Inhibitor Investor Room

HedgePath Pharmaceuticals Receives Clarity From FDA Regarding Pathway to Potential Regulatory Submission

HPPI to proceed with streamlined 505(b)(2) regulatory pathway for SUBA™-Itraconazole as a treatment for Basal Cell Carcinoma Nevus Syndrome

FDA provides written guidance on data from HPPI's ongoing clinicial trial and the data requirements for NDA filing and potential FDA approval

FOR IMMEDIATE RELEASE -- TAMPA, FLORIDA (July 25, 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 that the U.S. Food and Drug Administration (FDA) has provided further guidance regarding HPPI's ongoing, open-label Phase 2(b) clinical trial studying the effect of SUBA-Itraconazole (SUBA-Cap) oral capsules in patients with Basal Cell Carcinoma Nevus Syndrome (BCCNS), also known as Gorlin Syndrome.

The FDA's guidance came in the form of a written response by FDA to HPPI's Type-C meeting background package. Such a meeting is a standard element of the regulatory review process leading to a potential New Drug Application (NDA) to FDA.

Nicholas Virca, President and CEO of HPPI, stated that, "We are pleased with the FDA's guidance, since we believe it adds clarity to our regulatory and clinical road going forward for the BCCNS indication of SUBA-Cap. FDA confirmed that we may follow the more streamlined 505(b)(2) regulatory pathway, which will allow us to reference safety data from previous third-party itraconazole trials, to be supplemented by our own safety database. The acceptability of this combined safety database will then be determined by the FDA during the course of its review of the future NDA. FDA also agreed that no additional nonclinical toxicology studies appear necessary to support filing an NDA for SUBA-Itraconazole under the 505(b)(2) pathway."

Importantly, FDA also indicated that it "[w]ould accept a single study to support an NDA if results show a significant effect on a clinically meaningful endpoint. The results of the single trial must be sufficiently robust and so compelling that it would be unethical to repeat the study . . . [e]vidence of an objective reduction in tumor burden that is durable is important in order to demonstrate antitumor effects of SUBA-Itraconazole in patients with BCCNS and these data should be collected and independently reviewed."

Mr Virca further stated that, "In light of FDA's additional guidance on what might constitute a clinically significant response, we are now undertaking further detailed analyses of individual tumor responses from our ongoing trial seeking to verify the robustness of our therapy in reducing the tumour burden in BCCNS patients. We intend to present the results of this additional analysis to FDA and continue discussions with them about the utility of such results in a potential NDA submission."

Readers are cautioned that no assurances can be given that (i) the final study results will match the results previously reported on May 30, 2017 or (ii) the study, when and if completed, will achieve its primary and secondary endpoints or (iii) that the study results will be found by FDA to be sufficient for the filing of a NDA, or that one or more additional studies will not be required 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.

About BCCNS

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 has qualified SUBAItraconazole under the FDA's Orphan Drug Designation Program.

About SUBA-Itraconazole

SUBA-Itraconazole is a patented and proprietary itraconazole formulation that enhances the absorption of itraconazole to improve the bioavailability of orally administered drugs that are poorly soluble. The U.S. rights to SUBA-Itraconazole for the treatment of cancer are exclusively licensed to HPPI by an affiliate of Mayne Pharma Group Limited. SUBA-Itraconazole was developed to improve absorption and significantly reduce variability compared to generic itraconazole. These benefits provide enhancements to patients and prescribers with reduced intra- and inter-patient variability, enabling a more predictable clinical response and a reduction in the active drug quantity to deliver the required therapeutic blood levels.

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 plans to explore 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 "forwardlooking 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 any related New Drug Application by the Company) may differ significantly from those set forth or implied in the forwardlooking statements (and may further differ from the interim study results described herein). 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.

For more information:

Nicholas J. Virca, President and CEO

nvirca@hedgepathpharma.com

Investor Relations Contact:

Garrison Hasara, CFO and Treasurer

ghasara@hedgepathpharma.com

Investor Relations Contact:

Garrison Hasara, CFO and Treasurer

ghasara@hedgepathpharma.com

© 2017 HedgePath Pharmaceuticals, Inc. All rights reserved.

https://investors.inhibitortx.com/2017-07-25-HedgePath-Pharmaceuticals-Receives-Clarity-From-FDA-Regarding-Pathway-to-Potential-Regulatory-Submission