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Trial shows AZEDRA can be effective, safe for treatment of rare neuroendocrine tumors

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A radiotherapy drug that treats the rare neuroendocrine cancers pheochromocytoma and paraganglioma can be both effective and safe for patients, according to the findings of a multi-center trial led by researchers in the Abramson Cancer Center of the University of Pennsylvania. The study showed AZEDRA (ultratrace iobenguane I131) led to a significant reduction in the cardiovascular side effects that are associated with these cancers while also stopping tumor growth. The drug is designed to treat malignant, recurrent, or unresectable forms of the cancers—cases for which there are currently no approved non-surgical treatments. Progenics Pharmaceuticals, which manufactures AZEDRA, recently submitted the findings of this trial as part of an application for approval to the U.S. Food and Drug Administration. The study's principal investigator Daniel A. Pryma, MD, an associate professor of Radiology and Radiation Oncology, and Chief of Nuclear Medicine and Clinical Molecular Imaging at Penn's Perelman School of Medicine, will present the results at the American Society of Clinical Oncology 2018 Annual Meeting in Chicago as an oral abstract (Abstract #4005).

"This represents real hope for patients, since as of right now, there are no anti-tumor therapies available for patients with these tumors who are not candidates for surgery," Pryma said.

Pheochromocytoma and paraganglioma are neuroendocrine tumors that form from the same type of tissue. Pheochromocytoma forms in the adrenal gland, while paraganglioma forms outside of the gland. There are

an estimated 650 to 2600 new cases in the United States each year, with between 10 and 35 percent of cases metastatic or locally invasive at diagnosis. In addition, when the disease returns, it may not be resectable surgically. The five-year survival rate of unresectable cases can be as low as 12 percent.

AZEDRA is a radiotherapy drug that attacks these tumors with a high, specifically-targeted dose. The FDA gave it an Orphan Drug designation, Fast Track status, and Breakthrough Therapy designation in the U.S.

In the Penn-led trial, 68 patients received at least one therapeutic dose of AZEDRA. Twenty-five percent of patients who received at least one dose met the trial clinical benefit endpoint, and the number jumped to 32 percent in patients who received two doses. That clinical benefit was measured by a 50 percent or greater reduction in the amount of hypertensive medications these patients took lasting at least 6 months as high blood pressure and associated cardiovascular side effects are a major cause of harm from these cancers. Additionally, 92 percent of evaluable patients who received at least one dose achieved a partial response or stable disease.

"Our data shows this therapy provides a dual benefit to patients by not only controlling the tumor, but also the debilitating symptoms caused by their excess hormone production," Pryma said.

The drug was generally well-tolerated, with the most common side effects being consistent with what a patient would experience with radiation such as decreased blood counts, fatigue, nausea, and dizziness.

Provided by Perelman School of Medicine at the University of Pennsylvania