

## IDEAYA Biosciences and Boston Children's Hospital Collaborate on Preclinical Evaluation of IDE196 for Sturge Weber Syndrome - a Rare Disease Associated with Genetic Mutation of *GNAQ*

SOUTH SAN FRANCISCO, Calif., Jan. 10, 2020 /[PRNewswire](#)/ -- IDEAYA Biosciences, Inc.

(NASDAQ:IDYA), an oncology-focused precision medicine company committed to the discovery and development of targeted therapeutics, announced that the company has entered into a Sponsored Research Agreement with Boston Children's Hospital for preclinical evaluation of the role of protein kinase C (PKC) in Sturge Weber syndrome (SWS), a rare neurocutaneous disorder characterized by capillary malformations and associated with mutations in *GNAQ*.

Under the agreement, IDEAYA will collaborate with and support research at Boston Children's Hospital in the laboratory of Dr. Joyce Bischoff, Ph.D., Research Associate, Department of Surgery and Professor, Harvard Medical School, who is Principal Investigator of the research studies. The preclinical research will evaluate IDE196, a potent, selective PKC inhibitor, *in vitro* – to assess whether pharmacological inhibition of PKC in endothelial cells having *GNAQ* mutations will restore normal cell function, as well as *in vivo* – to assess whether pharmacological inhibition of PKC can regulate blood vessel size in murine models that recapitulate enlarged vessels seen in SWS capillary malformations.

SWS is a rare disease characterized by a facial birthmark, neurological abnormalities (e.g. seizures) and glaucoma, which occurs in 1 to 20,000 to 50,000 live births. The disease is believed to be mediated by a somatic *GNAQ* mutation in skin or brain tissue which enhances signaling in the PKC pathway in a reported 88% (n=26) of SWS patients. (NEJM Shirley et al., May 2019). "SWS is a rare disease that can present debilitating symptoms for patients, such as choroidal hemangiomas which may lead to glaucoma. There are no current FDA approved treatments specifically developed for SWS – highlighting the high unmet medical need for these patients," noted Dr. Bischoff, Ph.D.

IDE196 is a potent, selective, small molecule inhibitor of protein kinase C (PKC), which IDEAYA is evaluating in a Phase 1/2 basket trial in patients with Metastatic Uveal Melanoma or other solid tumors, such as cutaneous melanoma, having *GNAQ* or *GNA11* hotspot mutations which enhance signaling in the PKC

pathway. "We are excited to work with Boston Children's Hospital to evaluate IDE196 activity in preclinical models relevant to Sturge Weber, a rare disease believed to be driven by genetic mutation of *GNAQ*. This important work is part of our broader strategy to deliver precision medicine therapies for patients with *GNAQ* or *GNA11* mutations, by targeting the underlying biology of the disease," said Yujiro S. Hata, Chief Executive Officer and President at IDEAYA Biosciences.

### **About IDEAYA Biosciences**

IDEAYA is an oncology-focused precision medicine company committed to the discovery and development of targeted therapeutics for patient populations selected using molecular diagnostics. IDEAYA's approach integrates capabilities in identifying and validating translational biomarkers with small molecule drug discovery to select patient populations most likely to benefit from the targeted therapies IDEAYA is developing. IDEAYA is applying these capabilities across multiple classes of precision medicine, including direct targeting of oncogenic pathways and synthetic lethality – which represents an emerging class of precision medicine targets.

### **Forward-Looking Statements**

This press release contains forward-looking statements, including, but not limited to, statements related to IDE196 activity in preclinical models relevant to Sturge Weber and IDEAYA's ability to deliver precision medicine therapies. Such forward-looking statements involve substantial risks and uncertainties that could cause IDEAYA's preclinical and clinical development programs, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the uncertainties inherent in the drug development process, including IDEAYA's programs' early stage of development, the process of designing and conducting preclinical and clinical trials, the regulatory approval processes, the timing of regulatory filings, the challenges associated with manufacturing drug products, IDEAYA's ability to successfully establish, protect and defend its intellectual property and other matters that could affect the sufficiency of existing cash to fund operations. IDEAYA undertakes no obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of IDEAYA in general, see IDEAYA's recent Quarterly Report on Form 10-Q filed on November 13, 2019 and any current and periodic

reports filed with the U.S. Securities and Exchange Commission.

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For further information: IDEAYA Biosciences, Inc., Paul Stone, Chief Financial Officer,  
pstone@ideayabio.com

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