



Ceres Brain Therapeutics is a French clinical-stage biotechnology company developing **CBT101**, a first-in-class central nervous system (CNS) therapeutic designed to restore **neuronal energy homeostasis**. The company focuses on neurological diseases where impaired mitochondrial function and cellular bioenergetics drive disease progression.

Lead Product: CBT101

CBT101 is a **patented intranasal creatine prodrug**, specifically engineered to deliver creatine **directly into neurons via the nose-to-brain pathway**, bypassing the blood–brain barrier. Creatine is a key natural regulator of neuronal energy metabolism but cannot be efficiently supplemented in the brain neurons with oral formulations. CBT101 addresses this long-standing limitation.

Clinical Indications - two neurological indications with major unmet medical needs

- **Creatine Transporter Deficiency (CTD):** A rare genetic neurological disorder characterized by the absence of creatine in neurons leading to autism, severe intellectual deficiency, and seizures. CBT101 is designed to restore **intracellular neuronal creatine**, addressing the primary metabolic cause of the disease.
→ **Orphan Drug Designation (FDA & EMA) & Rare Pediatric Drug Designation (FDA)**
 - **Amyotrophic Lateral Sclerosis (ALS):** A progressive and fatal neurodegenerative disease associated with mitochondrial dysfunction and brain hypometabolism. In ALS, CBT101 targets **neuronal and mitochondrial energy failure** contributing to motor neuron degeneration.
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Validation & Development Status

- Strong preclinical evidence demonstrating **brain neuronal delivery of creatine** after nasal dosing.
 - Neuroprotective and metabolic efficacy shown in CTD and ALS-relevant models.
 - **Phase 1 completed** in healthy volunteers with good tolerability and **demonstrated CNS biological activity**, including **qEEG modulation (increase in beta-band power)**.
 - **Phase 2 clinical development starting** in ALS and CTD.
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Competitive Advantages

- First therapy designed to **deliver creatine directly into neurons**
 - **Non-invasive nasal spray** improving patient convenience and adherence
 - Proprietary **nose-to-brain delivery technology** & platform potential for additional neurological indications
 - Strong and extensible **intellectual property portfolio**
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Investment & Partnering

Ceres Brain Therapeutics is currently **seeking to raise USD 20 million** to advance Phase 2 clinical programs in ALS and CTD and is actively **exploring strategic partnerships** (co-development, licensing, regional collaborations).