Certa Therapeutics presents positive data from a Phase 2 clinical study highlighting the potential benefit of FT011 as a novel treatment for scleroderma

- Clinical trial data from a multi-national, double-blinded trial of FT011 being developed for the treatment of systemic sclerosis (scleroderma) presented at the American College of Rheumatology Annual Meeting 2023 (ACR Convergence)
- FT011 was safe and well tolerated; clinically meaningful improvement in patients with scleroderma over placebo in both treatment arms after 12 weeks
- Transcriptomic analysis confirmed positive impact of FT011 on fibrosis disease gene signature
- Patients in the open label extension show that clinical benefits continue beyond 19 months
- Preparations for a global pivotal trial of FT011 as a treatment for scleroderma are advancing

Melbourne, Australia: 16 November 2023 – Certa Therapeutics (Certa), a biotechnology company developing innovative precision therapies for patients with inflammatory and fibrotic diseases, announces the presentation of results of its Phase 2 clinical trial of FT011 in patients with scleroderma at ACR Convergence 2023, the annual scientific meeting of the American College of Rheumatology being held in San Diego, California (10 – 15 November 2023).

The positive clinical trial data was presented by study investigator Professor Chris Denton MD (University College London, UK), describing that treatment with FT011 for 12 weeks resulted in a clinically meaningful improvement in 60% of patients treated with FT011 400mg ($p = 0.019$ *vs.* placebo) and 20% of patients in the FT011 200mg group compared with 10% in the placebo group.[1,2]

The multi-national, double-blinded trial recruited a total of 30 adults who were randomly assigned to 3 treatment arms: oral FT011 400mg or FT011 200mg or placebo once daily, in addition to standard of care, for 12 weeks. In totality, treatment with FT011 led to significant improvements across multiple efficacy measures, including the American College of Rheumatology Combined Response Index in diffuse cutaneous Systemic Sclerosis (CRISS) score,[3] skin thickness (defined by the modified Rodnan Skin Score, mRSS), lung function (%FVC), physician-reported assessment and quality of life evaluations.

The study safety profile demonstrated that FT011 was safe and well tolerated, with no differences in adverse event (AE) rates between the treatment arms. There were no serious AEs reported in the study, nor any AEs resulting in study drug interruption, withdrawal or discontinuation.

Transcriptomic analysis was conducted from skin biopsy samples to investigate changes to gene signatures over the clinical trial. These transcriptomic results indicated that the systemic fibrotic disease gene signature is modulated after 12 weeks, with an increased fibrosis signature score in the placebo group but significantly, a decrease in the same fibrosis signature score following FT011 400mg treatment. This suggests that FT011 may have a positive effect for scleroderma patients by reducing the inflammation and fibrosis associated with the disease.

Initial results from an ongoing open label extension phase of the trial were also presented, where a subset of patients who completed the main study (up to week 12) elected to remain on FT011 400 mg treatment for up to an additional 24 months. Encouragingly, this open label extension indicates that FT011 continues to be safe and well tolerated, with clinical benefit for the scleroderma patients observed over the 19 months of therapy.

Professor Denton, a leading rheumatologist and Joint Director of the Centre for Rheumatology, Royal Free Hospital, London and Professor of Experimental Rheumatology at University College London, said “The Phase 2 trial demonstrates promising efficacy and safety data for FT011 after 12 weeks of treatment and certainly warrants a further study to assess the potential of FT011 to improve clinical outcomes for scleroderma patients. These results are very encouraging for the scleroderma community and a significant step towards helping patients with this debilitating disease.”

The U.S. Food and Drug Administration (FDA) recently granted Orphan Drug Designation for its investigational therapy FT011 for the treatment of systemic sclerosis (scleroderma).[4]

Certa is progressing preparations toward a pivotal clinical trial of FT011 as a treatment for scleroderma. Supported by global clinical experts, the clinical trial design and associated development plans will be discussed with the FDA in early-2024, with complementary scientific advice sought from the EMA mid-2024. The company intends to start the pivotal study in late-2024.

Professor Dinesh Khanna MD, Frederick G.L. Huetwell Professor of Rheumatology and Director, Scleroderma Program at the University of Michigan (Ann Arbor, MI, USA), a global expert in the clinical development of novel therapeutics for scleroderma said, “The mechanism of action for FT011 offers an important point of difference for the scleroderma research field as the upstream inhibition of inflammation and fibrosis may offer meaningful clinical outcomes. It is important to advance safe and effective treatments through clinical development, given the limited therapeutic options for scleroderma patients. I look forward to collaborating with the company in the FT011 development program ahead.”

Certa Therapeutics CEO and founder Professor Darren Kelly said, “We are pleased to have these exceptional clinical trial results presented to the scientific community at ACR Convergence, with FT011 demonstrating clinically important differences in multiple efficacy measures on top of standard of care in a short treatment timeframe. It is imperative that effective, safe, and well-tolerated therapeutics are efficiently developed, and which are truly beneficial for a scleroderma patient's quality of life.”

Professor Darren Kelly added, “Scleroderma is a debilitating and life-threatening condition, and extremely complex in the way that the disease manifests itself in patients. As the biological mechanism by which FT011 works precisely targets the root cause of fibrosis, we believe that FT011 is notably differentiated from previously unsuccessful clinical candidates. With the benefit of input from world-leading experts in the field, we are highly focused on advancing the clinical development program for FT011 towards the pivotal efficacy study.”

1. C Denton, W Stevens, N Kruger, M Papadimitriou, F Khong, M Bradney, D Kelly, R Lafyatis “FT011 for the Treatment of Systemic Sclerosis. Results from a Phase II Study” ACR Convergence 2023, abstract 2593

Session: Abstracts: Systemic Sclerosis & Related Disorders III: Clinical Trials
Date: Wednesday, November 15
Session Time: 11:00 AM - 12:30 PM
Presentation Time: 11:00 AM - 11:15 AM
Abstract ID: 1554380
View at - www.certatherapeutics.com

2. A formal sample size calculation for efficacy endpoints was not conducted for this study.

3. D Khanna, *et al.*, *Arthritis Rheumatol*. 2016; **68**(2): 299–311. doi:10.1002/art.39501

4. https://certatherapeutics.com/certa-therapeutics-ft011-granted-us-fda-orphan-drug-designation-for-the-treatment-of-systemic-sclerosis/

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About Systemic Sclerosis (Scleroderma)
Scleroderma is an extremely debilitating, potentially life-threatening autoimmune condition characterised by inflammation and fibrosis of the skin and other organs (commonly the lungs, kidneys, and heart). This condition results in high morbidity with substantial detriment to quality of life, with patients commonly experiencing loss of mobility and function, pain, and fatigue, often accompanied with a significant impact to their mental health. Scleroderma has the highest mortality among rheumatic diseases, associated with a 2.5-fold higher risk of mortality than the general population.

About the Phase 2 study

> *"A Phase II, randomised, double blind, placebo-controlled study of the pharmacokinetics, pharmacodynamic effects and safety of oral FT011 doses in participants with diffuse systemic sclerosis (SSc)"*
> ClinicalTrials.gov Identifier: NCT04647890

About Certa Therapeutics & FT011
Certa Therapeutics is a biotechnology company focused on improving lives by treating patients with debilitating diseases *via* novel targeted therapies. Since its inception in 2018, Certa has secured over $28m from Australian investors Brandon Biocatalyst and Uniseed.

FT011 is a novel, first-in-class oral therapy for the treatment of chronic fibrosis in multiple organs. It targets a previously important but undrugged membrane GPCR receptor with an extensive body of data demonstrating promising efficacy in multiple models of fibrotic disease. Transcriptomic research has demonstrated that treatment with FT011 results in reversal in the activation of genetic markers associated with fibrosis, providing potential for precision therapy.

These targeted drug candidates have established proof of concept as potential treatments for multiple fibrotic diseases including serious and chronic conditions impacting the kidney, lung, eye, skin, and heart. The morbidity and mortality impact of fibrotic diseases is substantial, ultimately causing 45% of all deaths globally.

Certa Therapeutics seeks to combine these innovative therapeutics with biomarkers and genetic analysis to identify those patients most likely to benefit from treatment. Significant breakthroughs are urgently needed in this field, addressing a global market worth more than US$15B annually.

FT011 is an investigational product that has not received marketing authorisation or approval by any regulatory agency, including the US Food and Drug Administration, the European Medicines Agency, or the Australian Therapeutic Goods Agency. The investigational drug products being developed by Certa Therapeutics are undergoing clinical studies to evaluate their safety and effectiveness in humans.

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