

## NEW THERAPEUTIC APPROACH AGAINST SPINAL MUSCULAR ATROPHY (SMA)

New therapeutic approach against SMA via a new mode of action: relocation of the SMN protein in order to restore its function, by using Flunarizine.

### APPLICATIONS

- Therapy against SMA
- Possible combination with therapeutics aiming at increasing SMN levels

### DEVELOPMENT PHASE

- *In vivo* evidence of SMN relocation into Cajal Bodies following Flunarizine treatment
- *In vivo* evidence of muscular default correction following Flunarizine treatment
- Ongoing: neuroprotective effect analysis, molecular and mechanistic studies

### INTELLECTUAL PROPERTY

International patent application  
WO2014044972 (EP, US, CA)

### CONTACT

Mail : dda@idfinnov.com  
Phone : 01 80 05 65 92

Spinal Muscular Atrophy ■ SMA ■ SMN ■  
Relocation ■ Flunarizine ■ Rare disease

### PRESENTATION

Spinal muscular atrophy (SMA) is a neurodegenerative disorder leading to progressive muscle weakness, atrophy, and in severe types, premature death. It is classified as **a rare disease** affecting 1 in 6000 births. SMA is caused by the mutation or deletion of the survival motor neuron-1 (SMN1) gene, which encodes the SMN protein, necessary for the survival of motoneurons. Several drug candidates aim at motoneuron protection or SMN protein levels increase. However, until now, no treatment is available against SMA.

It has been shown that SMN localizes in Cajal Bodies and that the localization is affected in SMA patients. **The present offer relates to a new therapeutic approach based on relocation of SMN into Cajal Bodies to restore its function and improve the SMA phenotype.** Through an *in vitro* screening on SMA patients' cells, Flunarizine has been identified as a hit candidate to relocate and restore SMN function. *In vivo* studies in a mouse model show relocation of SMN into Cajal Bodies as well as a strong improvement of survival and mobility. More recent studies show effects on both muscles and neurons (results available under NDA) and ongoing research will elucidate the details of the mechanism of action.



### COMPETITIVE ADVANTAGES

- New therapeutic approach based on relocation of SMN protein
- Well known compound (Flunarizine) as hit candidate