NEWS RELEASE

fda grants breakthrough therapy designation and orphan drug designation to pellepharm for topical patidegib in gorlin syndrome

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MENLO PARK, Calif.–(**BUSINESS WIRE**)–PellePharm, a clinical-stage biopharmaceutical company committed to targeting rare genetic dermatological conditions at the source, today announced that the U.S. Food and Drug Administration (FDA) has granted both Breakthrough Therapy Designation and Orphan Drug Designation to topical patidegib for reduction of the life-long serious clinical morbidity and disease burden of persistently developing basal cell carcinomas (BCCs) in patients with Basal Cell Carcinoma Nevus Syndrome (BCCNS), a rare genetic disease also known as Gorlin Syndrome.

“These two significant FDA designations mark important steps forward in our development of topical patidegib as a drug with substantial potential to reduce the BCC tumor burden in Gorlin Syndrome patients. From our co-discovery of the underlying genetic defect, through over two decades of preclinical research with hedgehog inhibitors, and our recent clinical trials, our team at PellePharm is deeply committed to delivering a safe and effective therapy for people living with Gorlin Syndrome,” said Ervin Epstein, MD, cofounder and chief medical officer of PellePharm. “We are eager to continue our development program and work closely with the FDA and other regulatory authorities to deliver this potential first-in-class therapy to patients as quickly as possible.”

The FDA granted PellePharm Breakthrough Therapy Designation based on results from its Phase 2 trial assessing the safety and efficacy of topical patidegib in patients with Gorlin Syndrome. Topline data from this study were announced in July 2017. Breakthrough Therapy Designation is designed to expedite the development and review of drugs that are intended to treat a serious condition, where preliminary clinical evidence indicates that the drug may

demonstrate substantial improvement over available therapy.

The FDA granted Orphan Drug Designation based on the relatively low prevalence of Gorlin Syndrome. The FDA's Orphan Drug Designation program is designed to incentivize and facilitate the development of drugs for rare diseases that affect fewer than 200,000 people in the U.S. Approximately 10,000 people in the United States, or one in 31,000, are believed to be affected by Gorlin Syndrome.

"We are pleased that the FDA recognizes the importance of accelerating the development of topical patidegib for people affected by BCCNS (Gorlin Syndrome)," said Jean Pickford, executive director of the Basal Cell Carcinoma Nevus Syndrome Life Support Network. "People living with BCCNS need additional therapeutic options to help lessen the burden of disease."

Currently, there are no FDA-approved therapies for Gorlin Syndrome, and the standard of care for this rare disease is surgery. Patients with severe disease have as many as 30 surgeries per year.

PellePharm intends to initiate a Phase 3 clinical trial for patidegib in Gorlin Syndrome in 2018.

About Patidegib

Topical patidegib gel has shown early promise in a Phase 2 clinical study 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, but without the adverse systemic side effects of oral hedgehog inhibitors. 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.

Topical patidegib currently is being studied in a United States-based Phase 2 clinical trial for the treatment of sporadic BCCs.

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 and sun-exposed areas.

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 BCCNS, Gorlin-Goltz Syndrome, Basal Cell Nevus Syndrome, or Nevoid Basal Cell Carcinoma Syndrome.

About PellePharm

Founded by world leaders in hedgehog signaling, PellePharm, a BridgeBio company, is committed to targeting rare genetic dermatological conditions, including Gorlin Syndrome and Basal Cell Carcinomas (BCCs), at the source. PellePharm is committed to improving 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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