

### Acronym

B-SMART

### Full Title

Brain-Specific, Modular and Active RNA Therapeutics

### Programme

H2020 – NMBP-10-2016 – Nanoformulation of biologicals

### Contract Number

721058

### Abstract

The overall objective of B-SMART is to:

1. design modular nanoparticles,
  2. manufacture them via a quality-by-design protocol,
  3. achieve delivery of therapeutic RNAs to the brain and treat neurodegenerative diseases.
- I. To design modular nanoparticles consisting of
    - an active RNA payload
    - established (lipid-based), emerging (trigger-responsive polymer-based) or exploratory (extracellular vesicle-based) nanoparticles
    - a targeting ligand consisting of the variable domain of heavy chain only antibodies - also known as VHHs or nanobodies, which are coupled to the carrier platform.
  - II. To manufacture the modular nanoparticles using a microfluidic assembly system that will ensure quality-by-design: uniform nanoparticles across research sites and excellent control over the physico-chemical parameters.
  - III. To test pre-clinical activity of formulations with promising in vitro activity with good cell/blood compatibility and to select the best RNA-formulation for clinical translation to treat neurodegenerative diseases. Pre-clinical efficacy is tested after
    - local injection
    - nasal administration
    - systemic administration

The neurodegenerative diseases carry a high burden for patients since they are without exception progressive. But they also carry a substantial socio-economic burden with estimated costs of 130 billion Euros per year (2008).

- IV. The technical work in B-SMART will be supported by project management. It ensures that the project is coordinated in a clear, unambiguous and mutually acceptable manner and that the project achieves its objectives, within the given financial and time constraints.

In B-SMART, we expect to arrive at a scale-able nanoparticle formulation with uniform characteristics that shows strong preclinical evidence of therapeutic efficacy and is ready for clinical translation.

**Duration**

60 months (01/01/2017 – 31/12/2021)

**Project Funding**

5,998,303.75 €

**Coordinator**

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- Istituto Biochimico Italiano Giovanni Lorenzini SpA, Italy
- Malvern Instruments Ltd, United Kingdom
- European Research and Project Office GmbH, Germany
- SINTEF AS, Norway

**Project Website**

[www.b-smart-project.eu](http://www.b-smart-project.eu)