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## LICENSING OPPORTUNITIES

Fondazione I.R.C.C.S.
Istituto Neurologico Carlo Besta

Sistema Socio Sanitario
Regione Lombardia

# PROTEINS AND PEPTIDES FOR PREVENTION AND CURE OF NEURODEGENERATIVE DISEASES

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Applications:
- Use of cellular models to study the molecular mechanisms of a-SMN axonogenic properties.
- Creation of animal models to study the pathogenesis of motor neuron degeneration in SMA and other neurodegenerative diseases characterized by motor neuron death.
- Production of antibodies potentially useful in diagnosis in SMA and in other human or animal pathologies characterized by motor neuron degeneration.
- Generation of viral vectors for genetic therapy protocols both in animal models and in patients.

Key benefits:
- Overexpression of a-SMN in vitro induced a strong functional effect on axonal growth, both in neuronal and non-neuronal cells.
- Silencing of a-SMN in neuronal cultures caused axon specification impairment, leading to neurons with multiaxons or many neurites with mixed axonal and dendritic markers.
- In a mouse SMA model, intracerebroventricular (ICV) treatment with an adenoviral vector encoding a-SMN showed high protein expression both in spinal cord and brain, and a delay in the disease onset compared to non-treated mice.

Offer:
- Licensing out.
- Co-Development.

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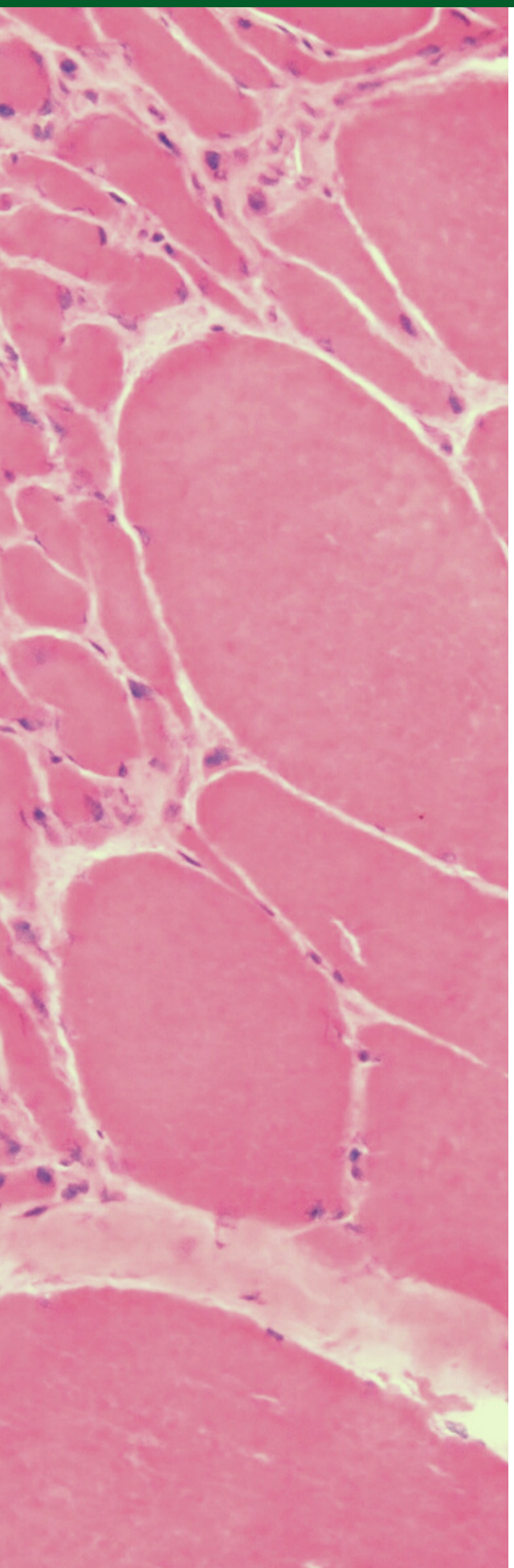

## PROTEINS AND PEPTIDES FOR PREVENTION AND CURE OF NEURODEGENERATIVE DISEASES

INVENTION

A new protein isoform of SMN displays strong axonogenic effect and acts as master-switch in axonal differentiation, opening new perspectives in neurodegenerative disease investigation.

BACKGROUND

Spinal Muscular Atrophy (SMA) is a severe pathology characterized by selective motor neuron degeneration, which leads to progressive muscle paralysis and, in the worse cases, to respiratory failure and death. SMA, caused by mutations in SMN1 gene, is the leading genetic cause of infant mortality, with an incidence of 1 in 6.000-10.000 newborns. In the last years, novel therapies targeted to increase SMN protein levels have revolutionized the approach to SMA. Yet, the timing of therapy administration plays a crucial role in attaining the maximal response from the treatment, which have limited efficacy in already symptomatic patients. Moreover, not all the patients respond equally to therapy. There is thus compelling need of prognostic biomarkers to predict the progressions of patients and of new targets for additional treatments to improve effective therapeutic strategies for SMA patients.

TECHNOLOGY

Axonal impairment of motor neurons is recognized as early event in SMA, yet the mechanisms that lead to neurodegeneration are still to be identified. The inventors describe a new transcript of SMN gene, encoding the protein a-SMN, selectively expressed in motor neurons and especially localized in axons, mainly in the early phases of development. a-SMN displays a strong axonogenic effect. It is also needed for the regulation of neuronal polarization and the correct organization of axonal and dendritic compartments, that are vital for neuronal function and survival. In a mouse SMA model, ICV treatment with adenoviral vector expressing a-SMN virus showed high protein expression both in spinal cord and brain, and a delay in the disease onset. This invention opens new perspectives in the definition of novel therapeutic strategies for neurodegenerative diseases.

INVENTORS

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INTELLECTUAL PROPERTY RIGHTS

Patent granted in Italy.

OFFER

Licensing out & co-development.

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