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Pharmacyclics Files Supplemental New Drug Application for IMBRUVICA® for Waldenstrom's Macroglobulinemia

Filing completed just three months after receipt of third FDA approval

SUNNYVALE, Calif., Oct. 20, 2014 /PRNewswire/ -- Pharmacyclics, Inc. (NASDAQ: PCYC) today announced that it has submitted a supplemental New Drug Application (sNDA) to the U.S. Food and Drug Administration (FDA) based on data from a Phase 2 study evaluating the use of IMBRUVICA® (ibrutinib) in patients with Waldenstrom's macroglobulinemia (WM), which was led by Dr. Steven Treon from the Dana-Farber Cancer Institute. IMBRUVICA, which received FDA Breakthrough Therapy Designation in February 2013 for patients with WM, is being jointly developed and commercialized by Pharmacyclics and Janssen Biotech, Inc.

WM is a slow-growing, currently incurable, rare type of B-cell lymphoma[1] for which no established standard of care - or approved therapeutic - exists.[2],[3] There are approximately 1,000 to 1,500 new cases each year and a prevalence of 12,000[4] in the United States, and the median age at diagnosis is 60 to 70 years of age.[1],[5] WM begins with a malignant change to the B cell, a type of white blood cell (lymphocyte), during its maturation so that it continues to reproduce more malignant B-cells. WM cells make large amounts of a certain type of antibody (immunoglobulin M, or IgM), which is known as a macroglobulin. Each protein made by the WM cells is the same, so it is called a monoclonal protein, or just an M protein.[6]

"Over the past five years, we have worked diligently on the clinical development for the use of IMBRUVICA in Waldenstrom's macroglobulinemia patients. Today marks an important milestone for these patients," said Thorsten Graef, M.D., PhD, Vice President of Clinical Science, Pharmacyclics. "Our supplemental New Drug Application for this underserved patient population and our ongoing Phase 3 study reinforce our commitment to developing novel therapies that address serious unmet medical needs."

Pharmacyclics has worked closely with Dr. Treon and investigators at the two other clinical trial sites to support and understand the applicability and potential of IMBRUVICA in patients living with WM.

IMBRUVICA is currently approved by the FDA for three indications: for the treatment of patients with mantle cell lymphoma (MCL) or chronic lymphocytic leukemia (CLL) who have received at least one prior therapy, and for CLL patients with a deletion of the short arm of chromosome 17 (del 17p), including treatment-naive and previously treated del 17p CLL patients.[7] Accelerated approval was granted for the MCL indication based on overall response rate (ORR). Improvements in survival or disease-related symptoms have not been established. Continued approval for the MCL indication may be contingent upon verification of clinical benefit in confirmatory trials.[7]

About IMBRUVICA®

IMBRUVICA® (ibrutinib) is a first-in-class, oral, once-daily therapy that inhibits a protein called Bruton's tyrosine kinase (BTK).[7] BTK is a key signaling molecule in the B-cell receptor signaling complex that plays an important role in the survival and spread of malignant B cells.[7],[8] IMBRUVICA blocks signals that tell malignant B cells to multiply and spread uncontrollably.[7]

IMBRUVICA is fully approved for the treatment of patients with CLL who have received at least one prior therapy, and for the treatment of CLL patients with del 17p,[7] a genetic mutation that occurs when part of chromosome 17 has been lost.

IMBRUVICA is also approved for the treatment of patients with mantle cell lymphoma (MCL) who have received at least one prior therapy. Accelerated approval was granted for the MCL indication based on overall response rate (ORR). Improvements in survival or disease-related symptoms have not been established. Continued approval for the MCL indication may be contingent upon verification of clinical benefit in confirmatory trials.[7]

IMBRUVICA is being studied alone and in combination with other treatments in several blood cancers including CLL, MCL, Waldenstrom's macroglobulinemia (WM), diffuse large B-cell lymphoma (DLBCL), follicular lymphoma (FL) and multiple myeloma (MM). Approximately 3,500 patients have received IMBRUVICA in clinical trials conducted in 35 countries, by more than 800 investigators around the world. As of June 30, 2014, 12 Phase III trials have been initiated with IMBRUVICA and approximately 50 trials are registered on www.clinicaltrials.gov. The overall clinical development program in CLL currently

includes seven Phase III trials and covers all lines of therapy and various combinations of treatments.

IMBRUVICA was one the first medicines to receive U.S. FDA approval via the new Breakthrough Therapy Designation pathway, and is the only product to have received three Breakthrough Therapy Designations. IMBRUVICA is jointly developed and commercialized by Janssen Biotech, Inc. and Pharmacyclics.

IMPORTANT SAFETY INFORMATION

Warnings and Precautions include hemorrhage, infection, cyptopenias, atrial fibrillation, second primary malignancies, and embryo-fetal toxicity.

The most common adverse reactions include thrombocytopenia, diarrhea, neutropenia, anemia, fatigue, musculoskeletal pain, peripheral edema, upper respiratory tract infection, nausea, bruising, dyspnea, constipation, rash, abdominal pain, pyrexia, vomiting, and decreased appetite.

For additional important safety information, please see Full Prescribing Information at http://www.imbruvica.com/downloads/Prescribing_Information.pdf.

About Pharmacyclics

Pharmacyclics®, Inc. (NASDAQ: PCYC) is a biopharmaceutical company focused on developing and commercializing innovative small-molecule drugs for the treatment of cancer and immune mediated diseases. The company's mission is to build a viable biopharmaceutical company that designs, develops and commercializes novel therapies intended to improve quality of life, increase duration of life and resolve serious unmet medical needs. It will do so by identifying and controlling promising product candidates based on scientific development and administrational expertise, developing its products in a rapid, cost-efficient manner, and pursuing commercialization and/or development partners when and where appropriate.

Pharmacyclics markets IMBRUVICA and has three product candidates in clinical development and several preclinical molecules in lead optimization. The company is committed to high standards of ethics, scientific rigor, and operational efficiency as it moves each of these programs to commercialization. Pharmacyclics is headquartered in Sunnyvale, CA. To learn more, visit www.pharmacyclics.com.

NOTE: This announcement may contain forward-looking statements made in reliance upon the safe harbor provisions of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, including statements, among others, relating to our future capital requirements, including our expected liquidity position and timing of the receipt of certain milestone payments, and the sufficiency of our current assets to meet these requirements, our future results of operations, our expectations for and timing of ongoing or future clinical trials and regulatory approvals for any of our product candidates, and our plans, objectives, expectations and intentions. Because these statements apply to future events, they are subject to risks and uncertainties. When used in this announcement, the words "anticipate", "believe", "estimate", "expect", "expectation", "goal", "should", "would", "project", "plan", "predict", "intend", "target" and similar expressions are intended to identify such forward-looking statements. These forward-looking statements are based on information currently available to us and are subject to a number of risks, uncertainties and other factors that could cause our actual results, performance, expected liquidity or achievements to differ materially from those projected in, or implied by, these forward-looking statements. Factors that may cause such a difference include, without limitation, our need for substantial additional financing and the availability and terms of any such financing, the safety and/or efficacy results of clinical trials of our product candidates, our failure to obtain regulatory approvals or comply with ongoing governmental regulation, our ability to commercialize, manufacture and achieve market acceptance of any of our product candidates, for which we rely heavily on collaboration with third parties, and our ability to protect and enforce our intellectual property rights and to operate without infringing upon the proprietary rights of third parties. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, performance or achievements and no assurance can be given that the actual results will be consistent with these forward-looking statements. For more information about the risks and uncertainties that may affect our results, please see the Risk Factors section of our filings with the Securities and Exchange Commission, including our transition report on Form 10-K for the six month period ended *December 31, 2012* and quarterly reports on Form 10-Q. We do not intend to update any of the forward-looking statements after the date of this announcement to conform these statements to actual results, to changes in management's expectations or otherwise, except as may be required by law.

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[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 October 2014.

[2] Treon SP, Gertz MA, Dimopoulos M, et al. (2006) Update on treatment recommendations from the Third International Workshop on Waldenstrom's macroglobulinemia. Blood 107:3442-3446.

[3] Ghobrial, I. Choice of Therapy for Patients with Waldenstrom Macroglobulinemia. Journal of Clinical Oncology. 2012. doi: 10.1200/JCO.2012.46.6177.

[4] IMS patient claims estimates for July 2013-June 2014. Note: This information is an estimate derived from the use of information under license from the following IMS Health Incorporated information service: IMS Oncology Tracking Reports for the period July 2013 to June 2014. IMS expressly reserves all rights, including rights of copying, distribution and republication.

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[6] American Cancer Society. "What is Waldenstrom macroglobulinemia?" Available at: http://www.cancer.org/cancer/waldenstrommacroglobulinemia/index. Accessed October 2014.

[7] IMBRUVICA Prescribing Information, July 2014.

[8] Genetics Home Reference. Isolated growth hormone deficiency. Available from: http://ghr.nlm.nih.gov/condition/isolated-growth-hormone-deficiency. Accessed October 2014.

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/pharmacyclics-files-supplemental-new-drug-application-for-imbruvica-for-waldenstroms-macroglobulinemia-433231354.html

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