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NEWLY PUBLISHED DATA SHOW BISCAYNE PHARMACEUTICALS' NOVEL ANTIEPILEPTIC DRUG CAN ELIMINATE SEIZURES IN MODELS OF DRAVET SYNDROME

--BIS-001 Completely Eliminated Seizures in Majority of Animals at Doses Expected to be Achievable and Well-Tolerated in Patients—
--With New BIS-001 Extended Release Formulation Almost Complete, Phase 1b Trial on Track to Start in Mid-2017--

Miami Beach, FL– October 24, 2016 – Biscayne Pharmaceuticals, Inc., today reported that a new scientific publication[1] shows that lead compound BIS-001 can eliminate all seizures in the majority of animals in models of the catastrophic type of epilepsy known as Dravet syndrome. BIS-001 is a clinical-stage, highly potent agent with a novel mechanism of action that is a synthetic form of huperzine A, an extract of a traditional Chinese medicine. Biscayne is initially developing BIS-001 for the treatment of children with Dravet syndrome and for adults with complex partial seizures.

Dravet syndrome is a catastrophic, genetically-mediated early life seizure disorder associated with prolonged, recurrent refractory seizures; cognitive and behavioral deficits and a 15–20% mortality rate. The SCN1A sodium channel mutations implicated in Dravet syndrome are also associated with other serious inherited epilepsy conditions that affect both children and adults. Available antiepileptic drugs often fail to protect against the severe seizures and cognitive deficits in these patients, and new treatments are urgently needed.

The new studies assessed BIS-001 in mice who have the same SCN1A mutations seen in human Dravet syndrome and are subject to similar severe seizures. The studies were conducted in the Emory University laboratory of Dr. Andrew Escayg, who is a leading expert in the human and mouse genetics of neurological diseases. In these studies, BIS-001 completely eliminated seizures in the majority of treated animals at doses expected to be well-tolerated and achievable in patients with a new extended release formulation of BIS-001. Importantly, the effects of BIS-001 were sustained over time, with high levels of anti-seizure activity maintained during repeat dosing for up to 21 days, with no evidence of the tachyphylaxis, or diminishing efficacy, that can be seen with other antiepileptic drugs. BIS-001 appeared to be well-tolerated in these animals.

"BIS-001 demonstrated a high level of efficacy in these models of extremely hard-to-treat epilepsy, eliminating all seizures in most animals and maintaining efficacy over repeat administration, at dose levels we are optimistic will be effective and well-tolerated in patients with our new extended release formulation," said Stephen Collins, MD, PhD, President and CEO of Biscayne Pharmaceuticals. "We are encouraged by these promising results in a model of devastating childhood epilepsy as we prepare to initiate a Phase 1b clinical trial of BIS-001 in another population with difficult-to-treat seizures--adults with refractory complex partial epilepsy."

Dr. Collins continued, "The active ingredient of BIS-001 is huperzine A, an acetylcholinesterase (AChE) inhibitor that has a long history of safe use in Chinese medicine. It also has exhibited the cognition-enhancing properties seen with some other AChE drugs, but has demonstrated much better central nervous system and systemic tolerability and safety than currently available agents. We will be assessing whether BIS-001 supports improved cognition in epilepsy patients, and at the least, we expect it to be devoid of the detrimental effects on cognition seen with many existing antiepileptic drugs. We are eager to test BIS-001 in a variety of conditions, given the high unmet therapeutic need that exists across the spectrum of seizure disorders."

Biscayne expects to advance BIS-001 into a Phase 1b study in adults in mid-2017, and if all goes well, to proceed to a Phase 2 study shortly thereafter. Clinical trials in children will commence after successful completion of FDA-required juvenile animal safety testing.

Steven Schachter MD, Professor of Neurology at Harvard Medical School, is a Scientific Co-Founder of Biscayne and a co-author of the new study. He commented, “It is noteworthy that these encouraging results are from the Emory lab of Dr. Escayg, who is a widely respected scientist and leading investigator in the genetics of neurological diseases. Severe seizure disorders can destroy the lives of patients and their families. We are eager to test BIS-001 in clinical trials to see if the dramatic elimination of seizures observed in these animals can be replicated in patients, who are in great need of better treatment options.”

1 - Wong JC, Dutton SBB, Collins SD, Schachter S and Escayg A (2016) *Huperzine A Provides Robust and Sustained Protection against Induced Seizures in Scn1a Mutant* Mice. Front. Pharmacol. 7:357. doi*:* 10.3389/fphar.2016.00357

About Biscayne Pharmaceuticals
Biscayne Pharmaceuticals is a clinical-stage biotechnology company developing drugs in two major disease areas: CNS disorders such as refractory epilepsy and drug-resistant cancer. Both programs are based on novel approaches with demonstrated potential for superior efficacy and safety. Lead CNS compound BIS-001 has shown striking efficacy in highly predictive models of difficult-to-treat epileptic conditions such as complex partial seizures and Dravet syndrome. Biscayne is also developing new cancer therapies that block growth hormone-releasing hormone (GHRH) receptors, a novel target that is present on many cancer cells. Biscayne’s technology is licensed from Harvard University, the University of Miami, Yale University and the University of South Florida. Biscayne is headquartered in Miami, FL. For more information, visit biscaynepharmaceuticals.com