Vicore reports new 36-week data from the AIR trial demonstrating sustained disease stabilization and increase in lung function in IPF patients

Stockholm, May 19, 2023 – Vicore Pharma Holding AB (publ) ("Vicore"), unlocking the potential of a new class of drugs to stop disease progression and restore function, today announces an updated interim analysis of its AIR phase 2a trial with C21 in idiopathic pulmonary fibrosis (IPF). With 51 patients enrolled, the data demonstrates that C21 has the potential to transform the treatment of IPF and restore lung function. The disease is currently considered to be incurable and inevitably progressive.

- C21 continues to be safe and well tolerated with no treatment-related serious adverse events
- C21 continues to demonstrate long-term efficacy, at 36 weeks the average FVC* had increased to +350 mL over baseline, which is +530 mL over the expected trajectory of untreated patients (n= 19; p=0.001)
- The data will be orally presented at the American Thoracic Society (ATS) international congress on May 21st and during a webcast on May 26th, including a Q&A session
- Vicore plans to progress clinical development of C21 through initiation of a phase 2b trial (ANDAS) and will conclude recruitment to the AIR trial

*FVC: Forced Vital Capacity, a measure of lung capacity

Professor Toby Maher, Keck School of Medicine at University of Southern California, commented *"The magnitude of FVC stabilization seen with C21 in the AIR trial is very different from what we normally see in clinical practice and certainly gives hope for patients. If data are replicated in the ANDAS trial, there will be a fundamental change in how IPF is treated with an opportunity to stop progression and restore lung function*".

The AIR trial[1], a multi-center, open label, single arm 24-week trial with a 12-week extension studying the safety and efficacy of the angiotensin II type 2 receptor agonist (ATRAG) C21 in patients with IPF, has now enrolled 51 patients. At the time of analysis, 27 patients had completed 24 weeks of treatment with an average increase in FVC of +50mL and a 3-visit average increase of +110 mL (p=0.007 versus the expected trajectory of untreated patients), and 19 patients had completed 36 weeks of treatment with an average increase in FVC of +350 mL and a 3-visit average increase of +220 mL (p=0.001 versus the expected trajectory of untreated patients). Out of the 19 patients that had completed 36 weeks of treatment, 17 presented an FVC value that was better than what would have been expected of an untreated population. The new dataset shows a stabilization of lung capacity already at week 6 and, in line with previous interim analysis, a subsequent increase of FVC from week 16 to 36. Now, with twice the number of patients versus the interim analysis announced in November 2022, the previously reported early stabilization followed by an increase in lung function is confirmed, suggesting that C21 has the potential to transform the treatment of IPF.

Carl-Johan Dalsgaard, CEO of Vicore, commented *"The long-term stabilization and increase in FVC is unique for patients treated with C21 and consistent with the mechanism of action of an ATRAG. Restoring alveolar integrity is key in treating IPF and that is what C21 is doing".*

C21 continued to be safe and well tolerated with no treatment-related serious adverse events; there was a low rate of disease progression or worsening of cough and no gastrointestinal tolerability issues. 94% and 96% of patients at week 12 and 24, respectively, showed a positive benefit/risk, according to a joint benefit/risk assessment by the patients and principal investigator.

Recruitment to the AIR trial will be concluded to fully focus on the next step of development, the phase 2b ANDAS trial. Vicore has engaged world leading experts and patient advocacy organizations in its advisory committee to aid in the design and successful conduct of the trial.

Rohit Batta, CMO of Vicore said *"We are thrilled to see that the previously reported long-term stabilization and increase in FVC holds through with now double the number of patients in the AIR trial. These results are truly encouraging with regard to the future clinical development of C21 and our ambition to provide a treatment for IPF patients as soon as possible."*

Biomarkers further validate C21 results
The clinical findings with FVC have been confirmed with relevant biomarkers, thereby increasing the confidence in C21. FVC correlated strongly with lung volume (p=0.001) as measured in 3D reconstructions of CT scans, reinforcing the accuracy of the FVC measurements. Furthermore, patients with early IPF disease showed significantly less end-terminal fibrosis in the scans (p<0.02) and a higher degree of FVC increase after 36 weeks of treatment compared to patients with established IPF. This is in line with the C21 mechanism of action, promoting alveolar repair. The biomarker TGFb1 was reduced from baseline by 57% at 24 weeks (n=18), suggesting a reduced fibrosis drive. TGFb1 is a key mediator of fibrosis and its reduction has consistently been seen in cell cultures, animal models as well as in slices of human IPF lung tissue exposed to C21.

Oral presentation during ATS on May 21
The updated interim analysis will be presented by Professor Toby Maher, Keck School of Medicine at University of Southern California, during the ATS congress in Washington, DC on Sunday, May 21, 2023 at 2:51 pm EDT (during session A99).

Webcast presentation on May 26
Vicore will host a webcast including a Q&A session to discuss the phase 2a AIR trial results of C21 in IPF.
C21 in IPF – Presentation of an advanced interim analysis
Date and time: Friday May 26, 2023 at 08:00 EST/14:00 CET
How to join: https://stream.brrmedia.co.uk/broadcast/645cb5017935152b5ae742f1
The webcast and presentation will be available after the webcast at: https://vicorepharma.com/investors/events-presentations/

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About idiopathic pulmonary fibrosis (IPF)
IPF is a progressive, lethal fibrotic lung disease that occurs primarily in middle-aged and elderly adults. The average life expectancy from diagnosis is 3-5 years. An increased prevalence of fibrotic diseases in combination with a rising geriatric population is driving future growth of the IPF patient population. IPF is considered an orphan disease and the worldwide estimated prevalence ranges from 0.3-4.5 per 10,000[2]. There are two anti-fibrotic therapies on the market today. These drugs can reduce the decline in lung function by about 50 percent, but they are also associated with side effects, causing a large share of patients to opt out of or not comply with their treatment. It is estimated that as much as 43% of patients in the US discontinue treatment[3]. Even though many patients are untreated today, the combined sales of these drugs was estimated to $4.2 bn in 2022[4]. The additional annual market potential (untreated patients) is estimated to be $2.8 bn[5]. With a growing patient population and limited treatment options, there is room for innovative and disease modifying treatments.

About the phase 2a trial in IPF (AIR)
The purpose of the trial is to investigate the safety and efficacy of C21 in treatment-naïve patients with IPF. The trial is an open-label, single-arm trial in which C21 is given orally twice daily as monotherapy for 24 weeks with an option to continue treatment for another 12 weeks. Patients with IPF have a well-characterized decline in lung function. The effect of C21 on lung function, measured by change from baseline in FVC, is investigated and interpreted in the light of the well-documented natural history of IPF. The trial has multiple centers with regulatory approvals obtained in the UK, India, Ukraine and Russia. The trial is paused in Ukraine and Russia due to the conflict.

About the renin-angiotensin system (RAS) and angiotensin II type 2 receptor (AT2 receptor)
The RAS is a hormone system that regulates several important physiological processes. In this system, the AT1 receptor is a well-established drug target with ARB's (Angiotensin Receptor Blockers), while the AT2 receptor has been more elusive and difficult to study. The AT2 receptor is part of the body's resolution and repair system and is thought to be protective in several diseases connected to ageing and cell senescence, including idiopathic pulmonary fibrosis, chronic kidney disease and heart failure, as well as cognitive disorders. Stimulating the AT2 receptor has been shown to be effective in combating disease in numerous animal models and clinical validation is well advanced in lung disease. Stimulating the AT2 receptor also dilates small diseased resistance vessels in animals and in humans, resulting in locally increased blood flow. With C21 as the first-in-class highly selective small molecule AT2 receptor agonist (ATRAG) taken into clinical trials, the therapeutic benefit of targeting the AT2 receptor is becoming increasingly apparent. Vicore is developing C21 for rare lung diseases and has a series of new ATRAGs in development for other indications, the first of which (C106) is in clinical phase 1.

About Vicore Pharma Holding AB (publ)
Vicore is an innovative Swedish clinical-stage pharmaceutical company unlocking the potential of a

new class of drugs to stop disease progression and restore function. The company is establishing a portfolio in rare lung diseases including idiopathic pulmonary fibrosis (IPF) and pulmonary arterial hypertension (PAH). C21 is a first-in-class orally available small molecule angiotensin II type 2 receptor agonist (ATRAG) currently in a phase 2a study of IPF. C21 is protected by US and European Orphan Designation. A variety of patents have been filed to provide further protection for C21, out to 2040 and onwards. Almee™ (an investigational medical device in clinical development) is a digital therapeutic (DTx) based on cognitive behavioral therapy (CBT) created to address the psychological impact of living with pulmonary fibrosis. Inhaled IMID is a new formulation and delivery route of thalidomide targeting the severe cough associated with IPF. Using its unique expertise in the ATRAG biology, Vicore is further fuelling its pipeline with several new small molecule drug assets, with long patent life and for a variety of indications, some of which could be partnered while others may be taken to the market by Vicore.

The company's shares (VICO) are listed on Nasdaq Stockholm's main market. For more information, see www.vicorepharma.com.

[1] NCT04533022
[2] Maher et al. Global incidence and prevalence of idiopathicpulmonary fibrosis. Respiratory research 22, 197 (2021)
[3] Dempsey et al. Adoption of the Antifibrotic Medications Pirfenidone and Nintedanib for Patients with Idiopathic Pulmonary Fibrosis. Ann Am Thorac Soc. 18, 7 (2021)
[4] Evaluate Pharma; Company reports, Roche and BoehringerIngelheim
[5] Estimated based on ~40% of IPF patients currently not on treatment

Attachments

Vicore reports new 36-week data from the AIR trial demonstrating sustained disease stabilization and increase in lung function in IPF patients