NEWS RELEASE

pellepharm to present topical patidegib and skin cancer epidemiological data at the 7th international investigative dermatology meeting

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SAN FRANCISCO–(**BUSINESS WIRE**)–PellePharm, a late clinical-stage biopharmaceutical company committed to targeting rare dermatologic conditions at their source, announced presentation of new data from PellePharm's clinical and scientific research programs at the International Investigative Dermatology (IID) Meeting in Orlando, FL. The data, being presented in three posters, highlight PellePharm's lead clinical program, patidegib topical gel, in patients with basal cell carcinomas (BCCs) and epidemiological data on sporadic, high frequency BCCs.

"This year at IID, we are pleased to have the opportunity to share our latest clinical experience with patidegib topical gel and continue to be most hopeful about the promise it holds for patients with rare dermatologic diseases," said Sanuj Ravindran, M.D., president and chief executive officer of PellePharm. "Our presentations will accentuate how PellePharm's scientific premise and clinical progress are paving the way for us to deliver much needed hope to these patients with no preventative therapeutic options."

IID Poster Presentations

- Anti-BCC efficacy of a topical Hedgehog inhibitor (HHi) without the systemic HHi adverse effects in BCNS patients in a Phase 2 randomized controlled trial
  Poster #139 — Thursday, May 17, 2018 at 11:45 a.m. – 1:45 p.m. ET

  This study highlights the recent results from PellePharm's Phase 2 trial treating patients with Gorlin

Syndrome. The company first shared topline data from a Phase 2 study conducted in the UK in July 2017 that showed early promise for topical patidegib for the mitigation of BCC tumors in Gorlin Syndrome. The data being presented at IID include a more recent analysis of that data that shows that patidegib topical gel treated patients developed three-fold fewer new facial BCCs compared to patients treated with vehicle gel for six months (0.4 vs. 1.4 new tumors). Only 16% of patidegib topical gel treated patients developed a new facial BCC compared with 60% in vehicle. PellePharm intends to begin enrollment for a Phase 3 trial starting in July 2018 based on these study results.

- Prevalence and risk factors for high-frequency basal cell carcinoma in a large commercially insured population in the US
  Poster #335 — Thursday, May 17, 2018 at 11:45 a.m. – 1:45 p.m. ET

  This study analyzed insurance claims data from 14 million U.S. adults aged 18 to 64 and found that 11,000 patients suffer from high-frequency BCCs. High-frequency BCC patients are a rare subset of patients with sporadic BCCs, who on average experience more than three BCCs annually.
- Frequent basal cell cancer development is a clinical marker for inherited cancer susceptibility
  Poster #164 — Wednesday, May 16, 2018 at 12:00 p.m. – 2 p.m. ET

  This study, conducted at Stanford University Medical Center, sequenced 29 cancer genes in 60 high-frequency BCC patients, defined in the study as the top 5% of patients with any BCC. Approximately 20% of sequenced patients had mutations in DNA repair genes.

“The epidemiology and gene sequencing data presented at IID identify patients with high-frequency BCC who, like patients with Gorlin Syndrome, develop multiple BCCs and are in need of safe and effective preventative therapies,” said Ervin Epstein, M.D., co-founder, chief medical officer and director of PellePharm. “With more than 40,000 cases of high-frequency BCC in the adult U.S. population, these results support our goal of working tirelessly to improve the quality of life for those suffering from these rare dermatological diseases and to reduce or even eliminate entirely the need for regular, painful surgeries.”

About Patidegib

Topical patidegib gel has shown early promise in a phase 2 clinical study for the mitigation of the BCC tumors in Gorlin Syndrome by blocking the disease at its source within the hedgehog signaling pathway. Topical patidegib was developed to provide the efficacy previously demonstrated by oral patidegib in phase 1 trials without the adverse systemic side effects. Patidegib’s gel formulation is stable at room temperature for at least two years, making it a viable potential therapy for ongoing, at-home management of Gorlin Syndrome. Patidegib has received both Orphan Drug Designation and Breakthrough Designation from the FDA.

About Gorlin Syndrome

Gorlin Syndrome is a rare, genetic, disease characterized by mutations in the tumor suppressor gene encoding PATCHED1 (PTCH1), which acts as the primary inhibitor of the hedgehog signaling pathway. This leads to hundreds of basal cell carcinomas, especially on the face.

With no FDA-approved drugs available for Gorlin Syndrome, also known as Basal Cell Carcinoma Nevus Syndrome (BCCNS), the standard of care is surgery. People with severe Gorlin Syndrome may have as many as 30 surgeries per year, which can be repetitive and scarring. 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.

About High Frequency Basal Cell Carcinoma
High Frequency Basal Cell Carcinoma, like Gorlin Syndrome, is a rare disease which is characterized by the development of an abnormally high number of BCCs (three or more BCCs per year). Unlike patients with Gorlin Syndrome, patients with high frequency BCC are not born with a germline PTCH1 mutation and do not suffer from the other systemic manifestations of Gorlin Syndrome.

The standard of care for patients with high frequency BCC is surgery. Approximately 11,000 adults aged 18 to 64 suffer from high frequency BCC in the United States. In addition, approximately 33,000 adults over the age of 65+ are affected in the United States.

About PellePharm
Founded by world leaders in hedgehog pathway signaling, PellePharm is a biotechnology company committed to targeting hedgehog-driven diseases, including Gorlin Syndrome and Basal Cell Carcinomas (BCCs), at their source. PellePharm's mission is to improve the quality of life for those suffering from Gorlin Syndrome and BCCs by providing an easy-to-use topical solution that eliminates the need for regular, painful surgeries. Topical patidegib is a first-in-class topical gel formulation of a proprietary hedgehog inhibitor.

About BridgeBio Pharma
BridgeBio is a clinical-stage biotech company developing novel, genetically targeted therapies to improve the lives of patients. The BridgeBio approach combines a traditional focus on drug development with a unique corporate model, allowing rapid translation of early stage science into medicines that treat disease at its source. Founded in 2015 by a team of industry veterans, the company has built a robust portfolio of ten transformative drugs ranging from pre-clinical to late stage development in multiple therapeutic areas including oncology, cardiology, dermatology and endocrinology. The company's focus on scientific excellence and rapid execution aims to translate today's discoveries into tomorrow's medicines.

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