



NEWS RELEASE

## pellepharm presents updated data from two phase 2 studies demonstrating the potential of patidegib topical gel to treat basal cell carcinomas in patients with gorlin syndrome and patients with non-gorlin sporadic bccs

2018-10-15

SAN FRANCISCO—(**BUSINESS WIRE**)—**PellePharm**, a late clinical-stage biopharmaceutical company committed to targeting rare skin conditions at their source, today presented updated clinical data from two Phase 2 studies of patidegib topical gel in a poster session at NORD's Rare Diseases and Orphan Products Breakthrough Summit 2018 in Washington, D.C. Results of the studies showed clinical clearance and prevention of basal cell carcinoma (BCC) tumors after 6 months of treatment with patidegib topical gel in patients with Gorlin Syndrome, a rare genetic disease; and both clinical and histologic clearance after 3 months of treatment in patients with sporadic, nodular BCCs.

"We are pleased by these positive results showing the potential of patidegib topical gel in preventing new facial BCCs in patients with Gorlin Syndrome, potentially reducing the number of surgeries and resulting facial scarring that patients experience," said Sanuj Ravindran, M.D., president and chief executive officer of PellePharm. "Based on these Phase 2 findings, we intend to initiate a randomized, one-year registrational Phase 3 trial of patidegib topical gel at the end of 2018 in patients with Gorlin Syndrome, and we also plan to study patidegib topical gel in non-Gorlin patients with high-frequency BCCs."

Title: Hedgehog Inhibition by Topical Patidegib Reduces Facial BCC Burden in Patients with Gorlin Syndrome

Date and Time: Monday, October 15 – Tuesday, October 16, 2018

Poster Number: #71

The data presented at the NORD Summit are from two Phase 2 trials of topical patidegib gel – a randomized, double-blind, placebo-controlled, study in patients with Gorlin Syndrome in the UK and in a study in patients with sporadic, nodular BCCs in the US. In the Gorlin Phase 2 trial, 17 patients with a total of 85 surgically eligible BCCs (SEBs) applied patidegib topical gel (administered in strengths of 2% and 4%) or a topical vehicle control twice daily for 6 months to their face for prevention and to 5 SEBs for treatment.

The Phase 2 Gorlin trial showed that for prevention of surgically-eligible BCCs (SEBs), patients in the topical control group developed an average of 1.4 new SEBs in 6 months. In contrast, patients treated with patidegib topical gel 2% and 4% developed only 0.4 SEBs in Intent to Treat analysis ( $p=0.096$ ) and only 0.3 SEBs in Per-Protocol analysis ( $p=0.008$ ). Additionally, clinical clearance of tumors was observed in 27% (12 out of 45 SEBs) of the patidegib topical gel treated subjects compared to no tumors with clinical clearance in subjects in the vehicle group ( $N=16$  SEBs,  $P=0.02$ ).

In the U.S. trial, 36 non-Gorlin patients with sporadic, nodular BCCs applied patidegib topical gel 2%, 4%, or topical control to BCCs for 3 months. Use of patidegib topical gel 2% was significantly more effective in clinical and histologic clearance of BCCs after 3 months compared with the topical vehicle gel ( $p=0.045$ ). This finding correlated with a decrease in hedgehog biomarker in BCC tumors after three months in patients using patidegib topical gel.

Across both studies, patients using topical patidegib gel did not experience any of the significant side effects characteristic of oral hedgehog inhibitors (e.g., hair loss, taste loss or frequent muscle cramps). Patients treated with patidegib topical gel 4% experienced mild skin irritation (e.g., redness, itching and swelling), but those using patidegib topical gel 2% did not.

#### About Patidegib

Patidegib topical gel has shown early promise in a Phase 2 clinical study for the mitigation of BCC tumors in Gorlin Syndrome by blocking the disease at its source within the hedgehog signaling pathway. Topical patidegib gel 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 Therapy Designation from the FDA.

#### About Gorlin Syndrome

Gorlin Syndrome is a rare, genetic, disease where patients are born with mutations in 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, often on the face.

With no FDA-approved drugs available for Gorlin Syndrome BCCs, the standard of care 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 (BCCs)

High-Frequency BCC, like Gorlin Syndrome, is a rare disease which is characterized by the development of an abnormally high number of BCCs. 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.

#### About PellePharm

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

#### contacts

W2O Group

Peter Duckler, 310-774-3425

**[pduckler@w2ogroup.com](mailto:pduckler@w2ogroup.com)**