NEWS RELEASE

pellepharm launches with financing from bridgebio pharma to develop topical therapy for basal cell carcinomas and gorlin syndrome

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MENLO PARK, Calif.–(**BUSINESS WIRE**)–PellePharm, a clinical-stage biopharmaceutical company committed to developing patidegib, a topical hedgehog inhibitor to treat basal cell carcinomas (BCCs), including those in Gorlin Syndrome, a devastating orphan disease, announced its launch with financing from BridgeBio Pharma. This financing supports the development of topical patidegib through the completion of two phase 2 clinical trials that are underway, including a clinical trial in Gorlin Syndrome that just completed enrollment. In addition, the company has a drawable pool of capital from BridgeBio Pharma that enables it to finance future clinical trials through registration.

PellePharm was founded by a team of globally recognized experts in dermatology, oncology and hedgehog signaling and is managed by a team of industry experts. The company is dedicated to finding an effective therapy to manage the extensive tumor burden in Gorlin Syndrome, a rare genetic disease that causes patients to develop multiple BCCs. Gorlin Syndrome affects about 10,000 people in the United States and 15,000 people in the European Union. The disease results from a mutation in a tumor-suppressor gene, which acts as the primary inhibitor of the hedgehog signaling pathway. PellePharm's founders developed a topical gel formulation of a proprietary hedgehog inhibitor that it exclusively licensed from Infinity Pharmaceuticals to help mitigate certain adverse events observed with oral hedgehog inhibitors.

“It made sense that a potent inhibitor of the hedgehog pathway should provide a therapeutic benefit for patients suffering from Gorlin Syndrome, but we needed an approach that would allow us to target the disease at its source without eliciting harmful toxicity reactions,” said Ervin Epstein, M.D., co-founder of PellePharm. “We are hopeful that patidegib will provide the balance of targeted treatment without the adverse events associated with oral formulations.”

Founders and Scientific Advisory Board Members
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PellePharm’s approach attracted the attention of industry veterans Mark de Souza, Ph.D. and Karl Beutner, M.D., Ph.D. who joined the team. “It’s not often that you find a program as attractive as PellePharm’s patidegib,” said de Souza, executive chairman of PellePharm. “This investigational treatment has the potential to address a very high unmet need among patients with Gorlin Syndrome, whose current standard of care is to undergo multiple surgeries each year to manage their symptoms. We know that hedgehog pathway inhibitors have therapeutic potential for this condition, and we believe PellePharm’s topical formulation may offer a truly effective treatment option for these patients.”

PellePharm is currently conducting two double-blinded, placebo-controlled, randomized, phase 2 clinical trials that are evaluating the safety, tolerability and effect of topical patidegib on both pre-existing tumors and the frequency of the development of new tumors. A U.K.-based study is evaluating 18 patients with Gorlin syndrome to determine whether the product shrinks the tumors present at the beginning of the study and reduces the number of new tumors that develop during the trial. A U.S.-based study is evaluating 36 patients with sporadic BCCs to determine whether tumor diameter decreases after 12 weeks of treatment. Both studies also will evaluate safety and tolerability, as well as a biomarker (GLI-1) of hedgehog signaling. Enrollment in the U.K. trial is complete and topline data is expected in the second quarter of 2017.

“PellePharm is really at the intersection of precision oncology and monogenic dermatology – these are the types of assets we look for at BridgeBio Pharma,” said Frank McCormick, investment committee member of BridgeBio Pharma and board member of PellePharm. “Gorlin Syndrome is a well-researched disease that can be treated

directly at its source. We have had the privilege of knowing the founding and operating team of PellePharm for years and look forward to working with them to bring patidegib to patients in need."

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- Philip Beachy, Ph.D., professor of biochemistry and developmental biology, Stanford University
- Ervin Epstein, M.D., senior scientist, Children's Hospital of Oakland Research Institute
- Jean Tang, M.D., Ph.D., associate professor of dermatology, Stanford University

About Basal Cell Carcinomas (BCCs) and Gorlin Syndrome

Basal cell carcinomas (BCCs) are a type of skin cancer that begins when one of the skin cells spontaneously develops a mutation in its DNA, usually due to UV radiation (sunlight, tanning lamps). They commonly occur in sun-exposed areas like the face and neck and generally in people of European descent with lighter skin and in the elderly and in people with chronic sun exposure. There are more than four million BCCs diagnosed in the United States each year. BCCs are usually treated with surgery, which can leave disfiguring scars.

Gorlin Syndrome, also known as nevoid basal cell carcinoma syndrome, is a rare genetic disease where patients develop many BCCs. Patients with Gorlin Syndrome have heritable 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 BCCs, especially on the face and sun-exposed areas. The standard of care is surgery, as there are no FDA-approved drugs for Gorlin Syndrome. Individuals who have severe Gorlin Syndrome have as many as 30 surgeries per year, many of which can be scarring.

About PellePharm

PellePharm is developing patidegib, a novel hedgehog pathway inhibitor, to treat basal cell carcinomas (BCCs), including those in Gorlin Syndrome, a devastating rare genetic skin disease. The company is focused on developing and commercializing this treatment to reduce the burden of disease and improve quality of life by potentially reducing invasive and painful surgeries and the scarring that often accompanies them. Topical patidegib is a first-in-class topical gel formulation of a proprietary hedgehog inhibitor exclusively licensed from Infinity Pharmaceuticals. Patidegib is currently being studied in two phase 2 clinical trials for the treatment of BCCs and Gorlin Syndrome.

About BridgeBio Pharma

BridgeBio Pharma develops medicines for genetic diseases with significant unmet need. The company is primarily focused on pre-commercial products, utilizing its advantages in sourcing and operating to find and build value at

each stage of development. Founded in 2015, and backed by KKR and Perceptive Life Sciences, BridgeBio Pharma is actively developing multiple clinical and pre-clinical programs.

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