



# Verona Pharma

26 October 2016

## Verona Pharma receives second Venture and Innovation Award from the Cystic Fibrosis Trust

*Supports first clinical study in patients to explore potential for RPL554 as a novel treatment for cystic fibrosis*

*New data from pre-clinical studies to be presented at Annual North American Cystic Fibrosis Conference, 26-28 October 2016*

**26 October 2016, Cardiff** – Verona Pharma plc (AIM: VRP.L), a clinical-stage biopharmaceutical company focused on developing and commercializing innovative therapeutics for the treatment of respiratory diseases, announces that it has received a second Venture and Innovation Award from the UK Cystic Fibrosis Trust. The award will be used to help fund a Phase 2a clinical study to investigate the potential of the Company's drug candidate, RPL554, as a novel treatment for cystic fibrosis (CF).

The study is expected to start recruiting patients with CF during the first half of 2017. The primary objective of the study will be to determine the pharmacokinetic and pharmacodynamics profile and assess the tolerability and safety of RPL554 in patients with this disease.

The first Venture and Innovation Award the Company received from the UK Cystic Fibrosis Trust was granted in November 2014 and was used to help fund exploratory pre-clinical studies, which delivered encouraging data presented last year at the North American Cystic Fibrosis Conference and published in the American Journal of Physiology that has shown the potential of RPL554 as a treatment of CF.

New pre-clinical data of RPL554 in CF will be available from 26 October 2016 and presented during two poster sessions on 27 and 28 October 2016 at the Annual North American Cystic Fibrosis Conference in Orlando, Florida.

CF is an orphan disease that afflicts approximately 70,000 people worldwide. The disease is caused by mutations in the gene for the protein cystic fibrosis transmembrane conductance regulator (CFTR). CF affects several organs in the body but pulmonary disease is the primary cause of mortality.

Current therapies are directed at the impaired mucociliary clearance, the chronic lung infections and the chronic inflammation of the lungs, but with limited efficacy. Recently, drugs targeting the mutant CFTR function have become available. While these latter therapies are effective in small sub-sets of patients, most patients are still without adequate therapy for this life-shortening, chronic progressive disease.

RPL554, a first-in-class, inhaled, dual PDE3/PDE4 inhibitor, is currently in Phase 2 clinical development as a nebulized treatment for Chronic Obstructive Pulmonary Disease (COPD). In clinical trials, RPL554 has been observed to be an effective bronchodilator and to have anti-inflammatory properties in vitro as well as in a standard inhaled lipopolysaccharide challenge study in healthy volunteers. In these studies, the drug candidate has been well tolerated. Pre-clinical data to date has suggested that RPL554 is also a stimulator of the CFTR, which is dysfunctional in cells of cystic fibrosis patients. Based on these observed favorable properties, the Company believes RPL554 may improve mucociliary clearance (reduce phlegm in the airways), reduce symptoms of chronic inflammation and ease breathing.

### **Commenting on the award, Verona Pharma's CEO, Jan-Anders Karlsson, said:**

*"We are delighted to have received a second Venture and Innovation Award from the Cystic Fibrosis Trust, which will enable us to accelerate the clinical development of RPL554 for the treatment of cystic fibrosis, an orphan disease where there is very high unmet medical need. The award recognises RPL554's potential to be a novel and important treatment for this debilitating condition and we look forward to progressing the drug candidate through clinical development."*

### **Ed Owen, outgoing Chief Executive of the Cystic Fibrosis Trust, said:**

"We are delighted to continue to support the development of this compound and explore its potential to be an effective and complementary new treatment for cystic fibrosis, which remains an area of significant unmet medical need.

A vital part of our fight for a Life Unlimited is to accelerate the development of potential new therapies for people with cystic fibrosis. Building innovative collaborations with companies like Verona ensures that promising drug candidates are developed for use in cystic fibrosis and are given the best chance to get to those who need them as quickly as possible."

The title, timing and location of the abstract presentations at the Annual North American Cystic Fibrosis Conference are as follows:

Abstract Number 40

Authors: M.Turner, K.H. Abbott-Banner, S.H Randell, J.W Hanrahan

Title: PHOSPHODIESTERASE 8 IS A NOVEL REGULATOR OF CFTR IN HUMAN BRONCHIAL EPITHELIA

Day/Date: October 26-28, 2016

Location: Hall C

Time: Thursday, October 27: 11:15 a.m. – 1:45 p.m.

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**About Verona Pharma plc**

Verona Pharma is a clinical-stage biopharmaceutical company focused on developing and commercializing innovative therapeutics for the treatment of respiratory diseases with significant unmet medical needs. Verona Pharma's product candidate, RPL554, is a first-in-class, inhaled, dual inhibitor of the enzymes phosphodiesterase 3 and 4 that acts as both a bronchodilator and an anti-inflammatory agent in a single compound. In clinical trials, treatment with RPL554 has been

observed to result in statistically significant improvements in lung function as compared to placebo and has shown clinically meaningful and statistically significant improvements in lung function when added to two commonly used bronchodilators as compared to either bronchodilator administered as a single agent. RPL554 has also shown anti-inflammatory effects and been well tolerated in clinical trials. Verona Pharma is developing RPL554 for the treatment of chronic obstructive pulmonary disease (COPD), cystic fibrosis, and potentially asthma.

### **Forward Looking Statements**

This press release contains forward-looking statements. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements.

These forward-looking statements are based on management's current expectations. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from our expectations expressed or implied by the forward-looking statements, including, but not limited to, the development of DPI and MDI formulations of RPL554 and the potential for these formulations to increase the market opportunity for the product, if approved.

These and other important factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management's estimates as of the date of this press release. While we may elect to update such forward-looking statements at some point in the future, we disclaim any obligation to do so, even if subsequent events cause our views to change. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date of this press release.