BrainEver Receives FDA Orphan Drug Designation for BREN-02, (human recombinant Engrailed 1) for the Treatment of Amyotrophic Lateral Sclerosis (ALS)

Paris, France, November 23th, 2020 - BrainEver, a biotechnology company dedicated to the research and development of innovative therapies for the treatment of neurodegenerative diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to its product BREN-02, the recombinant human homeoprotein Engrailed 1 (rhEN1), for the treatment of amyotrophic lateral sclerosis (ALS).

ALS (also known as Charcot disease) is a severe neurodegenerative disease affecting motor neurons, resulting in progressive muscle weakness leading to paralysis, with a vital prognosis of approximately 2-4 years from onset. ALS is diagnosed in approximately 16,000 people each year in Europe and North America.

"In ALS, motor neurons degenerate and die, leading to progressive muscle paralysis that includes respiratory muscles. In preclinical studies, we have observed that the intrathecal lumbar administration of rhEN1, in the early symptomatic stages, restores muscle function and prevents motor neuron death", said Prof. Alain Prochiantz, neurobiologist at the Collège de France and co-founder of BrainEver:

BREN-02 is expected to be administered in clinical trials in ALS patients in the second half of 2021, subject to preclinical toxicity results and regulatory review. Preclinical studies have shown that the homeoprotein hEN1 is essential for the survival and maintenance of spinal cord alpha motor neurons that innervate muscles throughout the body.

The FDA grants orphan drug status to encourage the development of therapies to treat, prevent or diagnose diseases or conditions affecting fewer than 200,000 people in the United States. Orphan drug designation in the United States recognizes the therapeutic potential of BREN02 in ALS and allows BrainEver to benefit from specific measures and advantages, including a seven-year marketing exclusivity period if hEN1 is approved for the treatment of ALS.

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About BrainEver

Founded in 2016 by Prof. Alain Prochiantz and Bernard Gilly, BrainEver is a biotechnology company specialized in the discovery and development of innovative treatments for neurodegenerative diseases. BrainEver harnesses the intrinsic properties of homeoproteins, a family of transcription factors that are essential during embryonic and postnatal development. Recent studies have also demonstrated that homeoproteins play a key role in the maintenance and survival of specific neuronal populations in adults. An important property of homeoproteins is their ability to penetrate cells, with privileged access to cytoplasmic and nuclear compartments. Therefore, they do not require any transportation system to reach the target cells, nor do they require an expression viral vector. BrainEver expects to initiate its first human clinical study in ALS in the year 2021. BrainEver has raised 21M € from iBionext, bpifrance and Turenne Capital. **To learn more visit** https://brainever.com/news/

About Engrailed-1 in ALS

Preclinical studies have demonstrated that the homeoprotein EN1 plays an essential role in the survival and maintenance of motor neuron-alpha spinal cells that innervate skeletal muscles. In ALS, these motor neurons degenerate and die, resulting in progressive muscle paralysis that includes respiratory muscles. The administration of BREN-02 by intrathecal injection in the lumbar region restores muscle function and prevents motor neuron death in preclinical models. ALS is diagnosed in approximately 16,000 people each year in Europe and North America.