

Carthera receives Orphan Drug Designation from US and EU authorities for carboplatin in malignant glioma

Orphan designation shows recognition of enhanced therapeutic potential of carboplatin when used with Carthera's SonoCloud® technology

EMA and FDA ODD status provides further support from regulatory authorities for Carthera's innovative approach to treating glioblastoma

Paris, France, May 14, 2024 – Carthera, a spin-off from Sorbonne University founded by Pr. Alexandre Carpentier, and developer of SonoCloud®, an innovative ultrasound-based medical device to treat a wide range of brain disorders, today announces the granting of Orphan Drug Designation (ODD) by the US Food and Drug Administration (FDA) and the European Medicines Agency (EMA) for the use of carboplatin in the treatment of malignant glioma.

The [FDA's ODD program](#) provides orphan status to drugs and biologics intended for the treatment, prevention or diagnosis of a rare disease or condition; those affecting less than 200,000 people in the US. The EMA's orphan designation is targeted at conditions that affect no more than five in 10,000 people in the EU. Both offer unique opportunities for Carthera to extend its market exclusivity in the US and EU.

Carthera is using carboplatin in the first registrational trial of its SonoCloud technology in the treatment of recurrent glioblastoma (rGBM). It is a randomized, multicenter, two-arm clinical trial with a 1:1 ratio, aiming to recruit 560 patients across 40 sites in Europe and the US. The open-label, comparative pivotal trial will evaluate overall survival in patients undergoing carboplatin chemotherapy and treated with the SonoCloud-9 system to open the Blood-Brain Barrier (BBB). This will be compared to the standard of care (lomustine and temozolomide) in patients with first recurrence of GBM.

"We are thrilled to receive orphan drug designation from the European and US authorities. This is an important milestone for the company as it encourages us to push ahead with our pivotal trial using carboplatin to treat recurrent glioblastoma," said Sandra Thiollière, head of regulatory affairs at Carthera.

Carboplatin has been chosen for its proven anti-tumoral activity and has already been tested as a monotherapy in patients with glioblastoma. It shows limited or no Central Nervous System (CNS) toxicity at high doses or in direct exposure. However, it has limited penetration through the BBB when administered using standard intravenous (IV) routes. Brain concentrations of carboplatin can be significantly enhanced when coupled with the SonoCloud-9 system to temporarily disrupt the BBB. Preliminary safety and efficacy results for combining the SonoCloud-9 with carboplatin (NCT03744026) have recently been published in [Nature Communications](#).

"This is an important milestone for Carthera, with both US and European authorities recognizing the significance of brain treatments when combined with our SonoCloud device. We believe that our technology can help harness the therapeutic efficacy of carboplatin to enhance the treatment of glioblastoma patients," said Frédéric Sottolini, CEO of Carthera.

Glioblastoma (GBM) is one of the most complex, deadly and treatment-resistant cancers, with [more than 10,000 people in the US](#) and [15,000 in the EU](#) succumbing to the disease every year.

Carthera recently enrolled the [first patients in its pivotal trial](#) (NCT05902169) in Europe and in the US, with a projected recruitment duration of two years. Interim analysis will be conducted over the course of the study. In parallel, investigator sponsored trials with different therapeutic agents will confirm the potential of the SonoCloud platform.

About SonoCloud®

SonoCloud is an innovative medical device developed by Carthera. It emits ultrasound to temporarily increase the permeability of the blood vessels in the brain to improve the delivery of therapeutic molecules. Invented by Pr. Alexandre Carpentier and developed in collaboration with the Laboratory of Therapeutic Applications of Ultrasound (Laboratoire Thérapie et Applications Ultrasonores, LabTAU, INSERM) in Lyon, France, SonoCloud is an implant inserted into the skull and activated prior to injection of a therapeutic agent. Several minutes of low-intensity ultrasound opens the blood-brain barrier for six hours and increases the concentration of therapeutic molecules in the brain. This ultrasound-induced opening of the blood-brain barrier is a world first; it offers a new treatment option for a wide range of indications, including brain tumors and Alzheimer's disease.

SonoCloud is an investigational product, the device has not yet received EMA or FDA approval.

About Carthera

Carthera is a clinical-stage medtech company focused on developing innovative ultrasound-based medical devices to treat a wide range of brain disorders.

The company is a spin-off from AP-HP Paris and Sorbonne University. Carthera leverages the inventions of Pr. Alexandre Carpentier, head neurosurgeon at AP-HP Sorbonne university, who has achieved worldwide recognition for his innovative developments in treating brain disorders. Carthera is developing SonoCloud®, an intracranial implant that temporarily opens the Blood-Brain Barrier (BBB). The device is currently in clinical trials in Europe and the United States. It received FDA Breakthrough Device Designation in 2022.

Founded in 2010 by Pr. Alexandre Carpentier, run by CEO Frederic Sottolini and chaired by Oern Stuge MD, Carthera has offices in France (Lyon and Paris) and a subsidiary in Boston, Massachusetts, USA. Since its inception, the technical and clinical development of SonoCloud has received support from the National Research Agency (ANR), the French public investment bank (Bpifrance), the National Institutes of Health (NIH) and the European Innovation Council (EIC).

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