Idera Provides Key Updates on Clinical Development of IMO-8400 for Treatment of Waldenstrom's Macroglobulinemia

December 30, 2014 1:00 PM EST

- FDA grants Orphan Drug Designation for IMO-8400 for the treatment of Waldenström's macroglobulinemia

- Four-week safety review by Data Review Committee for second dose cohort completed in ongoing WM trial; Third dose cohort opened for enrollment

CAMBRIDGE, Mass., Dec. 30, 2014 (GLOBE NEWSWIRE) -- Idera Pharmaceuticals, Inc. (Nasdaq:IDRA), a clinical-stage biopharmaceutical company developing nucleic acid therapeutics for patients with cancer 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 Waldenström's macroglobulinemia (WM). Additionally, Idera is providing a progress update on the ongoing Phase 1/2 clinical trial being conducted in WM.

Idera is currently conducting a Phase 1/2 clinical trial of IMO-8400 in patients with WM (ClinicalTrials.gov identifier NCT02092909) who have a history of relapse or failure to respond to one or more prior therapies. In B-cell lymphomas characterized by the MYD88 L265P oncogenic mutation, including WM, preclinical studies have shown that TLR signaling is overactivated, thereby enabling tumor cell survival and proliferation. About 90 percent of WM patients are reported to harbor the MYD88 L265P oncogenic mutation.

The objectives of the trial are to evaluate the compound's safety, tolerability and potential clinical activity. The protocol includes three dose-escalation cohorts of IMO-8400 administered subcutaneously. The trial's independent data review committee has completed its review of four-week safety data from the second dose cohort (1.2 mg/kg/week) and has determined that Idera may open enrollment in the third dose cohort (2.4 mg/kg/week). Final 24-week safety and clinical activity data are anticipated in the second half of 2015.

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 Waldenström's macroglobulinemia (WM)

Waldenström's macroglobulinemia is a non-Hodgkin lymphoma of malignant lymphoplasmacytic B-cells that commonly involves the blood and bone marrow and may spread to almost any organ in the body. Approximately 1,000 to 1,500 new cases of WM are diagnosed in the United States each year.[1] Symptoms include fatigue, night sweats, headaches, visual problems, pain and abnormal bleeding due to complications such as anemia, retinopathy and peripheral neuropathy.[2] The disease is incurable and there are currently no drugs specifically approved for the treatment of WM in patients harboring the MYD88 L265P oncogenic mutation. About 90 percent of WM patients are reported to harbor the MYD88 L265P oncogenic mutation.[3]

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 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. In March 2014 and December 2014, Idera announced top-line data from a Phase 2 trial that showed evidence of tolerability and clinical activity in patients with psoriasis who were treated with IMO-8400 at doses of up to 0.6 mg/kg/week for 12 weeks. 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

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 autoimmune diseases. Idera's proprietary technology involves creating novel nucleic acid therapeutics designed to inhibit over-activation of Toll-like receptors (TLRs). In addition to its TLR programs, Idera is developing gene silencing oligonucleotides that it has created using its proprietary technology to inhibit the production of disease-associated proteins by targeting RNA. To learn more about Idera, visit www.iderapharma.com.

References:

[1] American Cancer Society. What are the key statistics about Waldenstrom macroglobulinemia? Available at: http://www.cancer.org/cancer/waldenstrommacroglobulinemia/detailedguide/waldenstrom-macroglobulinemia-key-statistics-w-m. Accessed December 2014.

[2] American Cancer Society. Signs and Symptoms of Waldenstrom macroglobulinemia. Available at: http://www.cancer.org/cancer/waldenstrommacroglobulinemia/detailedguide/waldenstrom-macroglobulinemia-signs-symptoms. Accessed December 2014.

[3] Treon SP, et al. MYD88 L265P somatic mutation in Waldenström's macroglobulinemia. N Engl J Med. 2012 Aug 30;367(9):826-33.

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 preclinical and clinical data described in this release will be indicative of the results that will be generated in future clinical trials; whether interim results obtained in preclinical studies and clinical trials will be indicative of the final results of such preclinical studies and clinical trials; whether products based on Idera's technology will advance into or through the clinical trial process on a timely basis or at all, warrant submission for regulatory approval and receive approval from the United States Food and Drug Administration or equivalent foreign regulatory agencies; whether, if the Company's products receive approval, they will be successfully distributed and marketed; whether the Company's cash resources are sufficient to fund the Company's proposed programs and the Company's operations for the period anticipated; whether IMO-8400 will continue to qualify for orphan drug designation and receive market exclusivity pursuant to the applicable orphan drug regulations; and such other important factors as are set forth under the caption "Risk Factors" in the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 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.

CONTACT: Investor and Media Contact

Robert Doody
Vice President, Investor Relations and
Corporate Communications
484-639-7235
rdoody@iderapharma.com

Idera Pharmaceuticals Logo

Idera Pharmaceuticals