



## **Pharmacyclics® Announces Updated Results for BTK Inhibitor PCI-32765 for the Treatment of Relapsed or Refractory Chronic Lymphocytic Leukemia/Small Lymphocytic Lymphoma at American Society for Hematology (ASH) Annual Meeting**

### **Investigational agent PCI-32765 demonstrates prolonged progression free survival (PFS) in patients with advanced CLL/SLL**

SAN DIEGO, Dec. 13, 2011 /PRNewswire/ -- Pharmacyclics, Inc (Nasdaq: PCYC), a biopharmaceutical company focused on developing and commercializing innovative small molecule drugs for the treatment of cancer and immune mediated diseases, announced today updated results of a Phase Ib/II trial of the selective, irreversible Bruton's tyrosine kinase (BTK) inhibitor, PCI-32765, for the treatment of patients with relapsed or refractory chronic lymphocytic leukemia (CLL)/small lymphocytic lymphoma (SLL). The longer-term follow-up results of a previously reported multicenter Phase Ib/II trial (Byrd, ASCO 2011) presented today at the ASH Annual Meeting in San Diego, CA by Dr. Susan O'Brien, Professor of Medicine in the Department of Leukemia of the University of Texas MD Anderson Cancer Center, continue to demonstrate PCI-32765 as a highly active treatment in patients with relapsed or refractory CLL.

The trial included a total of 61 patients with relapsed or refractory CLL/SLL enrolled at two dose levels, 420 mg (n=27) or 840 mg (n=34). Oral PCI-32765 was administered daily until disease progression. Data was available from a landmark analysis of 12 months. With a median follow-up of 12.6 months in the 420mg cohort and 9.3 months in the 840mg cohort, the overall response rate (ORR), including PR and CR, for the 420mg dose level was 67% and for the 840 mg dose 68%, as measured by the 2008 International Workshop on Chronic Lymphocytic Leukemia criteria. The responses have been independent of high-risk clinical or genetic features. The estimated 12 month PFS for the pooled cohorts was 86%. The safety profile of PCI-32765 was particularly notable for minimal off target toxicities. The most common treatment related adverse events reported in the trial were Grade 1 (mild) or 2 (moderate) diarrhea, cough, fatigue, and upper respiratory infections, and only 2 patients have discontinued study treatment due to adverse events. Overall, these data support Phase III evaluation of PCI-32765 as a single agent in relapsed or refractory CLL/SLL.

"Our longer-term follow-up of an earlier analysis of this study clearly demonstrates encouraging results for relapsed or refractory CLL/SLL patients treated with PCI-32765," said Dr. Susan O'Brien, Professor in the Department of Leukemia at The University of Texas MD Anderson Cancer Center in Houston, who presented the study results. "We look forward to seeing further evaluation of this potent therapy in CLL/SLL patients."

#### **Conference Call Details**

The Company will be holding a conference call on Wednesday December 14, 2011 at 4:30pm ET. To participate in the conference call, please dial 1-877-407-8133 for domestic callers and 1-201-689-8040 for international callers. To access the live audio broadcast or the subsequent archived recording, log on to <http://ir.pharmacyclics.com/events.cfm>. The archived version of the webcast will be available for 30 days on the Investor Relations section of the company's Web site at [www.pharmacyclics.com](http://www.pharmacyclics.com).

#### **About PCI-32765**

PCI-32765 is a first-in-class oral, selective, irreversible Bruton's tyrosine kinase (BTK) inhibitor that is being investigated in clinical trials for its potential in treating patients with B-cell malignancies.

As of December 1st, 2011, 370 patients have been enrolled into clinical trials evaluating PCI-32765. Pharmacyclics' ongoing clinical development program includes the following trials:

- PCYC-04753: A Phase I of PCI-32765 in patients with recurrent B cell malignancies. This study is designed to assess the safety and tolerability of PCI-32765. Patient enrollment is completed with 66 patients enrolled in the main portion of the study. Enrollment into the diffuse large B-cell lymphoma (DLBCL) activated B-cell (ABC) investigator-led cohort is still ongoing.
- PCYC-1102-CA: A multicentre, open-label, single agent Phase Ib/II study of PCI-32765 in subjects with relapsed or refractory chronic lymphocytic leukemia (CLL) or small lymphocytic lymphoma (SLL). This study is designed to assess safety, tolerability, and efficacy of PCI-32765. Patient enrollment is complete, with 117 patients enrolled.

- PCYC-1104-CA: A Phase II, international, multicenter, single agent study of PCI-32765 evaluating efficacy, safety and tolerability in two patient populations with relapsed or refractory mantle cell lymphoma (MCL). This trial is active in several US and EU sites and has enrolled 82 patients. The company expects to complete enrollment from this study in Q1 2012.
- PCYC-1106-CA: A multicenter, open-label, Phase II study of PCI 32765 in subjects with relapsed or refractory DLBCL. This study is designed to assess the activity of PCI-32765 in two genetically distinct subtypes of DLBCL, the ABC subtype and the germinal center (GC) subtype. This trial is active in several US sites and has enrolled 27 patients. The company expects to complete enrollment by calendar Q1 2012.
- PCYC-1108-CA: A Phase Ib, multicenter, open-label study of PCI 32765, in combination with intensive immune chemotherapy (FCR = fludarabine, cyclophosphamide and rituximab; or BR = bendamustine and rituximab) in subjects with CLL/SLL. This trial is active in several US sites and enrollment is completed with 33 patients.
- PCYC-1109-CA: A Phase Ib/II study of PCI-32765 in combination with ofatumumab in subjects with relapsed or refractory CLL /SLL is ongoing and has enrolled 47 patients between two scheduled cohorts of the combination.
- PCYC-1111-CA: A Phase II study of PCI-32765 in subjects with relapsed or relapsed/refractory multiple myeloma (MM). Pre-clinical studies, both internally as well as through external collaborations, have suggested a potentially vital role for BTK in both malignant plasma cells and osteoclasts, which are involved in the bone complications of this disease. This Phase II trial of PCI-32765 is on track to start in Q1 2012.

## About Pharmacyclics

Pharmacyclics® is a clinical-stage 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 promising product candidates based on scientific development expertise, develop our products in a rapid, cost-efficient manner and pursue commercialization and/or development partners when and where appropriate.

Presently, Pharmacyclics has three product candidates in clinical development and several preclinical molecules in lead optimization. We are committed to high standards of ethics, scientific rigor, and operational efficiency as we move each of these programs to viable commercialization.

The Company is headquartered in Sunnyvale, California and is listed on NASDAQ under the symbol PCYC. To learn more about how Pharmacyclics advances science to improve human healthcare visit us at <http://www.pharmacyclics.com>.

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