Drug candidate granted FDA orphan drug status for pancreatic cancer

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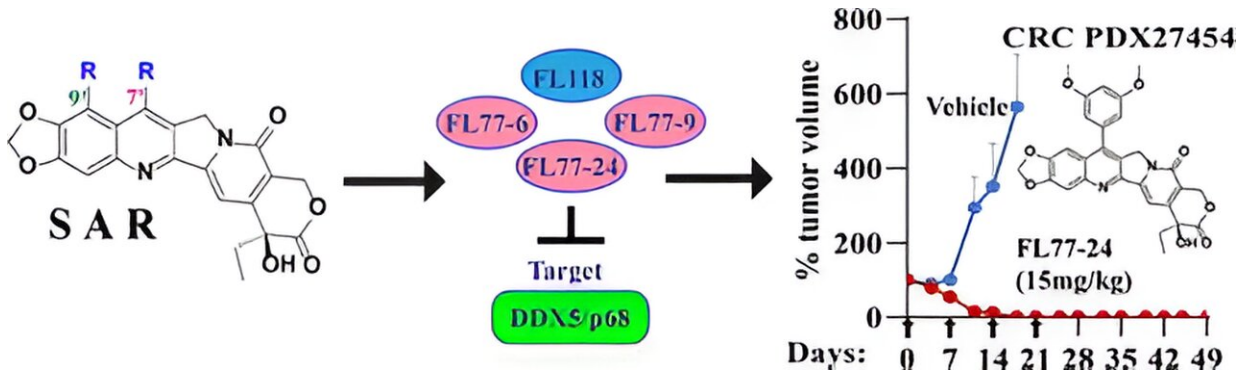

Credit: *Journal of Medicinal Chemistry* (2023). DOI: 10.1021/acs.jmedchem.3c01589

The U.S. Food and Drug Administration (FDA) has awarded Orphan Drug Designation to Canget BioTekpharma LLC for FL118, a drug candidate developed at Roswell Park Comprehensive Cancer Center, as a possible treatment for pancreatic cancer.

FL118, also known by its chemical name, 10,11-methylenedioxy-20(S)-camptothecin (10,11-MD-CPT), is one of only 206 pharmaceutical agents granted Orphan Drug Designation for the treatment of pancreatic cancer since the FDA initiated the incentive program in 1984.

Awarded to encourage development and evaluation of new treatments for rare diseases, orphan drug designation helps to speed and support research by providing investigators with incentives such as tax credits toward clinical trials, exclusive marketing rights and exemptions from user fees.

Because they affect fewer people, rare diseases like pancreatic cancer typically are associated with limited treatment options, with fewer drugs in development compared to more common diseases.

While pancreatic cancer accounts for only about 3% of all cancers in the U.S. today, it has been projected to become the second most common cause of cancer-related U.S. deaths before 2030.

Discovered by a team of scientists led by Fengzhi Li, Ph.D., Associate Professor of Oncology in the Department of Pharmacology and Therapeutics at Roswell Park, FL118 has been shown in preclinical studies to eliminate both pancreatic and colorectal tumor cells by binding to DDX5, a powerful cancer-causing protein. It's a small molecule derived from camptothecin, a component found in the bark and stem of a tree native to China—and used as a traditional Chinese remedy for centuries.

Research recently published by Dr. Li and colleagues in the *Journal of Medicinal Chemistry* documents the synthesis, identification and characterization of new analogs of FL118 shown to have enhanced comparative antitumor activity.

More information: Wenchao Wang et al, Structure–Activity Relationship of FL118 Platform Position 7 Versus Position 9-Derived Compounds and Their Mechanism of Action and Antitumor Activity, *Journal of Medicinal Chemistry* (2023). DOI: 10.1021/acs.jmedchem.3c01589