Surrogate marker and design of Calliditas Therapeutics' IgA nephropathy Phase 3 study agreed with the FDA

Stockholm, November 20, 2017. Calliditas Therapeutics AB (formerly Pharmalink AB) announced today that the US Food & Drug Administration (FDA) has accepted its Phase 3 study design for its lead candidate Nefecon based on proteinuria as endpoint for accelerated approval. A global Phase 3 program in IgA nephropathy has been initiated, and patient enrolment is expected to start in 2018.

The design for the Nefecon Phase 3 study was agreed at the latest meeting with the FDA in September 2017, building on the groundbreaking acceptance of proteinuria as a surrogate marker for accelerated approval, which the company received in an end of Phase 2b meeting in January 2017. The company believes that this was the first time the FDA granted approval for the use of proteinuria as surrogate endpoint for a Phase 3 nephrology study. The indication which Calliditas is pursuing is an orphan indication in both Europe and the US known as IgA nephropathy (IgAN).

Positive results of the concluded Phase 2b clinical trial of Nefecon in IgAN were published in The Lancet at the end of March 2017.

Renée Aguiar-Lucander, CEO of Calliditas Therapeutics, said: "I am delighted that our constructive and supportive discussions with the FDA have continued, and that we have taken another very significant step forward with the agency to ensure that we are able to start our Phase 3 study as planned. It is exciting to be pioneering the use of proteinuria as a surrogate marker for accelerated approval in this disease and we are hopeful that this will lead to the first approved drug for this patient population."

She added: "The importance of these meetings with the FDA cannot be overstated, as it allows us to proceed at full speed with our Phase 3 program which we started preparing earlier in the year. We are excited about continuing to progress our plans for initiating the clinical study as planned."

Professor Jonathan Barratt at the Department of Infection, Immunity & Inflammation, the University of Leicester, said: "Nefecon exploits a novel mode of action and truly has the potential to become the first approved treatment for IgA nephropathy".

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Notes to Editors

About Calliditas Therapeutics (formerly Pharmalink)
Calliditas Therapeutics is a specialty pharmaceutical company focused on developing high value medical products for patients with significant unmet medical needs in niche indications, in which the company can either partially or completely participate in the commercialization effort. Calliditas Therapeutics is focused on the development and commercialization of its lead product candidate Nefecon, which has successfully completed a Phase 2b clinical study as a potential new treatment for patients with an orphan designated inflammatory kidney disease, IgA nephropathy, who are at risk of progressing to renal failure. Backed by a strong investor base with a clear long-term vision and a track record of bringing products to the market, Calliditas Therapeutics aims to take Nefecon through a global Phase 3 study to commercialization. In addition, the company has identified other potential uses of Nefecon in niche indications, and will investigate the development of these to provide new treatments for patients with unmet medical needs.
Visit www.calliditas.se for further information.

About Nefecon
Nefecon is an investigational treatment for patients with IgA nephropathy (IgAN) at risk of developing end stage renal disease (ESRD). Nefecon has successfully completed a randomized, placebo-controlled Phase 2b study in 150 patients at risk of developing ESRD, under standardized blood pressure control with an angiotensin-converting enzyme inhibitor (ACEI) and/or angiotensin II receptor blocker (ARB). The Phase 3 registration trial is planned to start in 2018.

Nefecon is an oral, targeted-release and locally acting formulation, that down-regulates the disease process in the kidney through suppression of the gastrointestinal immune system thus exploiting the pivotal role the gastrointestinal tract plays in the overall immune response. Promising results indicate that treatment with Nefecon may provide clinical benefits to IgAN patients at risk of progressing to ESRD, and hence delay the need for dialysis or

transplantation. Nefecon has received orphan drug designation in IgA nephropathy by the US Food and Drug Administration (FDA) and the European Medicines Agency (EMA).

About IgA Nephropathy

IgAN (also known as Berger's disease) is an orphan disease and the most common cause of glomerulonephritis, a chronic inflammatory condition of the kidney in the developed world. It is a serious autoimmune, progressive disease that leads to decreasing kidney function over the course of ten to twenty years. The diseases can occur at any age but is commonly diagnosed when patients are in their 20s and 30s. Up to 50 per cent of the patients with IgAN will progress to ESRD, a disease state requiring dialysis or kidney transplant for survival due to insufficient kidney function, within 20 years.