

June 25, 2019



Lixte Biotechnology's PP2A Inhibitor, LB-100, Improves Brain Function and Motor Impairment in a Mouse Model of Angelman Syndrome, a Rare Human Neurodevelopmental Disorder

EAST SETAUKET, NY, June 25, 2019 (GLOBE NEWSWIRE) -- Lixte Biotechnology Holdings, Inc. ([OTCQB: LIXT](#)) noted that a group of neuroscientists in China and Japan reported that Lixte's lead clinical compound, LB-100, improved muscle strength, movement coordination and learning in a mouse model of Angelman syndrome (AS), a rare disease affecting 1/12,000 to 1/20,000 persons in the US (Wang et al., Proceedings of the National Academy of Science, June 3, 2019). This research reportedly involved Lixte's proprietary compound, but Lixte and its principals are not associated with this research.

Dr. John S. Kovach, CEO of Lixte, said "AS patients have a constellation of signs and symptoms that may include intellectual and speech limitation, difficulty moving or balancing, frequent smiling and laughter, seizures, rigid movements, hyperactivity and sleep disorders. Generally, there is no family history and most AS patients have a normal life span. Mice genetically modified to have reduced function of the UBE3A gene have features of human AS including movement disorder, seizure phenomena, and cognitive impairment as well as abnormalities in structural features of brain cells. In their publication, Wang and colleagues report that they found that brain tissue of AS mice has increased concentrations of protein phosphatase 2A (PP2A), a molecular target of Lixte's investigational compound, LB-100. They report showing that systemic administration of LB-100 to young AS mice improved their movement disorder and the structure and function of their abnormal brain cells. This is a completely unanticipated finding. Whether LB-100 could possibly be of therapeutic benefit in AS is a question Lixte is discussing with experts in the field."

Dr. Kovach continued "apart from the intriguing insight into a possible therapeutic approach to the symptoms of Angelman's syndrome, Lixte is encouraged by the study of Wang and colleagues because as reported it indicates that (some) LB-100 penetrates the brain, at least in mice with AS. In preclinical studies, LB-100 potentiates the effectiveness of standard treatments for several types of central nervous system cancers including glioblastoma multiforme, the most aggressive brain tumor of adults. However, it is not known whether LB-100 penetrates the human brain.

While the Wang publication is suggestive, as Lixte recently reported, the National Cancer Institute (NCI) is conducting a pharmacologic study to definitively determine if LB-100 enters the brain and tumor tissue in patients having surgical removal of recurrent glioblastomas (clinical trials registry: NCT03027388). If so, there will be a sound rationale for investigating whether the addition of LB-100 improves the efficacy of standard treatment for glioblastoma

and potentially other brain cancers.” For patients interested in enrolling in the NCI clinical study, please contact NCI’s toll-free number 1-800-4-Cancer (1-800-422-6237) (TTY: 1-800-332-8615) and/or the website: <https://trials.cancer.gov>.

About Lixte Biotechnology Holdings, Inc.

[Lixte](#) is a biotech company that identifies enzyme targets associated with serious common diseases and then designs novel compounds to attack those targets. Lixte's product pipeline is primarily focused on inhibitors of protein phosphatases, used alone and in combination with cytotoxic agents and/or X-ray and immune checkpoint blockers.

Forward-Looking Statements

This announcement contains certain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, and Section 21E of the Securities Exchange Act of 1934. For example, statements regarding the Company's financial position, business strategy and other plans and objectives for future operations, and assumptions and predictions about future product demand, supply, manufacturing, costs, marketing and pricing factors are all forward-looking statements. These statements are generally accompanied by words such as "intend," "anticipate," "believe," "estimate," "potential(ly)," "continue," "forecast," "predict," "plan," "may," "will," "could," "would," "should," "expect" or the negative of such terms or other comparable terminology. The Company believes that the assumptions and expectations reflected in such forward-looking statements are reasonable, based on information available to it on the date hereof, but the Company cannot provide assurances that these assumptions and expectations will prove to have been correct or that the Company will take any action that the Company may presently be planning. However, these forward-looking statements are inherently subject to known and unknown risks and uncertainties. Actual results or experience may differ materially from those expected or anticipated in the forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, regulatory policies, available cash, research results, competition from other similar businesses, and market and general economic factors. This discussion should be read in conjunction with the Company's filings with the United States Securities and Exchange Commission at <http://www.sec.gov/edgar.shtml>.

Additional information on the Company is available at www.lixte.com.

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Source: Lixte Biotechnology Holdings, Inc.