

# XOMA Receives Orphan Drug Designation for XOMA 358 From U.S. FDA for Treatment of Congenital Hyperinsulinism (HI)

BERKELEY, Calif., June 16, 2015 (GLOBE NEWSWIRE) -- XOMA Corporation (Nasdaq:XOMA), a leader in the discovery and development of therapeutic antibodies, announced today XOMA 358, a fully human allosteric monoclonal antibody that reduces both the binding of insulin to its receptor and downstream insulin signaling, has been granted Orphan Drug Designation by the U.S. Food and Drug Administration (FDA) for the treatment of congenital hyperinsulinism (HI).

"The orphan drug designation for XOMA 358 recognizes its potential to address a significant unmet medical need for patients with congenital hyperinsulinism. Patients with hyperinsulinism, a rare and devastating disease, secrete inappropriate and excessive insulin, which cause dangerously low blood sugar levels that can lead to brain damage or, in rare cases, death. Currently options to manage many of these patients are limited to continuous ingestion of glucose or pancreatectomy," said Paul Rubin, M.D., Senior Vice President, Research and Development, and Chief Medical Officer at XOMA. "We are developing XOMA 358 as a first-in-class therapeutic for conditions of hyperinsulinemic hypoglycemia. We recently completed a positive Phase 1 study, results of which suggest XOMA 358 is active against the insulin receptor and shows potential in treating patients who experience an endogenous over-production of insulin. Congenital hyperinsulinism is one of the indications we are considering for a Phase 2 study, which we expect to initiate later this year."

Orphan drug designation is granted by the FDA Office of Orphan Products Development (OOPD) to novel drugs or biologics that treat a rare disease or condition affecting fewer than 200,000 patients in the United States. The designation provides the drug developer with a seven-year period of U.S. marketing exclusivity, as well as tax credits for clinical research costs, the ability to apply for annual grant funding, clinical research trial design assistance, and waiver of Prescription Drug User Fee Act (PDUFA) filing fees. The OOPD also works on rare disease issues with the medical and research communities, professional organizations, academia, governmental agencies, industry, and rare disease patient groups.

# **About XOMA 358**

Insulin is the major hormone for lowering blood glucose levels. Abnormal increases in insulin secretion can lead to profound hypoglycemia (low blood sugar), a state that can result in

significant morbidities including cerebral damage and epilepsy. In some instances, profound hypoglycemia can be fatal.

XOMA, leveraging its scientific expertise in allosteric monoclonal antibodies, developed the XMet platform. This platform consists of three classes of selective insulin receptor modulators (SIRMs) that could have a major effect on treating patients with abnormal metabolic states. XOMA 358, the lead antibody in the XMetD program (designed to deactivate the insulin receptor), is a fully human allosteric monoclonal antibody that binds to insulin receptors and attenuates insulin action. XOMA 358 is being investigated as a novel treatment for non-drug-induced, endogenous hyperinsulinemic hypoglycemia (low blood glucose caused by excessive insulin production) and other related disorders. A therapy that safely and effectively mitigates insulin-induced hypoglycemia has the potential to address a significant unmet therapeutic need for certain rare medical conditions associated with hyperinsulinism.

XOMA presented positive Phase 1 data on XOMA 358 at the Endocrine Society's Annual meeting (ENDO 2015) in March 2015. Results of the study, in which 14 healthy volunteers received XOMA 358 and 5 received placebo, showed XOMA 358 reduced insulin receptor signaling and increased glucose production after exogenous insulin injection. In the study, XOMA 358 appeared to be well tolerated, with no serious adverse events observed.

# About Congenital Hyperinsulinism[i],[ii],[iii],[iv]

Congenital Hyperinsulinism (HI) is a genetic disorder in which the insulin cells of the pancreas (beta cells) secrete inappropriate and excessive insulin. Ordinarily, beta cells secrete just enough insulin to keep blood sugar in the normal range. In children with HI, the secretion of insulin is not properly regulated, causing excess insulin secretion and frequent episodes of low blood sugar (hypoglycemia). In infants and young children, these episodes are characterized by a lack of energy (lethargy), irritability or difficulty feeding. Repeated episodes of low blood sugar increase the risk for serious complications, such as breathing difficulties, seizures, intellectual disability, vision loss, brain damage, coma, and possibly death. About 60 percent of infants with HI experience a hypoglycemic episode within the first month of life. Other affected children develop hypoglycemia by early childhood. Current treatments for HI are limited to medical therapy and surgical removal of part or all of the pancreas (pancreatectomy).

Congenital HI is a rare disease, affecting approximately 1 in 50,000 newborns.

### **About XOMA Corporation**

XOMA Corporation is a leader in the discovery and development of therapeutic antibodies. The Company's innovative product candidates are the result of the Company's expertise in developing ground-breaking monoclonal antibodies, including allosteric antibodies, which have created new opportunities to potentially treat a wide range of human diseases. XOMA is developing its lead product gevokizumab (IL-1 beta modulating antibody) with Servier through a global Phase 3 program for Behçet's disease uveitis and non-infectious uveitis. XOMA also has an ongoing Phase 3 study of gevokizumab in pyoderma gangrenosum. Additionally, XOMA's scientific research has produced the XMet platform, which consists of three classes of Selective Insulin Receptor Modulators (SIRMs) antibodies. XOMA 358, the lead antibody in the XMetD program, is an allosteric monoclonal antibody that reduces both

the binding of insulin to its receptor and down-regulates insulin signaling and could have a major effect on the treatment of abnormal metabolic states. XOMA 358 recently completed Phase 1 testing. For more information, visit <a href="https://www.xoma.com">www.xoma.com</a>.

# **Forward-Looking Statements**

Certain statements contained in this press release including, but not limited to, statements related to anticipated timing of clinical trials, anticipated timing of the release of clinical data, regulatory approval of unapproved product candidates, the anticipated process of clinical data analysis, the anticipated success of any clinical trial, or statements that otherwise relate to future periods are 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. These statements are based on assumptions that may not prove accurate, and actual results could differ materially from those anticipated due to certain risks inherent in the biotechnology industry and for companies engaged in the development of new products in a regulated market. Potential risks to XOMA meeting these expectations are described in more detail in XOMA's most recent filling on Form 10-K and in other SEC fillings. Consider such risks carefully when considering XOMA's prospects. Any forward-looking statement in this press release represents XOMA's views only as of the date of this press release and should not be relied upon as representing its views as of any subsequent date. XOMA disclaims any obligation to update any forward-looking statement, except as required by applicable law.

[i] ghr.nlm.nih.gov/condition/congenital-hyperinsulinism. Accessed June 11, 2015.

[ii] <u>www.chop.edu/conditions-diseases/congenital-hyperinsulinism/about#.VXncFU3bKHt</u>. Accessed June 11, 2015.

[iii] www.chop.edu/conditions-diseases/congenital-hyperinsulinism/about#.VXneYE3bKHu. Accessed June 11, 2015.

[iv] www.ojrd.com/content/pdf/1750-1172-6-63.pdf. Accessed June 11, 2015.

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