Press release
Valby, 18 May, 2026

Lundbeck receives orphan drug designation in Japan for asedebart for the treatment of patients with congenital adrenal hyperplasia and Cushing's disease

- Congenital adrenal hyperplasia (CAH) and Cushing's disease (CD) are rare disorders characterized by excess adrenocorticotropic hormone (ACTH) produced in the brain[1,2]
- Current treatments are limited by suboptimal disease control and treatment-related complications[3,4]
- Asedebart (Lu AG13909) is a novel, investigational anti-ACTH monoclonal antibody targeting underlying disease pathology
- Proof-of-concept trials are currently ongoing to evaluate efficacy and safety of asedebart in CD and CAH

Valby, Denmark, 18 May 2026 – Lundbeck today announced that orphan drug designation (ODD) has been granted in Japan by the Ministry of Health, Labour and Welfare (MHLW) for asedebart (Lu AG13909) for the treatment of patients with congenital adrenal hyperplasia (CAH) and Cushing's disease (CD).

CAH and Cushing's disease are rare disorders with distinct origins and clinical features, but a shared underlying mechanism featuring elevated levels of ACTH.[1,2] In CAH, impaired cortisol production leads to chronically elevated ACTH levels, resulting in excess adrenal androgen production and requiring lifelong hormone replacement therapy.[1] In contrast, Cushing's disease is caused by an overproduction of ACTH by a pituitary adenoma (tumor), leading to chronic cortisol excess.[2]

CAH affects approximately 1 in 14,000–18,000 live births worldwide, while Cushing's disease has a global prevalence of ~2.2 cases per 100,000 people.[5,6] Both conditions are associated with substantial disease burden, including metabolic, cardiovascular, and neuropsychiatric complications, and are linked to increased morbidity and mortality.[3,7] Current treatment approaches aim to manage hormonal imbalance but are associated with important limitations such as variable efficacy and tolerability.[3,4]

"CAH and Cushing's disease are serious, chronic conditions that can have a profound impact on patients' lives," said Johan Luthman, EVP and Head of Research and Development at Lundbeck. "The investigational program for asedebart demonstrates Lundbeck's commitment to deepen our efforts in rare diseases and endocrinological conditions with links to brain function. This program also illustrates our approach to directly target underlying disease biology. As one of our focus markets, we are especially pleased to receive ODD for asedebart in Japan and the opportunity this provides to address significant unmet needs for people living with CAH and CD."

Asedebart, a novel monoclonal antibody targeting ACTH is advancing in clinical development as a potential first-in-class treatment for conditions characterized by excess ACTH. By targeting an

underlying disease mechanism, asedebart aims to offer a new treatment approach to managing CAH. Asedebart, formerly designated Lu AG13909, has recently been assigned an International Nonproprietary Name (INN) and has received Orphan Drug Designation for CAH in both the European Union and the United States. Lundbeck is currently conducting proof-of-concept clinical trials evaluating the efficacy and safety of asedebart in patients with classic CAH and CD.

Asedebart is an investigational compound that is not approved for marketing by any regulatory authority worldwide, and the efficacy and safety of asedebart 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 causes a decreased 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.[9] In this context, asedebart, a novel molecule, may provide a therapeutic approach for treating conditions associated with chronically elevated ACTH levels.

About congenital adrenal hyperplasia

Classic CAH is a rare, autosomal recessive disorder[11] affecting 1 in 14,000–18,000 live births worldwide.[5] Classic CAH is characterized by an enzyme deficiency, most commonly 21-hydroxylase deficiency, affecting the adrenal steroidogenesis, leading to cortisol and aldosterone deficiency. People with 21-hydroxylase deficiency are at risk of adrenal crisis, a life-threatening condition contributing to the increased mortality throughout life.[12] Balancing physiological glucocorticoid replacement and control of hyperandrogenism remains a challenge with the risk of long-term consequences of glucocorticoid overtreatment.[13-15]

About Cushing's disease

Cushing's disease is a rare endocrine disorder caused by a pituitary adenoma that secretes excess ACTH, leading to chronic overproduction of cortisol.[2] The condition is associated with significant morbidity and increased mortality, and patients may experience a wide range of physical and neuropsychiatric symptoms.[7] First-line treatment is surgical removal of the tumor; however, not all patients are eligible or achieve sustained remission. Current medical treatment options have variable efficacy and may be associated with safety and tolerability limitations, highlighting an ongoing unmet need for effective and well-tolerated therapies.

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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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