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# Mirati Therapeutics Receives Orphan Drug Designation from U.S. Food & Drug Administration for Mocetinostat in Myelodysplastic Syndrome

SAN DIEGO, June 17, 2014 /PRNewswire/ -- Mirati Therapeutics, Inc. (NASDAQ: MRTX) announced that mocetinostat, the company's spectrum selective HDAC inhibitor, has been granted Orphan Drug Designation by the U.S. Food & Drug Administration as a treatment for myelodysplastic syndrome (MDS). Mocetinostat is being developed in Phase 2 clinical studies in combination with Vidaza as a treatment for intermediate and high-risk MDS, as well as a single agent treatment in patients with diffuse large B-cell lymphoma (DLBCL) and bladder cancer targeting specific genetic mutations in histone acetylation that increase the likelihood of response in tumor cells.

"Orphan designation is an important piece of the development plan for mocetinostat as we evaluate combination and single agent clinical development opportunities for the program," said Dr. Charles Baum, president and CEO of Mirati. "We are excited about the opportunity to identify and select patients whose cancers may be especially sensitive to mocetinostat, and we expect to have initial data from Phase 2 studies by the end of the year which will allow us to move quickly into a registration path."

The FDA's Office of Orphan Drug Products grants orphan status to support development of medicines for underserved patient populations or rare disorders that affect fewer than 200,000 people in the United States. Orphan drug designation provides certain benefits, including market exclusivity upon regulatory approval if received, exemption of FDA application fees and tax credits for qualified clinical trials.

"HDAC inhibitors may significantly increase the efficacy of other epigenetic agents such as inhibitors of LSD1, EZH2 and DOT1L as well as hypomethylating agents such as Vidaza in the treatment of MDS and other malignancies," added Baum. "Epigenetic pathways can become dysregulated during cancer progression through a variety of mechanisms, including the genetic alteration of pathways that control DNA methylation and histone modification. These alterations often result in silencing of selected tumor suppressor genes and uncontrolled tumor growth in certain malignancies including MDS, lymphomas and solid tumors such as bladder cancer."

**About Mocetinostat**

Mocetinostat is an orally-bioavailable, spectrum-selective HDAC inhibitor. In addition to the ongoing Phase 2 clinical trials, 13 clinical trials have been completed, which enrolled over 400 patients with a variety of hematologic malignancies and solid tumors. Mocetinostat is enrolling patients in a Phase 2 dose confirmation study in combination with Vidaza as treatment for intermediate and high-risk MDS. Mirati also plans to initiate Phase 2 studies of mocetinostat as a single agent in patients with mutations in histone acetyl transferases in bladder cancer and DLBCL. Initial data from the Phase 2 studies is expected by the end of 2014.

**About Mirati Therapeutics**

Mirati Therapeutics is a targeted oncology company developing an advanced pipeline of breakthrough medicines for precisely defined patient populations. Mirati's approach combines the three most important factors in oncology drug development - drug candidates with complementary and compelling targets, creative and agile clinical development, and a highly accomplished precision medicine leadership team. The Mirati team is using a proven blueprint for developing targeted oncology medicines to advance and maximize the value of its pipeline of drug candidates, including MGCD265 and MGCD516, which are orally bioavailable, multi-targeted kinase inhibitors with distinct target profiles, and mocetinostat, an orally bioavailable, spectrum-selective histone deacetylase inhibitor. More information is available at www.mirati.com.

**Forward Looking Statements**

Certain statements contained in this news release, other than statements of fact that are independently verifiable at the date hereof, may constitute forward-looking information and forward-looking statements (collectively "forward-looking statements" within the meaning of applicable securities laws). Such statements, based as they are on the current expectations of management of Mirati and upon what management believes to be reasonable assumptions based on information currently available to it, inherently involve numerous risks and uncertainties, known and unknown, many of which are beyond Mirati's control. Such statements can usually be identified by the use of words such as "may", "would", "believe", "intend", "plan", "anticipate", "estimate" and other similar terminology, or state that certain actions, events or results "may" or "would" be taken,

occur or be achieved. Forward-looking statements in this release include, but are not limited to, statements regarding the response rates to mocetinostat for patients with bladder cancer or DLBCL, the success around selecting patients whose cancers may be sensitive to mocetinostat, and the timing of data readouts.

Whether actual results and developments will conform with our expectations and predictions is subject to a number of risks, assumptions and uncertainties, many of which are beyond our control, and the effects of which can be difficult to predict. These risks include those inherent in drug development, whether Mirati will be able to obtain financing when needed or on favorable terms, and other risks described in Mirati's filings with the Securities and Exchange Commission. In evaluating any forward-looking statements in this release, Mirati cautions readers not to place undue reliance on any forward-looking statements. Unless otherwise required by applicable securities laws, Mirati does not intend, nor does it undertake any obligation, to update or revise any forward-looking statements contained in this news release to reflect subsequent information, events, results or circumstances or otherwise.

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