October 29, 2020

PellePharm to Highlight Clinical Programs at the 29th Congress of the European Academy of Dermatology and Venereology

SAN FRANCISCO–PellePharm, Inc., a BridgeBio Pharma, Inc. company, today announced the presentation of various data highlighting product candidate patidegib topical gel for adults with Gorlin syndrome and high-frequency basal cell carcinoma (HF-BCC). The presentations will be made during the 29th Congress of the European Academy of Dermatology and Venereology (EADV), to be held virtually October 29 – 31, 2020.

Patidegib topical gel is in development to reduce the large volume of basal cell carcinoma (BCC) tumors that characterize Gorlin syndrome and HF-BCC, at their source, by inhibiting the hedgehog signaling pathway. It is designed to block the SMO signal, thus turning off oncogenic hedgehog activity. Unlike other hedgehog inhibitors, patidegib has been formulated into a topical gel that is stable at room temperature for use as a chronic therapy.

“For rare diseases such as Gorlin syndrome and HF-BCC, with symptoms that manifest on the skin and require treatments that are either invasive or poorly tolerated, we believe that a topical therapeutic designed to both reduce BCC burden and minimize systemic exposure and thereby side effects is ideal for this vulnerable patient population,” said Sanuj K. Ravindran, M.D., president and chief executive officer of PellePharm. “Our research continues to generate compelling evidence that supports patidegib as a clinical candidate and validates our approach in hedgehog inhibition.”

Details for the poster presentations at EADV Virtual are as follows:

Poster Title: A Phase 1, Single-center, Pharmacokinetic, Safety and Tolerability Study of Patidegib Topical Gel in Healthy Adult Volunteers Under Maximum Use Conditions
Date & Time: Thursday, October 29, 2020, 12:00 p.m. CET
Category: Cutaneous Oncology

Poster Title: A Proof of Concept, Phase 2 Study to Assess Efficacy and Safety of Patidegib for Reduction of Disease Burden of Persistently Developing Basal Cell Carcinomas (BCCs) in Non-Gorlin High-Frequency BCC Patients
Date & Time: Thursday, October 29, 2020, 12:00 p.m. CET
Category: Cutaneous Oncology

Poster Title: A Phase 3 Pivotal Study to Assess Efficacy and Safety of Patidegib for Reduction of Disease Burden of Persistently Developing Basal Cell Carcinomas in Patients With Gorlin Syndrome
Date & Time: Thursday, October 29, 2020, 12:00 p.m. CET
Category: Cutaneous Oncology

Poster Title: Gorlin Syndrome: It's the Pits*
Date & Time: Thursday, October 29, 2020, 12:00 p.m. CET
Category: Cutaneous Oncology
*This poster is presented by Julie Breneiser on behalf of GSA (Gorlin Syndrome Alliance).

"We are encouraged by the continuing promise that patidegib is demonstrating in the clinic. A topical formulation has the potential to bring significant benefit to the Gorlin Syndrome patient and for patients with HF-BCC, helping manage the burden of the diseases with convenient self-administration at home," said Patrice Baudry, vice president of rare disease at LEO Pharma.

In November 2018, PellePharm and LEO Pharma entered into a strategic collaboration to address the unmet medical needs for rare skin conditions, such as Gorlin syndrome and HF-BCC. LEO Pharma is providing resources to PellePharm to fund, among other activities, its Phase 3 trial of patidegib topical gel 2% in patients with Gorlin syndrome under the terms of the agreement.

About Patidegib

Patidegib topical gel, an investigational treatment, is designed to reduce the basal cell carcinoma (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 BridgeBio Pharma

BridgeBio Pharma 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 20 development programs includes product candidates ranging from early discovery to late-stage development. For more information visit www.bridgebio.com.

Forward-Looking Statements

This press release contains forward-looking statements, including statements relating to the expectations, plans, and prospects for PellePharm's product candidate, patidegib topical gel, and its potential use as a chronic therapy for adults with Gorlin syndrome and HF-BCC through hedgehog inhibition, as well as regarding the clinical development plan, clinical trial results, timing and completion of clinical trials for patidegib. Statements in this press release that are not statements of historical fact are considered forward-looking statements within the meaning of Section 27A of 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," "continues", "could", "estimates," "expects," "intends," "may," "plans," "potential", "predicts", "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 are neither forecasts, promises nor guarantees, and are based on the current beliefs of PellePharm's management as well as assumptions made by and information currently available to PellePharm. Such statements reflect the current views of PellePharm with respect to future events and are subject to known and unknown risks, uncertainties and assumptions, including, but not limited to, PellePharm's ability to advance patidegib in clinical development in accordance with its plans, the results from any clinical trials of patidegib, the results from prior clinical trials and nonclinical studies of patidegib not being indicative of the results from future clinical trials and nonclinical studies of patidegib, the nature of PellePharm's interactions with regulatory authorities, and the continuing success of PellePharm's strategic collaboration with LEO Pharma. Moreover, PellePharm operates in a very competitive and rapidly changing environment in which new risks emerge from time to time, and 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. Although PellePharm believes that its plans, intentions, expectations, strategies and prospects as reflected in or suggested by these forward-looking statements are reasonable, PellePharm cannot give any assurance that the plans, intentions, expectations or strategies will be attained or achieved. These forward-looking statements are based upon the current expectations and beliefs of PellePharm's management as of the date of this press 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. We anticipate that subsequent events and developments will cause our views to change. Except as required by law, PellePharm disclaims any intention

or responsibility for updating or revising any forward-looking statements contained in this press release in the event of new information, future developments or otherwise. You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this press release.

Contacts

Media Relations Contact:
Carolyn Hawley
Canale Communications
(619) 849-5382
carolyn.hawley@canalecomm.com