Press release
Valby, 24 August, 2026

European Commission grants orphan designation for Lundbeck's investigational anti-ACTH monoclonal antibody asedebart for Cushing's syndrome of endogenous origin

- The European Commission has granted orphan designation for asedebart (Lu AG13909), Lundbeck's novel investigational anti-ACTH monoclonal antibody, for the treatment of Cushing's syndrome of endogenous origin
- Cushing's syndrome of endogenous origin 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[3]
- Proof-of-concept trial currently ongoing to evaluate efficacy and safety in Cushing's disease (CD)

Valby, Denmark, Monday 24 August 2026 – Lundbeck today announced that the European Commission has granted orphan designation to asedebart (Lu AG13909), Lundbeck's novel investigational anti-ACTH monoclonal antibody, for the treatment of Cushing's syndrome of endogenous origin.

Cushing's syndrome of endogenous origin 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]

This increase in ACTH causes the adrenal gland to produce higher levels of steroid hormones, glucocorticoids, mineralocorticoids and androgens, leading to a wide range of homeostatic imbalances. One major change is chronic excess of the glucocorticoid, cortisol, which is associated with substantial disease burden, including metabolic, cardiovascular and neuropsychiatric complications, and is linked to increased morbidity and mortality.[1,2] Current medical therapies can help manage cortisol excess, however significant unmet medical need remains. Disease control remains challenging and treatment options may be limited by variable efficacy, safety and tolerability.[1,3]

Asedebart is a novel investigational monoclonal antibody targeting ACTH and is being developed for ACTH-dependent forms of 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.[5-7]

With this designation, asedebart continues to build regulatory momentum across rare ACTH-driven disorders. Previous orphan designations include CAH in the European Union and the United States, and CAH and CD in Japan.

"Orphan designation in the European Union is an important recognition of both the unmet need in Cushing's and the scientific rationale behind asedebart," said Johan Luthman, Executive Vice President, R&D, Lundbeck. "The asedebart program is a good representation of the type of strong targeted mechanism programs we like to advance in Lundbeck. The orphan designation in Cushing's syndrome for asedebart is also another example of a breakthrough innovation pipeline in neuroendocrine and rare disorders, where several programs have been granted a number of special regulatory designations in recent years."

In the European Union, orphan designation is intended to support the development of medicines for rare, life-threatening or chronically debilitating diseases. Designated orphan medicines may benefit from incentives including protocol assistance, fee reductions and, if approved, ten years of market exclusivity for the designated indication.[8,9]

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.[8,9]

ACTH plays a key role in the biosynthesis of adrenal steroids[10] 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.

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 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]

Contacts

Anders Crillesen
Senior Director, External & Internal Relations
AECE@lundbeck.com
+45 27 79 12 86

Jens Høyer
Vice President, Head of Investor Relations
JSHR@lundbeck.com
+45 30 83 45 01

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.

References:

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