

Press
Release

First patient enrolled in phase-IIa study of vamifeport in patients with sickle cell disease

- **Vamifeport (VIT-2763) is the first oral ferroportin inhibitor investigated for treatment of diseases with ineffective production of red blood cells and iron overload such as sickle cell disease (SCD)**
- **SCD is a rare blood disorder with currently limited treatment options**
- **Topline results are expected at the end of 2022**

St. Gallen, Switzerland, 10 December 2021 – Vifor Pharma today announced that the first patient has been enrolled in a double-blind, randomized phase-IIa clinical trial evaluating the safety, efficacy and tolerability of vamifeport in adult patients with sickle cell disease. Vamifeport, developed by Vifor Pharma, is a novel oral ferroportin inhibitor investigated for treatment of diseases characterized by ineffective production of red blood cells and iron overload, including SCD and beta-thalassemia. It has been granted orphan drug designation from both the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) for the treatment of SCD.

Dr. Klaus Henning Jensen, Chief Medical Officer of Vifor Pharma Group commented, “Starting the phase-IIa trial is an important milestone in the clinical development of vamifeport for sickle cell disease, a debilitating orphan condition for which there are very limited treatment options. Patients worldwide continue to suffer from consequences of the disease which dramatically impacts their quality of life and life expectancy. We look forward to continuing development of our potential new treatment for these patients”.

SCD is a group of inherited red blood cell disorders and is a genetic condition present at birth. In this disease, red blood cells carry abnormal haemoglobin, which makes them prone to rupture, causing adhesion of sickle cells and inflammatory cells to the blood vessels. This ultimately leads to an obstruction of blood flow and organ damage. There are an estimated 150,000 patients in Europe and the US combined living with sickle cell disease.

About the SCD-202 trial

The phase-IIa study is a randomized, double-blind, placebo-controlled, parallel group trial aiming to recruit 25 patients at 14 clinical sites in the United States, the United Kingdom, Lebanon and Greece. The primary objective of the trial is to assess the change in markers of hemolysis (the rupturing of red blood cells) upon treatment with vamifeport. Patients will undergo initial treatment for 4 weeks, followed by an additional 4-week period with either maintenance of the dose level or a dose step-up. The study also includes a 4-week follow-up period after treatment. End of enrolment is expected mid-2022 with topline results anticipated at the end of 2022.

Further information about the study, including eligibility requirements, is available at www.clinicaltrials.gov (NCT04817670).

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About Vifor Pharma Group

Vifor Pharma Group is a global pharmaceuticals company. It aims to become the global leader in iron deficiency, nephrology and cardio-renal therapies. The company is a partner of choice for pharmaceuticals and innovative patient-focused solutions. Vifor Pharma Group strives to help patients around the world with severe and chronic diseases lead better, healthier lives. The company develops, manufactures and markets pharmaceutical products for precision patient care. Vifor Pharma Group holds a leading position in all its core business activities and consists of the following companies: Vifor Pharma and Vifor Fresenius Medical Care Renal Pharma (a joint company with Fresenius Medical Care). Vifor Pharma Group is headquartered in Switzerland, and listed on the Swiss Stock Exchange (SIX Swiss Exchange, VIFN, ISIN: CH0364749348). For more information, please visit viforpharma.com.

About vamifeport

Vamifeport is an oral inhibitor of ferroportin, an enzyme essential for the body's transport of iron and plays a key role in regulating iron uptake and distribution in the body. Vamifeport binds to ferroportin and blocks it to prevent excessive iron release into the blood. As iron is needed for the formation of haemoglobin, this relative decrease in iron availability is expected to reduce the concentration of abnormal haemoglobin in red blood cells and prevent ensuing unfavorable events. Vamifeport-mediated ferroportin inhibition may lead to less haemolysis, decrease of inflammation and better blood flow with consequent improvement of both the symptoms and clinical outcomes of SCD.

Vamifeport is currently also in phase II development for beta-thalassemia, an inherited rare blood disorder that reduces the production of functional haemoglobin in red blood cells, which can lead to a lack of oxygen in many parts of the body and potentially cause anaemia.