



NEWS RELEASE

## leo pharma and pellepharm announce \$760 million collaboration to advance innovative therapies for rare skin diseases

2018-11-20

SAN FRANCISCO & BALLERUP, Denmark–(**BUSINESS WIRE**)– Danish dermatology specialists **LEO Pharma** and California rare disease pioneers **PellePharm** today announced a strategic development and commercialization collaboration to address unmet medical needs across various skin diseases with no approved treatments, advancing innovation and access to potential therapies for patients with life-altering conditions, such as Gorlin Syndrome and High Frequency Basal Cell Carcinoma (BCC), two distinct and rare forms of skin cancer.

This press release features multimedia. View the full release here: <https://www.businesswire.com/news/home/20181120005227/en/>

“We are very excited about the partnership with PellePharm, who are pioneers in Gorlin Syndrome and experts in rare skin diseases. Supporting our ambitious 2025 strategy, it marks LEO Pharma’s entrance in rare skin diseases and it offers a unique opportunity to bring the first treatment forward to people suffering from a very severe skin disease for which there currently are no approved therapies,” said Thorsten Thormann, vice president of research at LEO Pharma.

Under the terms of the agreement, LEO Pharma has initially committed \$70 million comprised of equity financing and financial R&D support to fund the global Phase 3 trial for patidegib topical gel 2% for the prevention and treatment of Gorlin Syndrome, with LEO Pharma securing an option to acquire all shares in PellePharm. PellePharm and its stockholders could receive up to an additional \$690 million including merger consideration, and regulatory

and commercial milestone payments. In addition, PellePharm stockholders are eligible to receive a double-digit royalty after achieving certain commercial milestones.

“Our company is founded on the commitment to targeting rare dermatologic diseases at the source and bringing new groundbreaking treatments forward to patients as efficiently and effectively as possible. As a global leader in medical dermatology, LEO Pharma is a great fit as both a development and commercialization partner. This collaboration puts us on track to commence our pivotal Phase 3 Gorlin Syndrome trial in early 2019. Then after the potential merger, we look forward to working with LEO Pharma to address other rare skin diseases with unmet needs,” said Sanuj Ravindran, president and chief executive officer of PellePharm.

The agreement establishes a joint development committee with PellePharm maintaining responsibility for global development and LEO Pharma supporting in an advising role. Both companies will jointly drive commercialization planning, and Anders Kronborg, chief financial officer of LEO Pharma, will join PellePharm’s board of directors.

“Gorlin Syndrome Group welcomes the news that PellePharm and LEO Pharma are collaborating in developing patidegib topical as a treatment for BCCs. Data from PellePharm’s Phase 2 study suggest patidegib is effective in treating BCCs, with minimal side effects. We hope that by working with LEO, these benefits can be confirmed in a Phase 3 study. An effective topical medication, which can prevent and/or treat BCCs, will avert the need for the painful, disruptive and disfiguring surgeries we currently undergo,” said Sally Webster, chairperson and Matthew Helbert, trustee of Gorlin Syndrome Group U.K.

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

PellePharm’s 2% topical formulation of patidegib aims to be the first approved therapy for the prevention of Gorlin Syndrome, also known as Basal Cell Carcinoma Nevus Syndrome (BCCNS), and has shown early promise in Phase 2 clinical trials treating patients with Gorlin Syndrome and Sporadic BCC in both the U.S. and the U.K.

“For more than 18 years, our organization has been dedicated to supporting, educating, and seeking much-needed treatments for people living with Gorlin Syndrome (BCCNS), and yet people with this devastating disease still have no FDA-approved therapies,” said Jean Pickford, executive director of the BCCNS Alliance. “With the Phase 3 study of patidegib beginning recruitment in early 2019, we continue to be encouraged by PellePharm’s dedication to our community, now in partnership with LEO Pharma.”

PellePharm was advised by Rothschild & Co. on this transaction.

## 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. The topical formulation of patidegib was developed to provide the efficacy previously demonstrated by oral patidegib in Phase 1 trials without the adverse systemic side effects. The 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 and High Frequency BCC. PellePharm has received both Orphan Drug Designation and Breakthrough Therapy Designation for patidegib topical gel in Gorlin Syndrome from the FDA.

## About Gorlin Syndrome

Gorlin Syndrome is a rare, genetic disease characterized by constitutional, heritable mutations in one allele of 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, 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 (BCC)

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 LEO Pharma

LEO Pharma helps people achieve healthy skin. By offering care solutions to patients in more than 130 countries globally, LEO Pharma supports people in managing their skin conditions. Founded in 1908 and owned by the LEO Foundation, the healthcare company headquartered in Denmark has devoted decades of research and development to delivering products and solutions to people with skin conditions. In 2017, LEO Pharma employed around 5,200 people worldwide and had sales of 1.4 billion euros. For more information, go to: [www.leo-pharma.com](http://www.leo-pharma.com) / [www.linkedin.com/company/leo-pharma](https://www.linkedin.com/company/leo-pharma)

## About PellePharm

Founded by world leaders in hedgehog pathway signaling, PellePharm, a BridgeBio Company, is committed to targeting rare, genetic dermatological diseases, including Gorlin Syndrome and High Frequency Basal Cell Carcinoma (BCC), at their source. PellePharm's mission is to improve the quality of life for those suffering from Gorlin Syndrome and High Frequency BCC 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.

## 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 seventeen 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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