Inhibitor Investor Room

HedgePath Pharmaceuticals Receives IND Clearance to Commence Development of its Proposed Cancer Treatment

HPPI to commence Phase IIb study in first quarter 2015 of SUBATM-Itraconazole for treatment of basal cell carcinoma in patients with basal cell carcinoma nevus syndrome

FOR IMMEDIATE RELEASE - TAMPA, FLORIDA and SAN DIEGO, CALIFORNIA – December 16, 2014 – HedgePath Pharmaceuticals, Inc. (OTCQB:HPPI), a clinical stage biopharmaceutical company that discovers, develops and plans to commercialize innovative therapeutics for patients with cancer, announced today that it received clearance of its first Investigational New Drug (IND) Application from the U.S. Food and Drug Administration (FDA).

The IND covers HPPI's clinical development plan for a patented formulation of itraconazole (known as SUBA-Itraconazole) with improved bioavailability to study the treatment of basal cell carcinoma (BCC) in patients with basal cell carcinoma nevus syndrome (BCCNS), also known as Gorlin's syndrome, an orphan disease with no approved pharmaceutical therapy. HPPI holds the exclusive U.S. license rights to SUBA-Itraconazole for the treatment of cancer, and this is the first cancer indication the company is seeking to develop.

Nicholas J. Virca, HPPI's President and Chief Executive Officer, commented, "We are very gratified and proud to have achieved FDA clearance of our first IND, under which we will seek to repurpose SUBA-Itraconazole as a candidate for anti-cancer therapy in humans. We now hope to establish a track record of achieving positive milestones as we progress this product through clinical development."

BCCNS results from a genetic mutation which causes the Hedgehog pathway (a major regulator of processes in cells) to function improperly, leading to the chronic formation of basal cell tumors, including potentially disfiguring lesions on the face. Industry sources estimate that there are approximately 10,000 patients in the United States with BCCNS, which could potentially qualify SUBA-Itraconazole under the FDA's Orphan Drug Designation Program for treatment of BCC in this patient population.

The trial design cleared by FDA is a single arm, Phase IIb, multi-center, open-label, nonplacebo-controlled study expected to involve the recruitment of 40 patients beginning in January 2015. The primary endpoint of this study will be the objective response rate of BCC lesions to SUBA-Itraconazole in subjects with BCCNS, under which a meaningful response will be defined as a 30% or greater reduction in the cumulative size of target tumors, with an appropriate sample size to be 33 of the 40 patients in order to maintain a 90% power. HPPI anticipates that if there is evidence of substantial clinical benefit to these patients, the company would seek to have further discussions with FDA as to whether results from this trial could potentially serve as the basis for a New Drug Application submission to FDA for this product candidate.

"We believe BCCNS provides us with an ideal first opportunity to test itraconazole's anti-cancer potential," continued Mr. Virca. "There is a relatively well-defined molecular mechanism associated with BCCNS that causes patients to suffer from multiple, chronically recurring tumors which can be debilitating and often require disfiguring surgeries to remove cancerous lesions.

Existing topical therapies have proven suboptimal due to lack of efficacy, and the only approved oral therapy for BCC is approved for irresectable or locally metastatic basal cell carcinomas and it has a significant side-effect profile. In our trial, the initial dosing period to study the primary endpoint of tumor response rate will be 16 weeks, followed by an additional 24 weeks to collect ongoing efficacy and safety data. We anticipate this timeline will enable us to continue our interactions with FDA regarding assessment of clinically meaningful responses in BCCNS patients. In short, this IND clearance lays out a defined regulatory pathway for us, as we move forward next year to develop what we believe will be an effective treatment for this underserved patient population."

About HedgePath Pharmaceuticals

HedgePath Pharmaceuticals, Inc. 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 plans to explore the effectiveness of SUBA-Itraconazole as an anti-cancer agent and to pursue its potential commercialization. HPPI has offices in Tampa, Florida and San Diego, California. For more information, please visit www.hedgepathpharma.com.

About SUBA-Itraconazole

SUBA-Itraconazole is a proprietary itraconazole formulation that enhances the absorption of itraconazole to improve the bioavailability of orally administered drugs that are poorly soluble. SUBA-Itraconazole was developed to improve absorption and significantly reduce variability compared to generic itraconazole. These benefits enable a more predictable clinical response and a reduction in the active drug quantity to deliver the required therapeutic blood levels. SUBA is a trade mark of Mayne Pharma International Pty Ltd.

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 upon the current beliefs and expectations of the

Company's management and are subject to significant risks and uncertainties, including those detailed in the Company's filings with the Securities and Exchange Commission. Actual results (including, without limitation, the actual timing for, or actual results of, the Company’s clinical trial described herein or the FDA’s review of such results) may differ significantly from those set forth or implied in the forward-looking statements. These forward-looking statements involve numerous risks and uncertainties that are subject to change based on various factors (many of which are beyond the Company's control). The Company undertakes no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law.

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