

TGen, Scottsdale Healthcare begin study of new drug for patients with solid tumors

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The Virginia G. Piper Cancer Center at Scottsdale Healthcare and the Translational Genomics Research Institute (TGen) are studying the safety and effectiveness of a new drug, AG-120, for treatment of patients with solid tumors, especially those with brain tumors and gallbladder bile duct cancer.

"AG-120 is designed specifically for those patients who carry the IDH1 gene mutation," said Dr. Daniel D. Von Hoff, Distinguished Professor and Physician-In-Chief at TGen, and Chief Scientific Officer for the Virginia G. Piper Cancer Center Clinical Trials at Scottsdale Healthcare, a partnership between Scottsdale Healthcare and TGen that delivers new treatments to cancer patients based on precision medicine.

The IDH1 gene mutation is most commonly found in gliomas, which make up the largest group of "primary" brain tumors, those that start in the brain. Gliomas include all tumors arising from the gluey or supportive tissue of the brain. They represent about 30 percent of all brain tumors, and about 80 percent of all malignant brain tumors.

As with all brain cancers, gliomas are difficult to treat and many grow back after surgery, radiation and standard of care chemotherapy. Many drugs cannot get to the brain because of a filtering mechanism in the body called the blood-brain barrier.

"There is a great need for more effective treatments for patients with gliomas and other <u>solid tumors</u>, such as gallbladder <u>bile duct cancer</u>,"



said Dr. Von Hoff, who is the Principal Investigator for AG-120 clinical trial. This study will enroll as many as 50 patients. Researchers will use precision medicine to match treatments to patient's specific genomic, or molecular, makeups.

Mutations in isocitrate dehydrogenase (IDH) 1 and 2, originally discovered in 2008, occur in the vast majority of low-grade gliomas and secondary high-grade gliomas. IDH mutations are oncogenic, meaning they have the potential to cause cancer. These mutations occur early in the formation of gliomas and in gallbladder bile duct cancer.

AG-120 is produced by Agios Pharmaceuticals Inc., based in Cambridge, Mass.

IDH1 is a metabolic enzyme identified by Agios as a protein that is mutated in a wide range of malignant tumors. Agios and its collaborators recently demonstrated that IDH1 mutations initiate and drive cancer growth by blocking differentiation, or maturation, of primitive cells. According to Agios, the inhibition of these mutated proteins may lead to clinical benefit for those <u>cancer</u> patients whose tumors carry them.

Provided by The Translational Genomics Research Institute

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