

HedgePath Pharmaceuticals Completes Enrollment for its Phase 2(b) Trial in BCCNS and Provides Trial Update

Positive outcomes continue to be observed in open label trial; Company will lock database to complete clinical study report in anticipation of Pre-NDA Meeting with FDA

FOR IMMEDIATE RELEASE -- TAMPA, FLORIDA (October 30, 2017) – HedgePath

Pharmaceuticals, Inc. (OTCQX:HPPI), a clinical stage biopharmaceutical company that discovers, develops and plans to commercialize innovative therapeutics for patients with cancer, announced today that it has completed enrollment in its previously announced open label, Phase 2(b) SCORING clinical trial, testing SUBA™ - Itraconazole in patients with BCCNS (Basal Cell Carcinoma Nevus Syndrome, also known as Gorlin Syndrome). The company also provided an update on the trial and the company's pathway forward towards potential regulatory filing.

BCCNS is a rare disorder caused by a genetic mutation that most frequently causes the continual growth of cancerous lesions on the skin. Although BCCNS tumors are typically not life-threatening, their control is extremely important to the welfare of patients whose only approved alternative is repeated surgical excision, with its attendant cost, time away from normal activities such as work, and challenging aesthetic outcomes. Facial tumors, in particular, are excised in an attempt to minimize the resultant defect and risk of scarring. However, because BCCNS causes many tumors to occur on a continual basis, the end result over an extended period of time is a high risk of disfigurement.

HPPI is studying the ability of SUBA-Itraconazole to inhibit the "Hedgehog Pathway" in BCCNS patients. As a major regulator of cellular processes in vertebrates, including cell differentiation, tissue polarity and cell proliferation, the Hedgehog Pathway, when inhibited, could result in delay or possibly prevent the development of certain cancers, such as basal cell carcinoma (BCC). Each of 38 patients in the trial was required to have at least 10 surgically eligible BCC tumors at the time of enrollment, as well as a history of prior BCC surgical excisions. Surgically eligible "target tumors" were of a size and location on the body such that, upon entry into the trial, surgery was an option based on standard of care.

Nicholas Virca, HPPI's President and Chief Executive Officer, stated that, "In order to ascertain the clinical impact of our therapy, the patients we enrolled had an average of 195 basal cell carcinoma tumors which had been removed by prior surgeries. The primary endpoint for our study is to measure how target tumors respond to twice daily dosing of SUBA-Itraconazole oral capsules and document the duration of tumor responses over time as well as overall safety. As of this reporting, the median time on study across all our patients is 38 weeks, with 32 patients having been dosed for 16 or more weeks and 13 patients exceeding one year on therapy."

As an open label study, HPPI has been able to follow and report the progress of this trial, and the impact of therapy has been impressive, with only one target tumor requiring surgical excision and 99% of target tumors being controlled. To date, HPPI has measured the response of 467 individual target tumors, with 54% exhibiting a 30% or greater reduction in size since dosing began and 28% completely disappearing. Approximately 60% of responding lesions have continued to respond during ongoing treatment with a duration of response yet to be determined, but currently exceeding one year at this point in time. The patient dropout rate has been only 11%, with SUBA-Itraconazole being well-tolerated with no reports of hair loss, loss of taste, or disabling muscle cramps typical of other hedgehog inhibitors. In addition, no serious adverse events attributable to SUBA-Itraconazole have been reported.

Based on the results above and the company's on-going communications with the U.S. Food and Drug Administration (FDA), HPPI believes the data from this trial will support the filing of a New Drug Application (NDA) for the management of basal cell carcinomas in BCCNS. In order to reach this objective, HPPI has begun the process of locking the study database, which the company intends to achieve by year-end, with the goal to complete a Clinical Study Report during the first quarter of 2018 while at the same time preparing for submission of a pre-NDA meeting request to FDA.

While the clinical data observed to date appear to be predictive of the desired final study results while HPPI seeks further guidance from FDA, readers are cautioned that no assurances can be given that (i) the final study results will match these latest results or (ii) the study, when and if completed, will achieve its primary and secondary endpoints or (iii) that the study will be found by FDA to be sufficient for the filing of an NDA or (iv) if an NDA is filed, that it will be approved by FDA. Further, HPPI is not committing to providing further interim updates prior to the reporting of the final study results.

Targeting the Hedgehog Pathway

Advances in the genomic analysis of cancers have led to the discovery that one molecular pathway, in particular, plays a critical role in the initiation, growth, and metastatic spread of cancer. That pathway is the hedgehog pathway. Its unusual name derives from the resemblance of fruit fly larvae to miniature hedgehogs when the pathway was blocked. HPPI is undertaking the clinical development of the first and only hedgehog inhibitor that, to the best of the company's knowledge, promises to be efficacious while also being extremely well tolerated in human testing. It therefore appears to be well-suited for chronic suppression of this important molecular pathway.

The hedgehog pathway may well be the single most important molecular pathway in oncology. There have been outstanding outcomes in physician-sponsored clinical trials using itraconazole in stage IV lung cancer and in metastatic castration-resistant prostate cancer that have been reported in peer reviewed journals. Now that the FDA has granted its first "molecular indication" for a drug such as Keytruda® (pembrolizumab), which is indicated based upon a tumor's biomarker that can be present without regard to the tumor's location, HPPI believes that SUBA-Itraconazole is an ideal candidate for an eventual FDA approval based on a "molecular indication", in this case, the inhibition of the hedgehog pathway, as opposed to the traditional, narrower, cancer-specific approvals.

About SUBA-Itraconazole

HPPI's lead drug candidate, SUBA-Itraconazole, is a patent-protected formulation of itraconazole, an approved oral antifungal drug that has been in use for over 25 years. HPPI is the exclusive U.S. licensee (through Mayne Pharma, the majority stockholder of HPPI) of SUBA-Itraconazole for the treatment of cancer. Prior to research at Johns Hopkins University, itraconazole was not known to have any target in mammalian cells. Investigators at Johns Hopkins discovered that itraconazole inhibits the hedgehog pathway by binding to a surface receptor in the pathway called Smoothened. Unlike generic itraconazole, that has poor and unpredictable bioavailability, SUBA-Itraconazole can be dosed at half the level of the generic formulation due to its superior bioavailability, which exceeds 90%. As such, HPPI believes that generic itraconazole cannot be substituted for SUBA-Itraconazole.

About BCCNS

HPPI's initial indication is for the orphan disease BCCNS. SUBA-Itraconazole has qualified under the FDA's Orphan Drug Designation Program as a potential therapy for BCCNS.

There is no approved pharmaceutical therapy for this familial cancer syndrome. There are estimated to be 10,000 patients in the U.S. with BCCNS. This is an autosomal dominantly inherited defect in the hedgehog pathway that causes the pathway to be up-regulated, resulting in hundreds or even thousands of basal cell carcinomas developing over the lifetime of the affected patients. In many types of cancers, the hedgehog pathway is basically hijacked by the cancer cells to assist their growth and metastatic spread, but in the case of basal cell carcinomas, whether in this hereditary syndrome or in the much more common, sporadic basal cell carcinomas, the hedgehog pathway has a mutation that makes it the sole driver of the development of BCC tumors. Inhibition of the pathway, then, can inhibit the appearance of new tumors, shrink existing tumors, and even cause some tumors to disappear altogether.

About HedgePath Pharmaceuticals

HedgePath Pharmaceuticals, Inc. (OTCQX:HPPI) is a clinical stage biopharmaceutical company that is seeking to repurpose the FDA approved antifungal pharmaceutical itraconazole as a potential treatment for cancer. HPPI is the exclusive U.S. licensee of a patented formulation of itraconazole, called SUBA-Itraconazole, which clinical studies have shown to have greater bioavailability than generic itraconazole.

The Hedgehog signaling pathway is a major regulator of cellular processes in vertebrates, including cell differentiation, tissue polarity and cell proliferation. Based on published research, HPPI believes that inhibiting the Hedgehog pathway could delay or possibly prevent the development of certain cancers in humans. Leveraging research undertaken by key investigators in the field, HPPI is exploring the effectiveness of SUBA-Itraconazole as an anti-cancer agent and to pursue its potential commercialization. HPPI is headquartered in Tampa, Florida. For more information, please visit www.hedgepathpharma.com. Cautionary Note Regarding Forward Looking Statements

This press release and any statements of representatives and partners of HedgePath Pharmaceuticals, Inc. (the "Company") related thereto contain, or may contain, among other things, certain "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements involve significant risks and uncertainties. Such statements may include, without limitation, statements with respect to the Company's plans, objectives, projections, expectations and intentions and other statements identified by words such as "projects," "may," "will," "could," "would," "should," "believes," "expects," "anticipates," "estimates," "intends," "plans," "potential" or similar expressions. These statements are based

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