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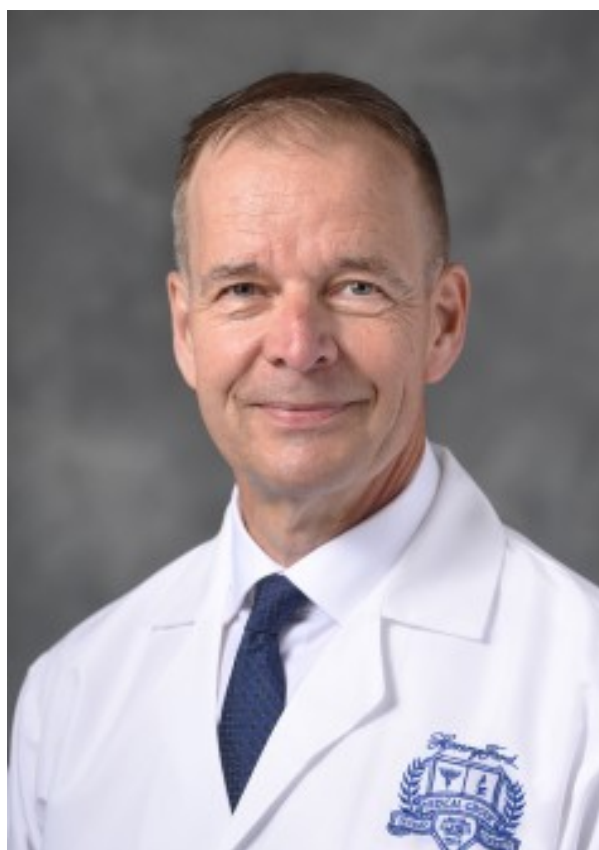


Henry Ford Cancer Institute is First in the World to Activate Two New Treatments in GBM AGILE Trial for Glioblastoma Brain Cancer

Newswise — DETROIT (Feb. 3, 2021) – [Henry Ford Cancer Institute](#) is the first site in the world to activate two new treatments for glioblastoma (GBM), the deadliest form of brain cancer, as part of a patient-centered adaptive platform trial known as [GBM AGILE](#) (Glioblastoma Adaptive Global Innovative Learning Environment). Led by [Global Coalition for Adaptive Research \(GCAR\)](#), GBM AGILE tests multiple therapies for patients with newly-diagnosed and recurrent GBM.

Henry Ford Cancer Institute was first-in-the-world to enroll a patient in GBM AGILE when enrollment began in 2019.

“We are excited for this major step forward in the GBM AGILE trial, and especially for the hope it provides those battling glioblastoma brain cancer,” said [Tom Mikkelsen, M.D.](#), principal investigator for GBM AGILE at Henry Ford Cancer Institute, and medical director of the [Precision Medicine Program](#) and [Clinical Trials Office](#) at Henry Ford Health System. “Through global collaboration, GBM AGILE is making it possible for some of the world’s foremost experts in glioblastoma research and treatment to collaborate and advance the pace at which scientific and clinical breakthroughs can be achieved.”



After opening at Henry Ford Cancer Institute, the two new interventions –VAL-083 from Kintara Therapeutics, Inc. and *paxalisib* from Kazia Therapeutics Limited– will subsequently open at more than 35 trial sites across the United States, with additional global sites in Canada, Europe and China to follow. VAL-083 is being evaluated in all three glioblastoma

patient subtypes: newly-diagnosed methylated MGMT; newly-diagnosed unmethylated MGMT; and recurrent. *Paxalisib* is being evaluated in newly-diagnosed unmethylated and recurrent glioblastoma.

VAL-083 is a "first-in-class" small molecule that has been studied in more than 40 Phase I and Phase II clinical trials in multiple indications sponsored by the National Cancer Institute. VAL-083 is independent of the MGMT resistance mechanism and has been granted Orphan Drug Designation for glioblastoma by the FDA and for Glioma by the European Medicines Agency. In addition, the FDA granted Fast Track Designation for VAL-083 in recurrent glioblastoma.

Paxalisib is a small molecule inhibitor of the PI3K / AKT / mTOR pathway. The PI3K pathway appears to be disordered in more than 85% of cases of glioblastoma, making this pathway a high-potential target for new glioblastoma therapies. *Paxalisib* is a potent inhibitor of the PI3K pathway, and has been shown to have an anti-tumor effect in animal models of glioblastoma. *Paxalisib* was granted Orphan Drug Designation for glioblastoma by the FDA in February 2018, and Fast Track Designation for glioblastoma by the FDA in August 2020.

GBM AGILE is an international, innovative platform trial designed to more rapidly identify and confirm effective therapies for patients with glioblastoma through response adaptive randomization and a seamless phase II/III design. The trial, conceived by over 130 key opinion leaders, is conducted under a master protocol, allowing multiple therapies or combinations of therapies from different pharmaceutical partners to be evaluated simultaneously. With its innovative design and efficient operational infrastructure, data from GBM AGILE can be used as the foundation for a new drug application and biologics license application submissions and registrations to the FDA and other health authorities.

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About [Global Coalition for Adaptive Research \(GCAR\)](#)

The Global Coalition for Adaptive Research (GCAR) is a 501(c)(3) nonprofit organization uniting physicians, clinical researchers, advocacy and philanthropic organizations, biopharma, health authorities, and other key stakeholders in healthcare to expedite the discovery and development of treatments for patients with rare and deadly diseases by serving as sponsor of innovative and complex trials including master protocols and platform trials. GCAR is the sponsor of GBM AGILE, an adaptive platform trial for patients with GBM – the most common and deadliest of malignant primary brain tumors. Key strategic partners for the GBM AGILE trial effort include the [National Brain Tumor Society](#), [National Foundation for Cancer Research](#), and [Asian Fund for Cancer Research](#), three nonprofit organizations that are working together to provide philanthropic support as well as assistance in communicating with patients and families and inviting all others to join in supporting this innovating approach to brain tumor treatment development.

To learn more about GCAR, visit www.gcaresearch.org and follow us: @GCARResearch and www.facebook.com/GCARResearch.

About [Henry Ford Cancer Institute](#):

The Henry Ford Cancer Institute is one of the largest cancer programs in Michigan, providing care at five hospitals, 11 outpatient facilities and hundreds of aligned doctor's offices

throughout southeast and southcentral Michigan. Cancer experts at Henry Ford communicate seamlessly across the institute's multiple cancer treatment locations, offering patients access to the most advanced treatment options and expertise, close to home. Treatment for the most complex or rare cancers and the Institute's extensive cancer research program is anchored at its new 187,000-square-foot cancer facility in Detroit, which is connected to Henry Ford Hospital. For more information, visit henryford.com/cancer.