

Title (en)

4 AMINOPYRIDINE AS A THERAPEUTIC AGENT FOR SPINAL MUSCULAR ATROPHY

Title (de)

4-AMINOPYRIDIN ALS THERAPEUTISCHES MITTEL FÜR SPINALE MUSKELATROPHIE

Title (fr)

4-AMINOPYRIDINE À TITRE D'AGENT THÉRAPEUTIQUE POUR L'AMYOTROPHIE SPINALE (SMA)

Publication

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Application

EP 13769101 A 20130326

Priority

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Abstract (en)

[origin: WO2013148740A1] It has been discovered that pharmacological inhibition of K⁺ channels (using the FDA-approved broad-spectrum K⁺ channel antagonist 4-AP) positively benefitted smn mutant phenotypes, a result that is consistent with the defective excitability of motor circuits by their interneuron or sensory neuron inputs being a critical consequence of SMN depletion. Based on these observations, certain embodiments of the invention are directed to methods of treatment of SMA by administering therapeutically effective amounts of one or more potassium channel antagonists, including 4-aminopyridine, 4-(dimethylamino)pyridine, 4-(methylamino)pyridine, and 4-(aminomethyl)pyridine. Other embodiments are directed to new pharmaceutical formulations comprising two or more potassium channel antagonists.

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