



## Idera Presents Preclinical Data at AACR Showing IMO-8400 Inhibits Tumor Growth and Survival Signaling in B-cell Lymphoma Cells with Oncogenic MYD88 L265P Mutation

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*- Data supports TLR antagonism as potential therapeutic approach for genetically defined forms of B-cell lymphoma -*

*- IMO-8400 in Phase 1/2 trial for Waldenström's macroglobulinemia -*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Apr. 7, 2014-- Idera Pharmaceuticals, Inc. (NASDAQ: IDRA) today presented new preclinical data demonstrating the ability of its Toll-like receptor (TLR) antagonist, IMO-8400, to inhibit the survival and proliferation of B-cell lymphoma cells harboring the oncogenic MYD88 L265P genetic mutation. These data add to the growing body of research which supports the Company's efforts to develop IMO-8400 for the treatment of genetically defined forms of B-cell lymphoma. The findings were presented today in a poster session titled "[Immune Modulatory Agents and Interventions](#)" at the 2014 American Association for Cancer Research (AACR) Annual Meeting in San Diego, California.

The data presented today at AACR build upon reports from several independent investigators and provide additional evidence that the MYD88 L265P mutation, which is present in certain B-cell lymphomas, results in over-activation of TLR7 and TLR9 mediated signaling. Blocking these TLRs leads to tumor cell death. IMO-8400, a first-in-class synthetic oligonucleotide-based antagonist of TLRs 7, 8, and 9, is in clinical development for the treatment of patients with Waldenström's macroglobulinemia and patients with diffuse large B-cell lymphoma (DLBCL) who harbor the MYD88 L265P mutation.

In the presentation, entitled "IMO-8400, a selective antagonist of TLRs 7, 8 and 9, inhibits MYD88 L265P mutation-driven signaling and cell survival: A potential novel approach for treatment of B-cell lymphomas harboring MYD88 L265P mutation," Idera observed that the knockdown of TLR7 and TLR9 expression in DLBCL cells with the MYD88 L265P mutation led to decreased cell signaling, and inhibition of cell survival, consistent with previous reports of other investigators. In the experiments presented today, treatment of DLBCL cells expressing the MYD88 L265P mutation with IMO-8400 led to cell death and decreased proliferative cell signaling. Key signaling pathways inhibited include IRAK-1, IRAK-4, BTK, STAT-3, Iκ-Bα, and NFκ-B. Treatment with IMO-8400 also inhibited growth of established tumors of human DLBCL cells harboring the MYD88 L265P mutation in a mouse model. In addition, treatment with IMO-8400 of Waldenström's macroglobulinemia cells expressing the MYD88 L265P mutation led to similar negative effects on cell signaling and survival.

"These preclinical data provide evidence that upstream blockade of the TLR signaling pathway can limit the proliferative effect of the oncogenic MYD88 L265P mutation. Blocking activation of this pathway via TLR antagonism represents a novel and promising approach to treating multiple B-cell lymphomas characterized by MYD88 L265P, when compared to the downstream inhibition of specific proteins," stated Lou Brenner, M.D., Senior Vice President and Chief Medical Officer of Idera Pharmaceuticals. "We have carved out a distinct position for IMO-8400 in the clinical development landscape for potential therapies to treat genetically defined forms of B-cell lymphoma and feel that this preclinical data provides additional support for our ongoing efforts."

"IMO-8400 has been well tolerated in a recently completed clinical proof-of-concept trial in patients with psoriasis, with a treatment duration of up to twelve weeks, and has shown clinical activity," said Sudhir Agrawal, D. Phil, Chief Executive Officer of Idera Pharmaceuticals. "With the accumulated clinical data on IMO-8400 and the preclinical data presented today, we have a strong foundation to accelerate clinical development in patients with B-cell lymphomas harboring the MYD88 L265P mutation."

Idera has opened enrollment in a Phase 1/2 trial of IMO-8400 designed to evaluate IMO-8400's safety, tolerability and potential clinical activity in patients with Waldenström's macroglobulinemia who are refractory to prior therapies. The Company anticipates that patient treatment in this trial will begin in the second quarter of 2014. Idera has also submitted a separate protocol to the U.S. Food and Drug Administration (FDA) to conduct a Phase 1/2 trial in patients with DLBCL who harbor the MYD88 L265P mutation.

A copy of the presentation, entitled "IMO-8400, a selective antagonist of TLRs 7, 8 and 9, inhibits MYD88 L265P mutation-driven signaling and cell survival: A potential novel approach for treatment of B-cell lymphomas harboring MYD88 L265P mutation" (Abstract #2570), is available at the following link: <http://www.iderapharma.com/our-science/key-presentations-and-publications>.

### **About IMO-8400**

Idera's 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 TLRs 7, 8, and 9. IMO-8400 has shown activity in preclinical models of autoimmune diseases, including psoriasis, lupus, and arthritis. 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 addition, recently announced top-line data from a Phase 2 trial demonstrated that IMO-8400 was well-tolerated and showed evidence of clinical activity in patients with psoriasis who were treated at doses of up to 0.3 mg/kg/week for 12 weeks. Idera is pursuing clinical development of IMO-8400 in genetically defined forms of B-cell lymphoma, including Waldenström's macroglobulinemia and diffuse large B-cell lymphoma, and orphan autoimmune diseases, including polymyositis and 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 orphan 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.

### **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, including in clinical trials in different disease indications; whether products based on Idera’s technology will advance into or through the clinical trial process on a timely basis or at all 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; 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, 2013. 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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