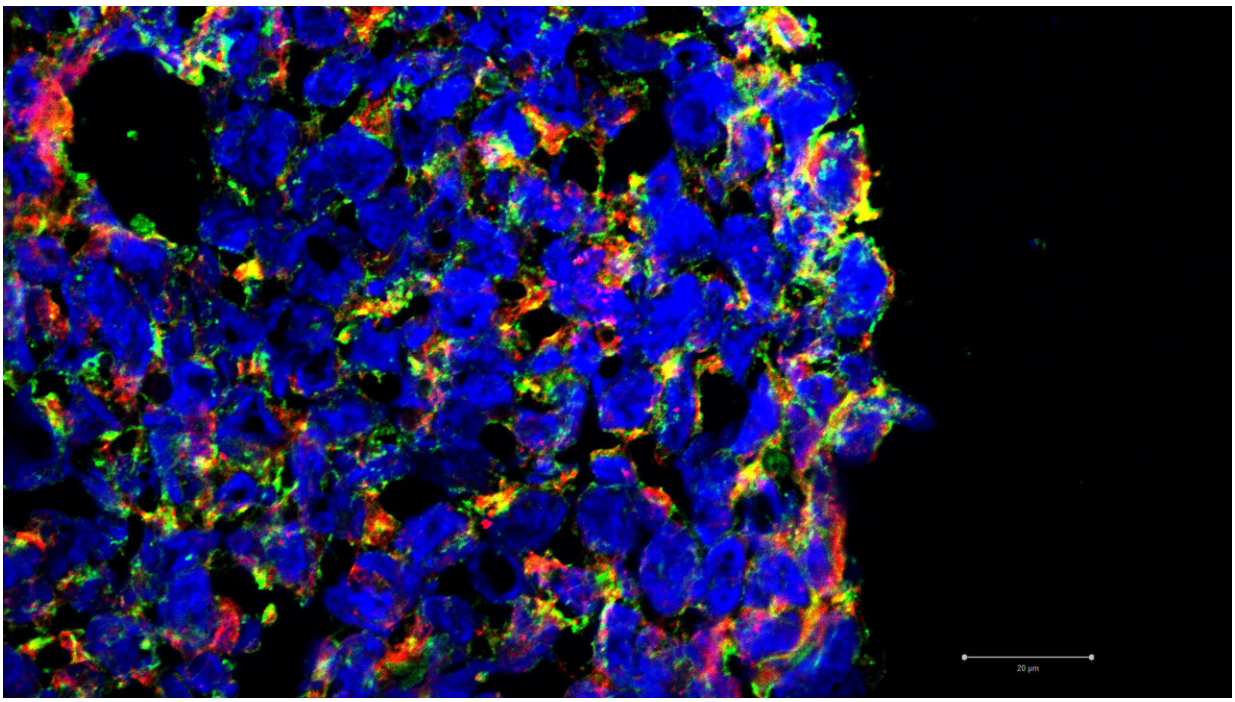
Metallodrug BOLD-100 receives FDA Orphan Drug Designation for treatment of gastric cancer

July 21 2021



In a 3-D colorectal cancer cell culture model, the active substance KP-1339 (IT-139) triggers characteristics that cause immunogenic cell death in the organism. Credit: Universität Wien

The ruthenium-based anticancer agent BOLD-100 just received an Orphan Drug Designation in the treatment of gastric (stomach) cancer

from the U.S. Food and Drug Administration. The small molecule therapeutic originally developed as KP1019 (KP1339) by chemist and physician Bernhard Keppler from the University of Vienna, showed promising efficacy and tolerability in clinical trials conducted so far. The current designation adds to the existing Orphan Drug Designation of BOLD-100 in pancreatic cancer.

The effects of the metallodrug result in cell death "in both sensitive and resistant cancers, giving BOLD-100 the potential to significantly improve outcomes in a wide range of both solid and liquid tumors in combination with other anti-cancer therapies ranging from traditional chemotherapies to targeted therapies," states Bold Therapeutics in a news release. The Vancouver-based clinical-stage biopharmaceutical company develops and commercializes BOLD-100 in close cooperation with the University of Vienna.

The Orphan Drug Act provides for granting special status to a therapeutic agent in order to treat a rare disease. While gastric cancer is highly prevalent in Asia, it is considered an orphan disease in North America and Europe, with approximately 27,000 new cases diagnosed in the U.S. annually. According to Bold Therapeutics, data submitted in the orphan drug application demonstrated that BOLD-100 is potentially effective against gastric cancer.

From the lab into the clinic

"Even if you find a compound that works in cell culture, statistically there is only a 1 in 10,000 chance that it will become something clinically applied. Therefore, we are very pleased to see that the decisively co-developed anticancer agent BOLD-100 delivers such promising results in several in vitro and in vivo tumor models as well as in early clinical trials, and that it now received the 2nd orphan drug designation," says Bernhard Keppler, Dean Faculty of Chemistry and

Head of Research Cluster "Translational Cancer Therapy Research" at the University of Vienna.

BOLD-100 is a small molecule, which is transported into the tumor cell via the protein albumin and selectively activated there, resulting in the death of the cancer cells. Bold Therapeutics continues to actively enroll patients in its Phase 1b trial of BOLD-100 in combination with FOLFOX (5-fluorouracil, leucovorin, oxaliplatin) for the treatment of patients with advanced gastrointestinal cancers at six sites in Canada. The researchers expect a transition into a Phase 2 trial at the end of the year, with additional clinical sites in both the U.S. and South Korea.

Provided by University of Vienna