.”