Hed: FDA Grants Amphix Bio’s AMFX-200 Orphan Drug Designation

Dek: Amphix Bio led breakthrough of regenerative spinal cord injury treatment.

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Amfx-200 received FDA Orphan Drug designation.

Amphix Bio’s treatments are breakthroughs in regenerative treatment options.

The FDA and Amphix Bio have formed regulatory moment for their medical breakthroughs.

Amphix Bio announced its leading treatment for spinal cord injuries (SCI) Amfx-200 received the Orphan Drug designation from the U.S. Food and Drug Association (FDA). An estimated 18,000 cases of SCI are reported annually in the U.S. and are labeled as a devastating health condition often resulting in permeant paralysis. Amphix Bio elected to combat against this with its SCI treatment Amfx-200.

Dr. James Guest, neurosurgeon at the University of Miami and the Miami Project to Cure Paralysis spoke on the impact SCIs have on patients and patients’ families saying, "Experiencing a spinal cord injury is devastating for patients and their families, as it leads to a lifetime of lost mobility, high healthcare costs, and a heavy reliance on caregivers. Previous attempts at treatments have faced many limitations but Amphix Bio is taking a completely new approach to neural regeneration, with the potential for a breakthrough in the field."**1**

**The backbone of Amfx-200**

The treatment, based on Supramolecular Therapeutic Peptides (STPs), allows peptides to function as a drug that activates cell receptors and as a physical nanofiber scaffold that aids tissue regeneration. STPs were engineered by Professor Samuel Stupp’s research group at Northwestern University, with the technology making a key breakthrough in 2021. The breakthrough discovered the power to control collective motion of nanofiber molecules, which enhanced biological potency and stimulated neural regeneration.

Amphix Bio expects to leverage its STP technology to advance regenerative therapeutics for patients suffering from neurological and musculoskeletal conditions. Planning to begin with established biology and documented high-impact areas of cellular regeneration, Amphix Bio expects to combine scientific discovery with clinically viable therapeutics.

Preclinical models of acute SCI are treated with a single injection of Amfx-200 into the spinal cord. This injection allows motor neurons from the brain to regrow past the injury site, repairing severed connections and restoring motor functions. This treatment effectively reverses paralysis in the injected area establishing the treatment as the first of its kind.

Stupp, chief scientific officer of Amphix Bio touched on the impact of both treatments, saying, “Since we first published these results in 2021, we have further validated the ability of these molecules to regenerate functional neural tissue in additional preclinical models, different types of spinal cord injury, and in other neural injuries and neurodegenerative diseases, this represents a paradigm shift in the development of neural regeneration therapeutics."**1**

Amfx-200 isn’t Amphix Bio’s first regenerative therapeutic option for SCIs.Amfx-200’s precursor Amfx-100, an off-the-shelf bone graft substitute treatment for spinal fusion surgery, enables orthopedic surgeons to perform spinal fusion procedures without donor tissue or recombinant proteins.

In October 2024, Amfx-100 received the FDA’s Breakthrough Device designation, a program designed to accelerate the development, assessment, and review of new medical devices.

Stupp touched on Amphix Bio’s treatments receiving FDA designations saying, "This designation from the FDA is a major milestone for supramolecular therapeutics and validates the high unmet need that our approach addresses. "The expedited assessment and review are especially important given that we are aiming to advance an entirely new regenerative medicine platform to the clinic." **2**

**Sources:**

Amphix Bio receives FDA Orphan Drug Designation for neural regeneration therapeutic to treat acute spinal cord injury *Amphix Bio* July 16, [2025 https://www.prnewswire.com/news-releases/amphix-bio-receives-fda-orphan-drug-designation-for-neural-regeneration-therapeutic-to-treat-acute-spinal-cord-injury-302506799.html](https://www.prnewswire.com/news-releases/amphix-bio-receives-fda-orphan-drug-designation-for-neural-regeneration-therapeutic-to-treat-acute-spinal-cord-injury-302506799.html)

FDA grants Breakthrough Device designation to Amphix Bio's bone regeneration product *Amphix Bio* October 3, 2025 <https://www.prnewswire.com/news-releases/fda-grants-breakthrough-device-designation-to-amphix-bios-bone-regeneration-product-302267126.html#:~:text=CHICAGO%2C%20Oct.,donor%20tissue%20or%20recombinant%20proteins.>