Press release

BioArctic receives Orphan Drug Designation for exidavnemab the US

Stockholm, March 17, 2025 – BioArctic AB (publ) (Nasdaq Stockholm: BIOA B) announced today that the US FDA Office of Orphan Products Development (OOPD) has granted orphan drug designation (ODD) to exidavnemab for the treatment of Multiple System Atrophy (MSA), providing incentives for the development of treatments for rare diseases with a high medical need.

Multiple System Atrophy (MSA) is a rapidly progressive and fatal rare disease affecting the central and autonomic nervous systems. MSA is characterized by pathological alpha-synuclein aggregation, that causes gradual damage to nerve cells in the brain. This affects balance, movement and the autonomic nervous system, which controls several basic functions, such as breathing, digestion and bladder control. Currently there is no cure and no available treatment to slow its progression.

Exidavnemab is being developed as a novel disease-modifying treatment for synucleinopathies such as MSA and Parkinson's disease. It is a monoclonal antibody (mAb) that selectively targets soluble alpha-synuclein aggregates, such as oligomers or protofibrils. By promoting the clearance of aggregated alpha-synuclein, exidavnemab may reduce the spreading and the negative effects of alpha-synuclein. Thereby, neuronal function and survival may be preserved, and disease progression ultimately slowed down.

The FDA's Orphan Drug Designation program provides orphan status to drugs or biologics intended for the treatment of diseases that affect fewer than 200,000 people in the United States. Sponsors of medicines that are granted Orphan Drug Designation are entitled to certain incentives and regulatory assistance, including tax credits for qualified clinical trials, prescription drug user-fee exemptions, and potential seven-year marketing exclusivity upon FDA approval.

This release discusses investigational uses of an agent in development and is not intended to convey conclusions about efficacy or safety. There is no guarantee that such investigational agents will successfully complete clinical development or gain health authority approval.

This information is information that BioArctic AB (publ) is obliged to disclose pursuant to the EU Market Abuse Regulation. The information was released for public disclosure, through the agency of the contact person below, on March 17, 2025, at 18:25 CET.

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About MSA
Multiple System Atrophy (MSA) is a rapidly progressive and fatal rare disease affecting the central and autonomic nervous systems. MSA is a synucleinopathy, a group of neurodegenerative diseases characterized by an abnormal alpha-synuclein aggregation, that causes gradual damage to nerve cells in the brain. This affects balance, movement and the autonomic nervous system, which controls several basic functions, such as breathing, digestion and bladder control. Currently there is no cure and no available treatment to slow its progression.

MSA is a condition with very high unmet medical need and poor prognosis. Currently, no cure or treatment is available to slow the progression of the disease. Patients typically live about 6 to 10 years after MSA symptoms first appear, with few patients surviving more than 15 years[1,2]. MSA is significantly debilitating and classified as a rare disease, affecting less than 42,000 persons in the U.S.

About Exidavnemab
Exidavnemab is a monoclonal antibody drug candidate that is designed to selectively bind and eliminate aggregated forms of alpha-synuclein such as oligomers and protofibrils and fibrillar forms, which participates in neurodegenerative disorders including Parkinson's disease and MSA. The goal is to develop a disease modifying treatment that stops or slow down the progression of Parkinson's disease and MSA.

About BioArctic AB
BioArctic AB (publ) is a Swedish research-based biopharma company focusing on innovative treatments that can delay or stop the progression of neurodegenerative diseases. The company is the originator of Leqembi® (lecanemab) – the world's first drug proven to slow the progression of the disease and reduce cognitive impairment in early Alzheimer's disease. Leqembi has been developed together with Eisai. BioArctic has a broad research portfolio within Alzheimer's disease, Parkinson's disease, ALS and enzyme deficiency diseases. Several of the projects utilize the company's proprietary BrainTransporter™ technology, which improves the transport of drugs into the brain. BioArctic's B share (BIOA B) is listed on Nasdaq Stockholm Large Cap. For more information, please visit www.bioarctic.com.

[1] Jellinger KA. J Alzheimers Dis. 2018;62(3):1141-1179.

[2] Jellinger et al. Biomedicines. 2022 Mar 3;10(3):599.