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A Groundbreaking Global Alliance Forms to Find Effective Response to the Deadliest Human Brain Tumor

GBM AGILE (An Adaptive, Global, Innovative Learning Environment) to Implement Unprecedented International Clinical Trial

WASHINGTON, D.C. – This year approximately 12,000 individuals in the US and tens of thousands more around the globe, including 35,000 in China alone, will receive a diagnosis of glioblastoma multiforme (GBM) from their doctors. GBM is the most common adult brain tumor and it is highly aggressive. In fact, fifty percent of GBM patients will survive for a year or less. Five-year survival for GBM is less than two percent – and, unfortunately, these dismal statistics have not changed for decades.

A broad coalition of GBM neuro-surgeons, neuro-oncologists, basic and clinical investigators, and representatives from the GBM advocacy communities shared the conviction that this situation was unacceptable—that new approaches must be pursued that can identify successful therapies. "It is in that spirit, that this broad 'coalition of the willing' today announce the design and plan for a new-generation clinical trial for GBM," said Dr. Anna Barker, GBM AGILE Project Director and

Executive Committee (EC) Chair, Director of the National Biomarker Development Alliance (NBDA) and Professor at Arizona State University's School of Life Sciences. "This new generation of clinical trials will be adaptive based on learning from the patients; global as it is to be performed across the U.S., China, Australia and Europe; and innovative in that it is driven by Bayesian statistics and molecular markers. The nature of this trial is that it will be a 'learning environment' that allows the response of each patient to inform the ongoing conduct of the trial," said Dr. Web Cavenee, GBM AGILE Co-investigator; EC Member; and Director, Strategic Alliances CNS, Ludwig Institute for Cancer Research and University of California San Diego. Although literally hundreds of clinical trials have tested numerous therapies for GBM, treatment options and patient outcomes have not changed for several decades. Notably, the single advance in the treatment of GBM occurred over a decade ago when a drug called temozolomide was tested with radiation therapy in a phase 3 trial and reported to extend life by about two months. In addition to the dismal record of clinical trials for GBM, there are virtually no biomarkers that can be employed to drive drug development and guide treatment. As a result, GBM patients have not benefited from the advances known as "precision medicine."

"We have to do something more – something different – something that brings the best science and innovative clinical trials together to identify therapies that work," said Dr. Mitchell Berger, GBM AGILE Co-investigator; EC Member; and Chief of Neurosurgery at the University of California at San Francisco. "GBM AGILE is the best path to achieve those goals than I have seen for decades."

"Since GBM AGILE will be performed on a global basis, we will finally be able to benefit from the convergence of the basic and clinical research that is driving our progress in neuro-oncology in Australia and across the globe. Moreover, this collaboration will enable recruiting sufficient numbers of patients to learn through the adaptive trial, which therapies do or do not work for GBM. Australia is actively involved in the planning and design of GBM AGILE, and we are excited to be able to contribute to its implementation," said Dr. Mustafa Khasraw, GBM AGILE EC Australian Liaison and Medical Oncologist, University of Sydney.

This "perfect storm" of factors led the GBM AGILE Global team to reach beyond classic clinical trials strategies to create a potentially paradigm shifting "next generation platform adaptive trial" for GBM. "GBM AGILE will employ advanced statistical tools to ensure that better treatments can be assigned to more patients and ineffective treatments can be eliminated from the trial," said Dr. Donald Berry, GBM AGILE co-principal investigator, EC Member and Professor of Biostatistics at The University of Texas MD Anderson Cancer Center.

GBM AGILE will be performed using a "master protocol." This innovative approach, developed by the U.S. Food and Drug Administration (FDA), will enable centralizing a number of functions for the trial and simplify the efforts needed to add new therapies to the ongoing trial process. Molecular biomarkers will be employed to assign specific patients to matching therapies (arms) of the trial. As observed in other types of cancers, GBM patients are likely to benefit most by receiving therapies which may be effective only in subsets of patients with a specific molecular alteration. This molecularly targeted approach is the basis for "precision medicine."

"Thanks to the deep molecular characterization of GBM, we are beginning to get a better picture of the genes and pathways that are altered in GBM, so there is finally an opportunity to identify real biomarkers and conduct a "smart" trial like GBM AGILE," said Dr. Alfred Yung, GBM AGILE EC

Member; Chairman and Professor, Department of Neuro-Oncology; Margaret and Ben Love Chair in Clinical Cancer Care, MD Anderson.

During the planning and design phase, GBM AGILE is "crowdsourcing" knowledge from a large number of international leaders in GBM basic and clinical research and using that knowledge to inform the design of a new-generation adaptive trial that will learn from every patient that enters the trial.

"The Chinese neurosurgery communities look forward to implementing GBM AGILE in China and to working with the GBM Global team," said Dr. Tao Jiang, GBM AGILE EC China Liaison, Vice Director of Beijing Neurosurgical Institute and Director and Founder of Chinese Glioma Genome Atlas (CGGA). "We finally have the opportunity to work together across borders to learn from every patient and identify better therapies for GBM patients no matter where they are on the Globe."

"As a neuro-oncologist taking care of GBM patients every day, GBM AGILE is groundbreaking in so many ways. The adaptive design will allow us to modify the trial as it proceeds based on the data collected – and to test many drugs and combinations versus single agents – and to do it faster," said Dr. Timothy Cloughesy, GBM AGILE Principal Investigator, EC Member; and Director, UCLA Neuro-Oncology Program. "It's an opportunity for patients to benefit from precision medicine, and a real source of hope for patients and their families."

The work to design and implement GBM AGILE is well underway. Currently, over 100 neurosurgeons, neuro-oncologists, pathologists, imagers, neuroscientists and patient advocates comprise 10 major committees that donate their time and pay their own travel costs to participate in planning and designing GBM AGILE. Despite time zone differences, the groups meet regularly by phone (and in person) to tackle the challenges related to everything from selecting drugs and biomarkers for the trial to design issues and numerous other challenges. A number of GBM patient advocacy groups have joined the GBM AGILE team, including the U.S.-based National Brain Tumor Society, Accelerate Brain Cancer Cure, National Foundation for Cancer Research and the Australian Cure Brain Cancer Foundation.

"I know I speak for all of the patient advocacy groups when I say that the commitment we have seen from the international GBM research communities to unite different disciplines and break down barriers for the benefit of GBM patients is inspiring to all of us," said Dr. Sujuan Ba, GBM AGILE EC Member and President of National Foundation for Cancer Research.

The GBM AGILE global team has two ambitious goals: to begin enrolling patients by mid-year in 2016; and even more audacious, to raise funds for GBM AGILE from philanthropists, research foundations and crowd funding. Several organizations are supporting the planning and design phase for GBM AGILE, including the: National Biomarker Development Alliance (Arizona State University), Cure Brain Cancer Foundation, National Foundation for Cancer Research, ASU Foundation and the GBM AGILE global team.

"None of us are willing to continue to tolerate the tragic and costly loss of life inflicted on patients who are stricken with GBM," said Dr. Barker. "GBM AGILE is truly a 'global coalition,' and it's always humbling to see the power of a group this committed to changing the world."

About GBM AGILE and the National Biomarker Development Alliance (NBDA)

GBM AGILE is being developed through the National Biomarker Development Alliance (NBDA), a non-profit organization created as part of the Research Collaboratory at Arizona State University (ASU). The NBDA's mission is to collaboratively create standards-based, end-to-end systems solutions for biomarker discovery, development and delivery to advance precision medicine. The NBDA operates through trans-disciplinary and trans-sector networks to develop new research networks and consortia that focus on "demonstration projects" that advance all aspects of biomarker research and applications – especially developing smarter and more efficient clinical trials. Although NBDA is generally disease agnostic, rare diseases like GBM are a focus for the Alliance since in most cases few if any biomarkers exist and most clinical trials fail. Overall, the NBDA is dedicated to moving high quality, effective biomarkers through all phases of discovery, development and validation through more innovative and efficient clinical trials to ensure that the promise of precision (molecularly based) medicine will be available to all patients.