Press release
Valby, 11 September, 2026

The U.S. FDA grants orphan drug designation for Lundbeck's investigational anti-ACTH monoclonal antibody asedebart for treatment of endogenous Cushing's syndrome

- The U.S. FDA has granted Orphan Drug Designation for asedebart (Lu AG13909), Lundbeck's novel investigational anti-ACTH monoclonal antibody, for the treatment of endogenous Cushing's syndrome
- Endogenous Cushing's Syndrome is a rare endocrine disorder driven in most cases by excess adrenocorticotropic hormone, or ACTH, leading to chronic cortisol excess and substantial disease burden, with current treatment options often limited by suboptimal disease control and treatment-related complications [1–3]
- Proof-of-concept trial currently ongoing to evaluate efficacy and safety of Lu AG13909 in Cushing's disease (CD)[5]

Valby, Denmark, 11 September, 2026 – Lundbeck today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to asedebart (Lu AG13909), Lundbeck's novel investigational anti-ACTH monoclonal antibody, for the treatment of endogenous Cushing's syndrome.

Endogenous Cushing's syndrome includes both ACTH-dependent and ACTH-independent forms. ACTH-dependent Cushing's syndrome is a rare, serious endocrine disorder caused by excess secretion of adrenocorticotropic hormone (ACTH), most commonly from a pituitary tumor (Cushing's disease) and less frequently from an ectopic ACTH-secreting tumor.[1] Elevated ACTH drives increased adrenal production of glucocorticoids, mineralocorticoids and androgens, disrupting normal physiological homeostasis. Of particular importance is sustained cortisol excess, which contributes to a considerable disease burden through metabolic, cardiovascular and neuropsychiatric complications and is associated with increased morbidity and mortality.[1,2] Although existing medical therapies can reduce or control elevated cortisol levels, important treatment gaps remain.[1,3] Achieving and maintaining adequate disease control can be difficult, with available therapies differing in their efficacy and potentially constrained by safety and tolerability considerations.[4]

Asedebart is a novel investigational monoclonal antibody targeting ACTH and is being developed for ACTH-dependent Cushing's syndrome. It is advancing in clinical development as a potential first-in-class treatment for rare conditions characterized by excess ACTH, with proof-of-concept trials ongoing in CD and classic congenital adrenal hyperplasia (CAH) to evaluate efficacy and safety.[5–8]

With this designation, asedebart continues to build momentum with regulatory authorities across rare ACTH-driven disorders. Asedebart has also received orphan designation in the European Union for Cushing's syndrome of endogenous origin, in addition to previous orphan drug designations for CAH in the European Union and the United States, and for CAH and CD in Japan.[6,9,10]

"The FDA Orphan Drug Designation is an important step for asedebart and for Lundbeck's growing commitment to rare neuroendocrine disorders" said Tarek Samad, Executive Vice President and Head of Research & Development at Lundbeck. "ACTH-dependent Cushing's syndrome can be a devastating condition for patients, with long-term consequences that remain difficult to control despite available treatments. This milestone reflects the strength of the science behind asedebart's development to date and supports our ambition to advance innovative medicines in areas where patients continue to face significant unmet need."

Orphan Drug Designation is granted by the US FDA to drugs and biologics intended to treat, diagnose or prevent rare diseases or conditions. The designation may provide certain development incentives, including tax credits for qualified clinical testing, exemption from certain FDA application fees and, if approved, potential seven years of market exclusivity for the designated indication.[11]

Asedebart is an investigational compound that is not approved for marketing by any regulatory authority worldwide, and its efficacy and safety have not been established.

About asedebart

Asedebart is a humanized anti-ACTH monoclonal antibody that specifically recognizes ACTH with high affinity. It blocks the binding of ACTH to the melanocortin 2 receptor in the adrenal glands and thereby inhibits the neurohormonal signalling of ACTH. This inhibition reduces secretion of glucocorticoids, mineralocorticoids and androgens from the adrenal glands.[12,13]

ACTH plays a key role in the biosynthesis of adrenal steroids[13] and is therefore considered a promising therapeutic target in conditions characterized by elevated ACTH levels. Through its mechanism of action, asedebart has the potential to treat conditions associated with chronically elevated ACTH levels, such as CD and CAH.

About ACTH-dependent Cushing's syndrome

ACTH-dependent Cushing's syndrome is a rare, serious endocrine disorder caused by excess secretion of adrenocorticotropic hormone (ACTH), most commonly from a pituitary tumor (Cushing's disease) and less frequently from an ectopic ACTH-secreting tumor.[1] Chronic cortisol excess is associated with substantial disease burden, including metabolic, cardiovascular and neuropsychiatric complications, and is linked to increased morbidity and mortality.[1,2] Surgery to remove the source of excess ACTH is the preferred first-line treatment when feasible; however, not all patients are eligible for surgery or achieve sustained remission. Current medical therapies can help manage cortisol excess, but disease control remains challenging and treatment options may be limited by variable efficacy, safety and tolerability.[1,3]

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About H. Lundbeck A/S

Lundbeck is a biopharmaceutical company focusing exclusively on brain health. With more than 70 years of experience in neuroscience, we are committed to improving the lives of people with neurological and psychiatric diseases.

Brain disorders affect a large part of the world's population, and the effects are felt throughout society. With the rapidly improving understanding of the biology of the brain, we hold ourselves accountable for advancing brain health by curiously exploring new opportunities for treatments.

As a focused innovator, we strive for our research and development programs to tackle some of the most complex neurological challenges. We develop transformative medicines targeting people for whom there are few or no treatments available, expanding into neuro-specialty and neuro-rare from our strong legacy within psychiatry and neurology.

We are committed to fighting stigma and we act to improve health equity. We strive to create long term value for our shareholders by making a positive contribution to patients, their families and society as a whole.

Lundbeck has more than 5,000 employees in more than 20 countries and our products are available in more than 80 countries. For additional information, we encourage you to visit our corporate site www.lundbeck.com and connect with us via LinkedIn.

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