

# BioAxone BioSciences Inc. Announces NIH/SBIR Fast Track Grant to Develop BA-1049 for Treatment of Cerebral Cavernous Malformation/Angioma

*Fifth SBIR Grant Received Since 2014 for a Total of \$4.3 Million in Funding*



CAMBRIDGE, MA -- (Marketwired) -- 02/08/16 - [BioAxone BioSciences Inc.](#) announced today that the Company was awarded a Fast-Track grant



from the NIH Small Business and Innovation Research Program (SBIR) and the National Institute of Neurological Disorders and Stroke (NINDS). The \$3.2 million Phase I/Phase II grant will support development of BioAxone's small molecule kinase inhibitor, BA-1049, for treatment of cerebral cavernous malformations (CCM). Under this grant, BioAxone will collaborate with a consortium of CCM experts from the University of Chicago, Duke University and the Los Angeles Biomedical Research Institute at Harbor-UCLA Medical Center (LA BioMed).

CCM (also known as cavernous angioma) is a genetic disorder caused by loss of function in one of three CCM genes, resulting in over-activation of Rho kinase in cerebral endothelial cells. The CCM mutation leads to benign endothelial cell tumors that cause headache, seizures and neurological deficits such as paralysis. The tumors may bleed, requiring emergency medical intervention. Cavernous angiomas are estimated to occur in approximately 0.2% of the general population. This includes the incidence of sporadic forms, which may be up to 70% of diagnosed cases. Early-stage sporadic lesions can be asymptomatic and remain undetected until they grow or bleed, and multiple lesions occur in people with the familial disease. Individuals often experience their first symptoms in their 20s or 30s. There is no current treatment to stop progression except surgical removal.

Dr. Lisa McKerracher, CEO of BioAxone, stated, "We are delighted that this grant has allowed us to form a strong consortium of industry and academic partners to develop the first effective drug to treat this disorder, which is devastating for patients and their families. The Angioma Alliance was instrumental in helping us form the consortium."

The project funds a consortium of collaborators including Dr. Issam Awad (University of Chicago), Dr. Douglas Marchuk (Duke University), and Dr. Rebecca Stockton (LA BioMed and the David Geffen School of Medicine at UCLA). They are all experts in diverse aspects of CCM, ranging from the genetic basis of disease to neurosurgical treatment. The academic and medical collaborators will complete proof of concept studies to determine efficacy of BA-1049 with human vascular tissue and transgenic mice expressing defective copies of the CCM gene. BioAxone will coordinate the collaboration, complete dose-ranging studies in mice, and carry out good-laboratory practice (GLP) safety studies to support an IND application to the FDA.

Dr. Amy Akers, Chief Scientific Officer of Angioma Alliance, remarked, "Angioma Alliance and the CCM patient community is committed to facilitating novel scientific collaborations and driving research for a cure. We are thrilled by

the possibility of a non-surgical treatment of this debilitating illness. We look forward to working with BioAxone to bring BA-1049 to clinical trials."

This grant is the fifth SBIR grant the company has received since 2014, allowing the company to develop a strong and diverse pipeline of products that target neurodegeneration and neurovascular disease. BioAxone has received a total of \$4.3 million from SBIR grants.

*About BioAxone BioSciences, Inc.*

BioAxone is a clinical-stage biopharmaceutical company developing a pipeline of regenerative neuroscience drugs based on a deep understanding of axon regeneration and neuronal signaling pathways. The company's lead drug for spinal cord injuries, VX-210 (formerly Cethrin□), was licensed to Vertex Pharmaceuticals and is expected to enter a Phase IIb/III clinical study in the first half of 2016. BioAxone's research programs include NIH-funded work to develop additional mono- and combination therapies for neurotrauma, neurovascular disorders, and glaucoma. BioAxone is based in Cambridge, Massachusetts. For more information please visit [www.bioaxonebio.com](http://www.bioaxonebio.com).

Michelle Linn

774-696-3803

[michelle@linndencom.com](mailto:michelle@linndencom.com)

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