U.S. FDA Grants Regular (Full) Approval for IMBRUVICA® for Two Indications

Previously Treated Chronic Lymphocytic Leukemia (CLL) Based on Statistically Significant Progression-Free and Overall Survival Benefits
Del 17P CLL, Only FDA-Approved Agent

SUNNYVALE, Calif., July 28, 2014 /PRNewswire/ -- Pharmacyclics, Inc. (NASDAQ: PCYC) today announced that the U.S. Food and Drug Administration (FDA) has granted IMBRUVICA® (ibrutinib) regular (full) approval for the treatment of patients with chronic lymphocytic leukemia (CLL) who are del 17p positive and for patients with the del 17p mutation. Patients receiving IMBRUVICA demonstrated a statistically significant improvement in progression-free survival (PFS), overall survival (OS) and overall response rate (ORR) as compared to patients treated with ofatumumab. The median PFS and OS has not been reached on the IMBRUVICA arm. There was a 78% statistically significant reduction in the risk of progression or death as assessed by an independent review committee (IRC) according to the modified IWCLL criteria (HR 0.22, 95% CI, 0.15 to 0.32). In addition, the analysis of overall survival demonstrated a 57% statistically significant reduction in the risk of death for patients in the IMBRUVICA arm (HR 0.43; 95 CI, 0.24 to 0.79). This was observed despite a total of 57 patients who were initially randomized to ofatumumab crossing over to receive IMBRUVICA prior to the analysis. For previously treated del 17p CLL patients, there was a 75% reduction in the risk of progression or death as assessed by an IRC (HR 0.25, 95% CI, 0.14 to 0.45).

"IMBRUVICA demonstrated substantial evidence of its superiority over ofatumumab and significant benefit for previously treated CLL patients, while maintaining a favorable safety profile. This FDA approval for IMBRUVICA is a major step toward chemo-free treatment in CLL," said John Byrd, M.D.,* Director, Division of Hematology, The Ohio State University Comprehensive Cancer Center - Arthur G. James Cancer Hospital & Richard J. Solove Research Institute and lead investigator for RESONATE.

"Patients with deletion 17p CLL are at particularly high risk for poor outcomes. Today's approval of IMBRUVICA provides these patients with the only FDA-approved treatment, regardless of whether their disease is treatment naïve or previously treated. I continue to be awed by the duration of my patients’ responses to IMBRUVICA and am grateful IMBRUVICA now is available to a broader group of CLL patients."

CLL is a slow-growing blood cancer of the white blood cells. CLL is the most common adult leukemia in the Western world and predominately a disease of the elderly with a median age at diagnosis of 72 years.

"We are delighted IMBRUVICA has received full approval by demonstrating its ability to improve progression-free survival and, importantly, overall survival as compared to an approved standard of care, and that IMBRUVICA is now available to all patients with del 17p CLL," said Danelle James, M.D., Vice President, Clinical Development, Pharmacyclics. "Our goal is to provide patients with clinically meaningful treatments. Thanks to the physicians and patients who helped us complete this trial in near record time, today, we have delivered on that goal by bringing IMBRUVICA to an even broader group of patients."

Within CLL, the most commonly occurring adverse reactions (>20%) were thrombocytopenia, neutropenia, diarrhea, anemia, upper respiratory tract infection, musculoskeletal pain, bruising, rash, fatigue, nausea, and pyrexia. Approximately 5% of patients with CLL receiving IMBRUVICA discontinued treatment due to adverse events. These included infections (2%), subdural hematoma (2%) and diarrhea (1%). Adverse events leading to dose reduction occurred in approximately 6% of patients. The Warnings and Precautions include: hemorrhage, infections, cytopenias, atrial fibrillation, secondary primary malignancies, embryo-fetal toxicities.
This approval for IMBRUVICA triggers $60 million in milestone payments to Pharmacyclics under its collaboration agreement with Janssen Biotech, Inc.

Corporate Conference Call
The Company will hold a conference call today at 11:00 AM PT. To participate in the conference call, please dial 1-877-303-7908 (domestic callers) or 1-678-373-0875 (international callers), and use conference ID number 81316392. To access the live audio broadcast or the subsequent archived recording, log on to http://ir.pharmacyclics.com/events.cfm.

About IMBRUVICA®
IMBRUVICA® is a first-in-class, oral, once-daily therapy that inhibits a protein called Bruton's tyrosine kinase (BTK). 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. IMBRUVICA blocks signals that tell malignant B cells to multiply and spread uncontrollably.

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.

To date, 12 Phase III trials have been initiated with IMBRUVICA and a total of 50 trials are currently 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. Janssen and Pharmacyclics entered a collaboration and license agreement in December 2011 to jointly develop IMBRUVICA.

Patient Access to IMBRUVICA

Patients who are prescribed IMBRUVICA can receive access support through a variety of programs:

- The YOU&i Start™ program enables eligible patients who are experiencing insurance coverage delays to access free product for a limited time.
- The YOU&i Access™ Instant Savings Program helps commercially insured, eligible patients who have difficulties with out-of-pocket expenses for IMBRUVICA. Eligible patients may receive support to reduce their monthly out-of-pocket costs to $25 per month.
- The YOU&i Access Service Center assists patients with all access-related administration issues.
- The Johnson & Johnson Patient Assistance Foundation (JJPAF), an independent, non-profit organization to which Pharmacyclics makes donations, allows patients who are deemed uninsured and eligible, and who qualify based on financial need, access to IMBRUVICA.

For more information about these comprehensive patient access programs, call or visit 1-877-877-3536 or www.IMBRUVICA.com.

About Chronic Lymphocytic Leukemia (CLL) / Small Lymphocytic Leukemia (SLL)
The prevalence of CLL/SLL is approximately 115,000 patients in the United States, with approximately 16,000 newly diagnosed patients every year. As this orphan disease frequently progresses following first-line therapy, patients are faced with fewer treatment options and often are prescribed multiple lines of therapy as they relapse or become resistant to treatments.

In CLL/SLL, the genetic mutation del 17p occurs when part of chromosome 17 has been lost or deleted. CLL/SLL patients with del 17p have poor treatment outcomes. Del 17p is reported in approximately 7% of treatment-naïve CLL/SLL cases, and approximately 20% to 40% of relapsed/refractory patients harbor the mutation.

About Pharmacyclics
Pharmacyclics® is a biopharmaceutical company focused on developing and commercializing innovative small-molecule drugs for the treatment of cancer and immune mediated diseases. Our mission and goal 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 healthcare needs; and to identify and control promising product candidates based on scientific development and administrative expertise, develop our products in a rapid, cost-efficient manner and pursue commercialization and/or development partners when and where appropriate.

Pharmacyclics markets IMBRUVICA and has three product candidates in clinical development and five 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 viable commercialization.
Pharmacyclics (NASDAQ: PCYC) is headquartered in Sunnyvale, CA. To learn more about Pharmacyclics, please visit www.pharmacyclics.com.

INDICATIONS

IMBRUVICA is indicated to treat people with:

- Chronic lymphocytic leukemia (CLL) who have received at least one prior therapy
- Chronic lymphocytic leukemia (CLL) with 17p deletion

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS

Hemorrhage - Grade 3 or higher bleeding events (subdural hematoma, gastrointestinal bleeding, hematuria, and post-procedural hemorrhage) have occurred in up to 6% of patients. Bleeding events of any grade, including bruising and petechiae, occurred in approximately half of patients treated with IMBRUVICA®.

The mechanism for the bleeding events is not well understood. IMBRUVICA® may increase the risk of hemorrhage in patients receiving anti-platelet or anti-coagulant therapies. Consider the benefit-risk of withholding IMBRUVICA® for at least 3 to 7 days pre- and post-surgery depending upon the type of surgery and the risk of bleeding.

Infections - Fatal and non-fatal infections have occurred with IMBRUVICA®. Twenty-six percent of patients with CLL had infections Grade 3 or greater NCI Common Terminology Criteria for Adverse Events (CTCAE). Monitor patients for fever and infections and evaluate promptly.

Cytopenias - Treatment-emergent Grade 3 or 4 cytopenias including neutropenia (range, 26 to 29%), thrombocytopenia (range, 10 to 17%), and anemia (range, 0 to 9%) occurred in patients treated with IMBRUVICA®. Monitor complete blood counts monthly.

Atrial Fibrillation - Atrial fibrillation and atrial flutter (range, 6 to 9%) have occurred in patients treated with IMBRUVICA®, particularly in patients with cardiac risk factors, acute infections, and a previous history of atrial fibrillation. Periodically monitor patients clinically for atrial fibrillation. Patients who develop arrhythmic symptoms (eg, palpitations, lightheadedness) or new-onset dyspnea should have an ECG performed. If atrial fibrillation persists, consider the risks and benefits of IMBRUVICA® treatment and dose modification.

Second Primary Malignancies - Other malignancies (range, 5 to 10%) including carcinomas (range, 1 to 3%) have occurred in patients treated with IMBRUVICA®. The most frequent second primary malignancy was non-melanoma skin cancer (range, 4 to 8%).

Embryo-Fetal Toxicity - Based on findings in animals, IMBRUVICA® can cause fetal harm when administered to a pregnant woman. Advise women to avoid becoming pregnant while taking IMBRUVICA®. If this drug is used during pregnancy or if the patient becomes pregnant while taking this drug, the patient should be apprised of the potential hazard to a fetus.

ADVERSE REACTIONS

The most common adverse reactions (>20%) in the clinical trials were thrombocytopenia (56%), neutropenia (51%), diarrhea (51%), anemia (37%), fatigue (28%), musculoskeletal pain (28%), upper respiratory tract infection (28%), rash (26%), nausea (25%), and pyrexia (24%). Approximately 5% of patients receiving IMBRUVICA® discontinued treatment due to adverse events. These included infections, subdural hematomas, and diarrhea. Adverse events leading to dose reduction occurred in approximately 6% of patients.

DRUG INTERACTIONS

CYP3A Inhibitors - Avoid concomitant administration with strong or moderate inhibitors of CYP3A. If a moderate CYP3A inhibitor must be used, reduce the IMBRUVICA® dose.

CYP3A Inducers - Avoid co-administration with strong CYP3A inducers.
SPECIFIC POPULATIONS

Hepatic Impairment - Avoid use in patients with baseline hepatic impairment.

Please see full prescribing information: http://www.imbruvica.com/downloads/Prescribing_Information.pdf

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.

* Dr. Byrd serves as national principal investigator of this Pharmacyclics-sponsored clinical study. He has served as an unpaid advisor to both Pharmacyclics and Janssen in developing the compound ibrutinib. Dr. Byrd does not have a financial interest in either company.

Contacts:

Investors
Ramses Erdtmann
Phone: 408-215-3325

Media
Samina Bari
Phone: 408-215-3169

Physicians
U.S. Medical Information
877-877-3536

IMBRUVICA is a registered trademark of Pharmacyclics, Inc.

SOURCE Pharmacyclics, Inc.

News Provided by Acquire Media