

Calliditas Therapeutics granted orphan drug designation by the FDA for Autoimmune hepatitis

Calliditas Therapeutics AB (publ) ("Calliditas") today announced that the US Food and Drug Administration (FDA) has granted orphan drug designation (ODD) to the company for the treatment of Autoimmune hepatitis (AIH). More information about the ODD has been posted on www.fda.gov.

AIH is a chronic, autoimmune disease of the liver that occurs when the body's immune system attacks liver cells causing the liver to be inflamed. The disease may occur in any ethnic group and at any age but is most often diagnosed in patients between age 40 and 50. It is estimated that the patient population in the US amounts to approximately 50,000.

Today there is a significant unmet medical need related to lack of tolerability and side effects of systemic steroids which are administered to patients. The company plans to agree the regulatory pathway for this indication in consultation with the FDA later this year.

"We are very pleased to receive ODD in the US for the treatment of AIH. This confirms the high unmet medical need and further encourages us to continue to explore orphan indications in which we could leverage our existing expertise. This is a devastating disease with few medical alternatives today, which we believe is the case also in other chronic liver indications", commented Renée Aguiar-Lucander, CEO of Calliditas Therapeutics.

The company is currently running a global, pivotal Phase 3 with study for the treatment of the rare disease IgA nephropathy, and which has already obtained ODD by the FDA and the European Medicines Agency (EMA). Top line data for IgA nephropathy is expected in H2 2020.

The information in the press release is such that Calliditas Therapeutics AB (publ) is required to disclose pursuant to the EU Market Abuse Regulation. The information was submitted for publication, through the agency of the contact person set out below, at 10:15 CET on February 5, 2019.

For further information, please contact:

Mikael Widell, Head of Communications
Email: mikael.widell@calliditas.com
Telephone +46 703 11 99 60

About Calliditas

Calliditas Therapeutics is a specialty pharmaceutical company based in Stockholm, Sweden. It is focused on developing high quality pharmaceutical products for patients with a significant unmet medical need in niche indications, in which the company can partially or completely participate in the commercialization efforts. The company is focused on the development and commercialization of the product candidate Nefecon, a unique formulation optimized to combine a time lag effect with a concentrated release of the active substance budesonide, within a designated target area. This patented, locally acting formulation is intended for treatment of patients with the inflammatory renal disease IgA nephropathy (IgAN). Calliditas Therapeutics is running a global Phase 3 study within IgAN and aims to commercialize Nefecon in the US. The company is listed on Nasdaq Stockholm (ticker: CALTX). Visit www.calliditas.com for further information.

About Autoimmune hepatitis (AIH)

Autoimmune hepatitis (AIH), formerly called lupoid hepatitis, is a chronic, autoimmune disease of the liver that occurs when the body's immune system attacks liver cells causing the liver to be inflamed. Common initial symptoms include fatigue or muscle aches or signs of acute liver inflammation including fever, jaundice, and right upper quadrant abdominal pain. Individuals with autoimmune hepatitis often have no initial symptoms and the disease is detected by abnormal liver function tests.

Anomalous presentation of MHC class II receptors on the surface of liver cells, possibly due to genetic predisposition or acute liver infection, causes a cell-mediated immune response against the body's own liver, resulting in autoimmune hepatitis. This abnormal immune response results in inflammation of the liver, which can lead to further symptoms and complications such as fatigue and cirrhosis. The disease may occur in any ethnic group and at any age but is most often diagnosed in patients between age 40 and 50.

About Orphan Drug Designation (ODD)

The FDA Orphan Drug Act (ODA) provides for granting special status to a drug or biological product to treat a rare disease that affect fewer than 200,000 people in the US. Orphan drug designation qualifies the sponsor of the drug for various development incentives of the ODA, including tax credits, protocol assistance and up to seven years of US marketing exclusivity from time of approval of a Biologics License Application (BLA).