NEWS RELEASE

pellepharm initiates phase 2 clinical trial of patidegib topical gel for people with high frequency basal cell carcinoma

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SAN FRANCISCO--(BUSINESS WIRE)--Jan. 8, 2020-- PellePharm, Inc., a BridgeBio Pharma, Inc. (Nasdaq: BBIO) company, today announced it has dosed the first two participants in a Phase 2 clinical trial of Patidegib Topical Gel, 2%, vs. vehicle gel for people with non-Gorlin High Frequency Basal Cell Carcinoma (HF-BCC). HF-BCC is a rare disease that causes a higher than average number of BCCs to develop, specifically in the facial area. PellePharm is a late clinical-stage biopharmaceutical company committed to targeting rare forms of basal cell carcinoma (BCC).

“There are approximately 35,000 people with HF-BCC in the United States. Their quality of life is significantly altered due to the multiple, invasive surgeries they must undergo during their treatment process,” said Sanuj K. Ravindran, M.D., president and chief executive officer of PellePharm. “Our goal is to provide people living with HF-BCC better, non-surgical options, and we are pleased to have initiated our multicenter Phase 2 trial to further evaluate Patidegib Topical Gel.”

The randomized, double-blinded, stratified, vehicle-controlled Phase 2 trial is evaluating the safety and efficacy of Patidegib Topical Gel 2% applied twice daily to the face over nine months vs. vehicle gel. The primary endpoint of the study is the number of surgically eligible basal cell carcinoma (nSEB) that develop on the face of participants over the nine-month period. The primary endpoint will be assessed by imaging and tracking of BCCs consistently throughout the study in order to identify nSEBs, consistent with the methods employed in the ongoing Phase 3 study of Patidegib topical gel for people living with Gorlin Syndrome. Approximately 40 participants will be enrolled in the Phase 2 trial.

“People with non-Gorlin HF-BCC are phenotypically similar to people with Gorlin Syndrome with respect to their BCCs, but are not born with a germline PTCH1 mutation. Those with HF-BCC are faced with the challenge of frequent BCC surgeries, which can be debilitating, painful and disfiguring, particularly to the face,” said Srikanth Pendyala, M.D., vice president of clinical development at PellePharm. “Due to the success we have found with enrolling our Gorlin Syndrome Phase 3 pivotal trial, we are thrilled about this important Phase 2 study milestone, and hope that by initiating this trial, we are closer to being able to mitigate the significant burden of frequent surgeries for those living with HF-BCC.”

PellePharm recently completed enrollment for its Phase 3 clinical trial of Patidegib Topical Gel 2% for people living with Gorlin Syndrome. PellePharm entered into a strategic collaboration with LEO Pharma in November 2018, which includes an option for LEO Pharma to acquire PellePharm.

About Patidegib

Patidegib Topical Gel, an investigational treatment, is designed to reduce the BCC tumor burden in people living with Gorlin Syndrome and High Frequency BCC (HF-BCC) by blocking the disease at its source within the hedgehog signaling pathway. Patidegib Topical Gel has shown early promise in a Phase 2 clinical study for the mitigation of BCC tumors in Gorlin Syndrome. The topical formulation of Patidegib was developed with a goal of providing the clinical activity previously demonstrated by oral Patidegib in Phase 1 trials and a favorable tolerability profile without the adverse systemic side effects observed with the oral class of hedgehog inhibitors. The topical gel formulation is stable at room temperature for at least two years, potentially making it an option for ongoing, at-home management of Gorlin Syndrome and HF-BCC. PellePharm has received both Orphan Drug Designation and Breakthrough Therapy Designation for Patidegib Topical Gel in Gorlin Syndrome from the FDA, as well as Orphan Drug Designation in Gorlin Syndrome from EMA’s Committee for Orphan Medicinal Products in the EU.

About Gorlin Syndrome

Gorlin Syndrome is a rare, genetic disease characterized by constitutional, heritable mutations in one allele of the tumor suppressor gene encoding PATCHED1 (PTCH1), which acts as the primary inhibitor of the hedgehog signaling pathway. This leads to the formation of multiple basal cell carcinomas (BCCs), often on the face.

With no FDA-approved drugs available for people living with Gorlin Syndrome, the standard of care for treating BCCs is surgery. People with severe Gorlin Syndrome may have as many as 30 surgeries per year, which can be repetitive, scarring and disfiguring. Approximately 10,000 people in the United States, or one in 31,000, are believed to be affected by Gorlin Syndrome. Gorlin Syndrome is known by several names, including Gorlin-Goltz Syndrome, Basal Cell Nevus Syndrome (BCNS) and Nevoid Basal Cell Carcinoma Syndrome (NBCCS).

About High Frequency Basal Cell Carcinoma (HF-BCC)

HF-BCC, like Gorlin Syndrome, is a rare disease which is characterized by the development of an abnormally high number of basal cell carcinomas (BCCs). Unlike people with Gorlin Syndrome, people with HF-BCC are not born with a germline PTCH1 mutation and do not suffer from the other systemic manifestations of Gorlin Syndrome. The current standard of care for people living with HF-BCC is BCC surgery.

About PellePharm

Founded by world leaders in hedgehog pathway signaling, PellePharm, a BridgeBio company, is committed to targeting rare forms of basal cell carcinoma, including Gorlin Syndrome and High Frequency Basal Cell Carcinoma (HF-BCC), at their source. PellePharm's mission is to improve the quality of life for those suffering from Gorlin Syndrome and HF-BCC by providing an easy-to-use topical gel that could potentially reduce the need for regular, painful and disfiguring surgeries. Patidegib topical gel is a topical formulation of a proprietary hedgehog inhibitor.

About LEO Pharma

LEO Pharma A/S helps people achieve healthy skin. The company is a leader in medical dermatology with a robust R&D pipeline, a wide range of therapies and a pioneering spirit. Founded in 1908 and owned by the LEO Foundation, LEO Pharma has devoted decades of research and development to advance the science of dermatology, setting new standards of care for people with skin conditions. LEO Pharma is headquartered in Denmark with a global team of 6,000 people, serving 76 million patients in 130 countries. In 2018, the company generated net sales of DKK 10,410 million.

About BridgeBio Pharma

BridgeBio is a team of experienced drug discoverers, developers and innovators working to create life-altering medicines that target well-characterized genetic diseases at their source. BridgeBio was founded in 2015 to identify and advance transformative medicines to treat patients who suffer from Mendelian diseases, which are diseases that arise from defects in a single gene, and cancers with clear genetic drivers. BridgeBio's pipeline of over 15 development programs includes product candidates ranging from early discovery to late-stage development.

BridgeBio Pharma Forward-Looking Statements

This press release contains forward-looking statements. Statements we make in this press release may include statements that are not historical facts and are considered forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), which are usually identified by the use of words such as "anticipates," "believes," "estimates," "expects," "intends," "may," "plans," "projects," "seeks," "should," "will," and variations of such words or similar expressions. We intend these forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act and Section 21E of the Exchange Act and are making this statement for purposes of complying with those safe harbor provisions. These forward-looking

statements, including statements relating to expectations, plans and prospects regarding PellePharm's clinical development plans, clinical trial designs, the clinical and therapeutic potential of Patidegib Topical Gel, the results of clinical trials, the funding by LEO Pharma of PellePharm's pivotal Phase 3 trial of Patidegib Topical Gel in people living with Gorlin Syndrome, PellePharm's Phase 2 clinical trial of Patidegib Topical Gel 2% in people living with High Frequency Basal Cell Carcinoma, enrollment of PellePharm's Phase 2 clinical trial, and the timing of these events, reflect our current views about our plans, intentions, expectations, strategies and prospects, which are based on the information currently available to us and on assumptions we have made. Although we believe that our plans, intentions, expectations, strategies and prospects as reflected in or suggested by those forward-looking statements are reasonable, we can give no assurance that the plans, intentions, expectations or strategies will be attained or achieved. Furthermore, actual results may differ materially from those described in the forward-looking statements and will be affected by a number of risks, uncertainties and assumptions, including, but not limited to, PellePharm's ability to enroll participants in and generate data from the ongoing Phase 2 and Phase 3 trials, the benefits that will be derived from these trials, the progress and results of the trials, PellePharm's ability to advance Patidegib Topical Gel in clinical development in accordance with its plans, the timing of these events, and PellePharm's ability to maintain its collaboration with LEO Pharma, as well as those risks set forth in the Risk Factors section of BridgeBio Pharma Inc.'s most recent Quarterly Report on Form 10-Q and our other SEC filings. Moreover, PellePharm operates in a very competitive and rapidly changing environment in which new risks emerge from time to time. These forward-looking statements are based upon the current expectations and beliefs of PellePharm's management as of the date of this release, and are subject to certain risks and uncertainties that could cause actual results to differ materially from those described in the forward-looking statements. . Except as required by applicable law, we assume no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

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Source: PellePharm, Inc.

Lauren Barbiero
W2O pure
(646) 564-2156
lbarbiero@w2ogroup.com