



## NEWS RELEASE

# pellepharm announces topline results from phase 2 study of topical patidegib in gorlin syndrome basal cell carcinomas and third closing of a \$20 million series b financing

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MENLO PARK, Calif.-(**BUSINESS WIRE**)-PellePharm, a clinical-stage biopharmaceutical company committed to targeting hedgehog-driven disorders at the source, today announced topline data from its phase 2 trial evaluating the safety and efficacy of topical patidegib in patients with Gorlin Syndrome, a rare genetic disease that causes patients to develop multiple basal cell carcinomas (BCCs). PellePharm intends to initiate discussions with the FDA, with the goal of initiating a phase 3 clinical trial for patidegib in the first half of 2018.

The double-blinded, placebo-controlled, randomized, three-arm, UK-based trial evaluated a patidegib topical therapy. Seventeen patients participated in the trial and were self-treated with topical patidegib, a novel hedgehog inhibitor, for six months. There was a statistically significant difference in complete response between treatment and vehicle groups in the per protocol analysis, with complete response demonstrated in 12 tumors across both treatment arms, compared with zero tumors in the vehicle arm. The complete responses were correlated with a meaningful reduction of a biomarker for hedgehog signaling (GLI1). Partial responses were also demonstrated in both treatment groups. Importantly, there were zero detectable levels of patidegib in plasma after topical application of patidegib gel, and patients treated with this topical gel had none of the devastating class-specific systemic side effects observed with oral hedgehog inhibitors.

"The search for safe and effective chronic mitigation of the tumor burden for patients suffering from Gorlin Syndrome has been my life's work, and I am encouraged by the safety and efficacy signals indicated by this trial,"

said Ervin Epstein, MD, co-founder, president and director of PellePharm. “We very much look forward to further development of this compound, which should reduce greatly the patient need for surgeries by blocking the molecular driver of BCCs.”

“Having worked closely with those affected by Basal Cell Carcinoma Nevus Syndrome, also known as Gorlin Syndrome, on a daily basis for more than 15 years, I know first-hand how devastating this condition is to quality of life,” said Kristi Burr, founder of the Basal Cell Carcinoma Nevus Syndrome Life Support Network and patient advocate. “PellePharm’s trial represents important progress, bringing hope to people affected by the disease who currently have extremely limited options for addressing their invasive BCCs throughout their lifetime.”

Currently, there are no FDA-approved drugs to treat Gorlin Syndrome and the standard of care for this rare disease is surgery.

#### Additional Corporate Updates

PellePharm confirmed that it has raised an aggregate of \$23.5 million to date, with a third closing of a \$20 million Series B financing round led by BridgeBio. A prior closing was announced in December 2016. BridgeBio and other current investors are expected to participate in a planned Series C financing to support the company’s Phase 3 clinical trial for patidegib through NDA filing.

“PellePharm continues to demonstrate patidegib’s potential to expand the treatment landscape for Gorlin Syndrome patients, and we are pleased to see the progress this development candidate is making in the clinic,” said Frank McCormick, Ph.D., co-founder of BridgeBio and former president of the American Association for Cancer Research (AACR). “As PellePharm begins discussions with the FDA about initiating a pivotal phase 3 trial, we believe that this financing will help propel the company through this important step forward for both patients and investors.”

PellePharm also announced the completion of enrollment for its second phase 2 trial, a double-blinded, dose-escalating, placebo-controlled proof-of-concept clinical trial evaluating topical patidegib gel in patients with sporadic BCCs. Topline data from this trial is expected to be reported in Q4 2017.

#### 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.

#### About Basal Cell Carcinomas (BCCs) and Gorlin Syndrome

Basal cell carcinomas (BCCs) are a type of skin cancer that begins when skin cells develop mutations in their 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 Basal Cell Carcinoma Nevus Syndrome, nevoid basal cell carcinoma syndrome, Gorlin-Goltz syndrome, is a rare genetic disease in which 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 mitigate the tumor burden in patients with 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 a phase 2 clinical trial for the treatment of sporadic BCCs.

#### 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.

#### contacts

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