Idera Announces FDA Orphan Drug Designation for IMO-8400 for the Treatment of Diffuse Large B-Cell Lymphoma

April 1, 2015 12:30 PM EDT

CAMBRIDGE, Mass. and EXTON, Pa., April 1, 2015 (GLOBE NEWSWIRE) -- Idera Pharmaceuticals, Inc. (Nasdaq:IDRA), a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of novel therapeutics for oncology and rare diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation for IMO-8400, an antagonist of the endosomal Toll-like receptors (TLRs) 7, 8 and 9, for the treatment of diffuse large B-cell lymphoma (DLBCL).

Idera is currently conducting a clinical trial of IMO-8400 in patients with relapsed or refractory DLBCL harboring MYD88 L265P oncogenic mutation (ClinicalTrials.gov identifier NCT02252146). Preclinical studies have shown that in B-cell lymphomas characterized by the MYD88 L265P oncogenic mutation, including DLBCL, TLR signaling is over-activated, thereby enabling tumor cell survival and proliferation. As a TLR antagonist, IMO-8400 inhibits TLR signaling.

The objectives of the trial are to evaluate the compound's safety, tolerability and clinical activity. The protocol includes three dose-escalation cohorts of IMO-8400 administered subcutaneously.

"The Orphan Drug designation granted today represents another positive milestone for our B-Cell Lymphoma clinical development program," stated James J. O'Leary, MD, Idera's interim Chief Medical Officer. "We continue to advance our efforts in DLBCL, as well as our ongoing clinical trial in Waldenstrom's macroglobulinemia (WM), which we expect to complete and have full data available in the fourth quarter of this year."

Orphan drug designation is granted by the FDA Office of Orphan Products Development to drugs intended for the treatment of a rare disease or condition that affects fewer than 200,000 people in the United States. This designation provides certain incentives, including eligibility for federal grants, research and development tax credits, waiver of PDUFA filing fees and a seven-year marketing exclusivity period, once the product is approved and as long as orphan drug designation is maintained.

The approval of an orphan drug designation request does not alter the standard regulatory requirements and processes for obtaining marketing approval of an investigational drug. Sponsors must establish safety and efficacy of a compound in the treatment of a disease through adequate and well-controlled studies.

About Diffuse Large B-cell Lymphoma (DLBCL)

DLBCL is the most common form of non-Hodgkin lymphoma (NHL) with approximately 20,000 new cases diagnosed in the United States each year.[1] DLBCL is a fast-growing and potentially lethal lymphoma. Initial symptoms may include rapid swelling in the neck, armpit and groin due to enlarged lymph nodes. Other symptoms include night sweats, unexplained fevers and weight loss.[2] The company believes that about 10 percent of DLBCL patients harbor the MYD88 L265P oncogenic mutation, and data from an independent trial of DLBCL patients have shown that prognosis in this population is poor, with overall survival markedly impaired compared to patients without the MYD88 L265P mutation.[3,4,5]

About IMO-8400

Idera's Toll-like receptor (TLR) antagonist drug candidates have been created using a proprietary chemistry-based drug discovery platform. IMO-8400 is a first-in-class synthetic oligonucleotide-based antagonist of endosomal TLRs 7, 8, and 9. In April 2014, Idera presented preclinical data at the American Association for Cancer Research Annual Meeting from preclinical studies in which IMO-8400 inhibited the survival and proliferation of human B-cell lymphoma cells, including Waldenstrom's macroglobulinemia (WM) cells, harboring the oncogenic MYD88 L265P genetic mutation. IMO-8400 has been well-tolerated in a Phase 1 trial in 42 healthy subjects at single and multiple escalating doses up to 0.6 mg/kg for four weeks, and has shown inhibition of immune responses mediated by TLRs 7, 8, and 9. Idera is pursuing clinical development of IMO-8400 in genetically defined forms of B-cell lymphoma, including WM and diffuse large B-cell lymphoma in patients harboring the MYD88 L265P mutation, and in rare autoimmune diseases, including dermatomyositis.

About Idera Pharmaceuticals, Inc.

Idera Pharmaceuticals is a clinical-stage biopharmaceutical company developing a novel therapeutic approach for the treatment of genetically defined forms of B-cell lymphoma and rare diseases. Idera's proprietary technology involves using a TLR targeting technology, to design synthetic oligonucleotide-based drug candidates to act by modulating the activity of specific TLRs. In addition to its TLR programs, Idera is developing gene silencing oligonucleotides (GSOs) that it has created using its proprietary technology to inhibit the production of disease-associated proteins by targeting RNA.

References:

[1] Cultrera JL, et al. Diffuse large B-cell lymphoma: current strategies and future directions. Cancer Control. 2012; 19(3):204-213.

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[3] Wang, et al. Emerging targets in human lymphoma: targeting the *MYD88* mutation. Blood Lymph Canc 2013; 3: 53-61.

[4] Rosenwald A, et al. The use of molecular profiling to predict survival after chemotherapy for diffuse large-B-cell lymphoma. N Engl J Med. 2002 Jun 20; 346(25):1937-47.

[5] Fernandez-Rodriguez C, et al. MYD88 (L265P) mutation is an independent prognostic factor for outcome in patients with diffuse large B-cell

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Forward Looking Statements

This press release contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. All statements, other than statements of historical fact, included or incorporated in this press release, including statements regarding the Company's strategy, future operations, collaborations, intellectual property, cash resources, financial position, future revenues, projected costs, prospects, plans, and objectives of management, are forward-looking statements. The words "believes," "anticipates," "estimates," "plans," "expects," "intends," "may," "could," "should," "potential," "likely," "projects," "continue," "will," and "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Idera cannot guarantee that it will actually achieve the plans, intentions or expectations disclosed in its forward-looking statements and you should not place undue reliance on the Company's forward-looking statements. There are a number of important factors that could cause Idera's actual results to differ materially from those indicated or implied by its forward-looking statements. Factors that may cause such a difference include: whether results obtained in preclinical studies and clinical trials such as the results described in this release will be indicative of the results that will be generated in future clinical trials; whether products based on Idera's technology will advance into or through the clinical trial process when anticipated or at all or warrant submission for regulatory approval; whether such products will receive approval from the U.S. Food and Drug Administration or equivalent foreign regulatory agencies; whether, if the Company's products receive approval, they will be successfully distributed and marketed; orphan drug designation will result in orphan drug exclusivity for IMO-8400; and such other important factors as are set forth under the caption "Risk Factors" in the Company's Annual Report on Form 10-K for the year ended December 31, 2014. Although Idera may elect to do so at some point in the future, the Company does not assume any obligation to update any forward-looking statements and it disclaims any intention or obligation to update or revise any forward-looking statement, whether as a result of new information, future events or otherwise.

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