Idera Announces FDA Orphan Drug Designation for IMO-2125 for the Treatment of Melanoma

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CAMBRIDGE, Mass. and EXTON, Pa., June 22, 2017 (GLOBE NEWSWIRE) -- Idera Pharmaceuticals, Inc. (NASDAQ:IDRA), a clinical-stage biopharmaceutical company developing toll-like receptor and RNA 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-2125, an agonist of endosomal Toll-like receptor (TLR) 9 for the treatment of melanoma Stages IIb to IV.

Idera is currently conducting the Phase 2 portion of the ipilimumab combination arm of a Phase 1/2 clinical trial of intratumoral IMO-2125 in patients with anti-PD-1 refractory metastatic melanoma. The objectives of the current trial are to evaluate IMO-2125's safety, tolerability and clinical activity. The company expects to complete enrollment of the Phase 2 multicenter trial in the second half of 2017 with overall response rate (ORR) data available in the first quarter of 2018. The company has submitted an abstract to provide an update of clinical data from the ongoing trial at the European Society of Medical Oncology (ESMO) Congress being held in September, in Spain.

"The Orphan Drug Designation bestowed by the FDA today, represents another important step in the development of IMO-2125," stated Joanna Horobin, M.B. Ch.B., Idera's Chief Medical Officer. "A substantial proportion of patients with metastatic melanoma do not benefit from anti-PD-1 therapy. For these patients, with PD-1 refractory melanoma, ipilimumab offers a modest benefit with an overall response rate of 10-13%[1,2]. Our goal is to significantly improve on this through the combination of IMO-2125 with ipilimumab. We are increasingly encouraged with the data seen to date and look forward to providing our next clinical data update."

Idera is also enrolling a second arm in the Phase 1/2 clinical trial in patients with PD-1 refractory melanoma to study the combination of IMO-2125 and pembrolizumab which is currently in the dose escalation phase.

In addition to the above mentioned clinical trial, the company recently initiated a trial of IMO-2125 monotherapy in refractory solid tumors, including PD-1 refractory melanoma.

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 the Phase 1/2 trial of IMO-2125 in PD-1 Refractory Melanoma
The Phase 1/2 trial of intratumoral IMO-2125 in combination with ipilimumab or pembrolizumab is being conducted in patients who are refractory to anti-PD-1 therapy. The phase 1 portion of the trial was conducted at MD Anderson Cancer Center and the phase 2 portion of the trial is being conducted at multiple clinical sites. In the Phase 1 arms of the trial, four dose levels of IMO-2125 (4, 8, 16 and 32 mg) have been administered intratumorally in one selected lesion at weeks 1, 2, 3, 5, 8 and 11, in combination with the standard dosing regimens of ipilimumab or pembrolizumab, beginning on week 2. The Phase 2 expansion portion of the trial utilizes a Simon two-stage design. If at least 2 of the first 10 patients treated at the Phase 2 dose experience confirmed response the futility hurdle has been met and the trial may continue to enroll. Phase 2 will evaluate 21 patients at the phase 2 dose. Tumor biopsies have been collected pre- and post-24 hours of the first dose of IMO-2125, as well as at 8 and 13 weeks to evaluate multiple immune markers. Clinical activity has been evaluated by the RECIST v1.1 criteria. Clinical data from this study has been presented at SITC 2017, ASCO-SITC 2017 and AACR 2017, and can be found also on Idera's corporate website at http://www.iderapharma.com/our-approach/key-publications/.

About IMO-2125
Toll-like receptors (TLRs) play a central role in the innate immune system, the body's first line of defense against invading pathogens, as well as damaged or dysfunctional cells including cancer cells. The innate immune system is also involved in activating the adaptive immune system, which marshals highly specific immune responses to target pathogens or tissue. Cancer cells may exploit regulatory checkpoint pathways to avoid being recognized by the immune system, thereby shielding the tumor from immune attack. Checkpoint inhibitors such as agents targeting CTLA4 or programmed cell death protein 1 (PD1) are designed to enable the immune system to recognize tumor cells. In this setting, intratumoral TLR9 agonist administration may increase the tumor-infiltrating lymphocytes (TILs), and thereby potentiate anti-cancer activity of checkpoint inhibitors in the injected tumor as well as systemically.

IMO-2125, Idera's TLR9 agonist, has been created using the company's proprietary chemistry-based discovery platform. IMO-2125 has been shown in various scientific presentations and publications to activate dendritic cells and induce interferon. Idera selected IMO-2125 to advance into clinical development in combination with checkpoint inhibitors based on this immunological profile. In previously completed clinical trials, subcutaneous administration of IMO-2125 was very well tolerated in about 114 patients with hepatitis C. Idera has conducted further preclinical and clinical research evaluating the potential of IMO-2125 to enhance the anti-tumor activity of other checkpoint inhibitors in cancer immunotherapy with data has been presented at several scientific and medical conferences during the past few years. The posters from these presentations can be found at http://www.iderapharma.com/our-approach/key-publications.

About Metastatic Melanoma
Melanoma is a type of skin cancer that begins in a type of skin cell called melanocytes. As is the case in many forms of cancer, melanoma becomes more difficult to treat once the disease has spread beyond the skin to other parts of the body such as by through the lymphatic system (metastatic disease). Melanoma accounts for only one percent of skin cancer cases, but causes a large majority of skin cancer deaths. The American Cancer Society estimates that in 2017, there will be 87,110 new cases of melanoma in the U.S., and about 9,730 will die of this disease. Based on proprietary Idera research, the company anticipates by the year 2025, there will be roughly 13,000 anti-PD-1 refractory metastatic melanoma patients.

About Idera Pharmaceuticals
Idera Pharmaceuticals is a clinical-stage biopharmaceutical company developing novel nucleic acid-based therapies for the treatment of certain cancers and rare diseases. Idera's proprietary technology involves designing synthetic oligonucleotide-based drug candidates to modulate the activity of specific TLRs. In addition to its TLR programs, Idera has used its proprietary knowledge to create a third generation antisense technology platform which inhibits the production of disease-associated proteins by targeting RNA. To learn more about Idera, visit www.iderapharma.com.

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 interim results from a clinical trial, such as preliminary results reported in this release, will be predictive of the final results of the trial, whether results obtained in preclinical studies and clinical trials such as the preclinical data 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 and on Form 10-Q for the period ended March 31, 2017. 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.

References

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2. Long GV, et al. Outcomes in patients treated with Ipilimumab after pembrolizumab in KEYNOTE-006. Soc Mel Res, 2016.

Investor and Media Contact
Robert Doody
Vice President, Investor Relations and Corporate Communications
Office: 617-679-5515
Mobile: 484-639-7235
rdoody@iderapharma.com

Idera Pharmaceuticals, Inc.