



September 2, 2015

## **Spectrum Pharmaceuticals Announces Publication of Pivotal EVOMELA™ (melphalan hydrochloride) for Injection Data in the Biology of Blood and Marrow Transplantation Journal**

- **Published Data Highlights Achievement of Myeloablation (Median Day 5) and Engraftment (Median Day 12-13) with no mortality (Day 100)**
- **EVOMELA is Free of Propylene Glycol, a Diluent Required for Reconstitution of Current Melphalan Formulations that is Associated with Renal and Cardiac Toxicities**
- **Increased Stability of EVOMELA Allows Longer Use Time Facilitating Clinical Administration Logistics**
- **On Track for PDUFA Decision on October 23, 2015; Company Plans to Launch EVOMELA with Existing Sales Force in a Market Estimated at Approximately \$100 Million**

HENDERSON, Nev.--(BUSINESS WIRE)-- Spectrum Pharmaceuticals, Inc. (NasdaqGS: SPPI), a biotechnology company with fully integrated commercial and drug development operations with a primary focus in Hematology and Oncology, today announced publication of results from the pivotal clinical study for EVOMELA, used for myeloablative conditioning in multiple myeloma (MM) patients undergoing autologous transplantation. (ASCT). The study, led by Dr. Parameswaran Hari from Froedtert Hospital and Medical College of Wisconsin, was published in the Biology of Blood and Marrow Transplantation (BBMT) journal.

"We are pleased to have these clinical data selected for publication in the BBMT journal," said Rajesh C. Shrotriya, MD, Chairman and Chief Executive Officer of Spectrum Pharmaceuticals. "These study data confirm the efficacy and acceptable safety profile of EVOMELA as a high-dose conditioning regimen for ASCT in patients with MM. Our novel EVOMELA formulation uses Captisol to improve the solubility and stability of Melphalan, and has eliminated the need for a propylene glycol-containing cosolvent. Importantly, this allows for longer use and infusion times with EVOMELA, which potentially simplifies its clinical use and administration logistics. Instead of propylene glycol, which is associated with toxicities including renal dysfunction and arrhythmias, this new formulation uses a standard aqueous diluent for reconstitution. We look forward to FDA's NDA decision on EVOMELA in October. Spectrum continues to deliver on its commitment to develop improved cancer therapies that benefit patients and health care providers."

The BBMT journal publication includes data on 61 patients who were enrolled in this open-label Phase 2b pivotal study at five US study sites; 56 patients had newly diagnosed disease and five had relapsed MM following prior ASCT. Patients enrolled in this study received 200 mg/m<sup>2</sup> of EVOMELA as two doses on Day -3 and Day -2 prior to ASCT (Day 0). Efficacy was assessed by clinical response at Day +100 with an ORR of 95% and CR rate of 31% (16% stringent CRs) based on investigators' assessments, and rates of 100% and 21%, respectively based on independent pathology review; the lower rate of confirmed CRs in the independent review was due to missing data. Importantly, the five patients who had previously relapsed from a prior ASCT were all shown to achieve a response to EVOMELA. All patients in the study achieved myeloablation with a median of 5 days post-ASCT, and all patients had successful neutrophil and platelet engraftment (median of 12 days and 13 days post-ASCT, respectively). Treatment-related mortality was 0%, and non-hematologic adverse events were mostly Grade 1 and Grade 2 in severity. The incidence of Grade 3 mucositis and Grade 3 stomatitis were 10% and 5%, respectively with no Grade 4 mucositis or stomatitis reported. Twenty percent of patients experienced treatment-emergent serious adverse events, most of which were Grade 3, and consisted of events commonly reported in patients undergoing myeloablative chemotherapy; no new safety signals were identified.

In December 2014, Spectrum submitted an NDA to the FDA for the approval of EVOMELA for use as a high-dose conditioning treatment prior to ASCT in patients with MM. Spectrum is also seeking approval for the palliative treatment of patients with MM for whom oral therapy is not appropriate, which is the indication for the currently approved IV melphalan products. The NDA was accepted and a PDUFA decision is expected on October 23, 2015.

### **About Spectrum Pharmaceuticals, Inc.**

Spectrum Pharmaceuticals is a leading biotechnology company focused on acquiring, developing, and commercializing drug products, with a primary focus in Hematology and Oncology. Spectrum currently markets five hematology/oncology drugs, and expects an FDA decision on another hematology drug later this year. Additionally, Spectrum's pipeline includes three drugs

targeting blockbuster markets in advanced stages of clinical development. Spectrum's strong track record for in-licensing and acquiring differentiated drugs, and expertise in clinical development have generated a robust, diversified, and growing pipeline of product candidates in advanced-stage Phase 2 and Phase 3 studies. More information on Spectrum is available at [www.sppirx.com](http://www.sppirx.com).

## About Multiple Myeloma

Multiple Myeloma is a systemic malignancy of plasma cells that accumulate in the bone marrow, usually associated with monoclonal antibody secretion, and results in bone marrow failure and bone destruction. It is the second most common hematologic disease with nearly 27,000 new cases projected in the US in 2015 and over 11,000 deaths annually (American Cancer Society Stats, 2015). The rate of ASCT for patients with multiple myeloma is growing by approximately 3.3% annually.

While MM is usually sensitive to cytotoxic chemotherapy, most responses are transient and patients frequently relapse. The demonstrated superiority of high-dose chemotherapy with stem cell support over conventional chemotherapy for the treatment of MM has made ASCT the current standard of care for patients, particularly the young, with adequate organ function. Melphalan is the most commonly used IV agent for high-dose conditioning for patients undergoing ASCT for MM. The current IV melphalan market is approximately \$100 million annually, with predominant use in ASCT.

## About EVOMELA™

EVOMELA is a new propylene glycol-free melphalan formulation that has been previously shown to be bioequivalent to the standard melphalan formulation (Alkeran) in a Phase 2 clinical study (Aljitawi et al, Bone Marrow Transplant, 2014). EVOMELA has been granted an Orphan Drug Designation by the FDA for the MM setting.

This formulation eliminates the need to use a propylene glycol containing custom diluent, which is required with other formulations and has been reported to cause renal and cardiac side effects. The use of the Captisol® technology to reformulate melphalan also improves its stability, and is anticipated to allow for slower infusion rates and longer administration durations, potentially enabling clinicians to safely achieve a higher dose intensity for pre-transplant chemotherapy.

Spectrum Pharmaceuticals gained global development and commercialization rights to EVOMELA from Ligand Pharmaceuticals Incorporated (NASDAQ: LGND) in March 2013. Spectrum assumed responsibility for completing the pivotal clinical trial and was responsible for filing the NDA. Under the license agreement, Ligand received a license fee and is eligible to receive milestone payments, as well as royalties following potential commercialization.

## About Captisol®

Captisol is a patent-protected, chemically modified cyclodextrin with a structure designed to optimize the solubility and stability of drugs. Captisol was invented and initially developed by scientists in the laboratories of Dr. Valentino Stella at the University of Kansas' Higuchi Biosciences Center for specific use in drug development and formulation. This unique technology has enabled six FDA-approved products, including Onyx Pharmaceuticals' Kyprolis®, Baxter International's Nexterone® and Merck's NOXAFIL IV. There are also more than 30 Captisol-enabled products currently in clinical development.

Forward-looking statement — This press release may contain forward-looking statements regarding future events and the future performance of Spectrum Pharmaceuticals that involve risks and uncertainties that could cause actual results to differ materially. These statements are based on management's current beliefs and expectations. These statements include, but are not limited to, statements that relate to our business and its future, including certain company milestones, Spectrum's ability to identify, acquire, develop and commercialize a broad and diverse pipeline of late-stage clinical and commercial products, leveraging the expertise of partners and employees around the world to assist us in the execution of our strategy, and any statements that relate to the intent, belief, plans or expectations of Spectrum or its management, or that are not a statement of historical fact. Risks that could cause actual results to differ include the possibility that our existing and new drug candidates may not prove safe or effective, the possibility that our existing and new applications to the FDA and other regulatory agencies may not receive approval in a timely manner or at all, the possibility that our existing and new drug candidates, if approved, may not be more effective, safer or more cost efficient than competing drugs, the possibility that our efforts to acquire or in-license and develop additional drug candidates may fail, our lack of sustained revenue history, our limited marketing experience, our dependence on third parties for clinical trials, manufacturing, distribution and quality control and other risks that are described in further detail in the Company's reports filed with the Securities and Exchange Commission. We do not plan to update any such forward-looking statements and expressly disclaim any duty to update the information contained in this press release except as required by law.

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