Composition for use in the prevention and/or treatment of intellectual disability and neurodegenerative diseases in a subject with Down's Syndrome

KEYWORDS

- ❑ DOWN SYNDROME
- ❑ SITAGLIPTIN
- ❑ NEURODEGENE–RATION
- ❑ AGING
- ❑ ALZHEIMER DISEASE

AREA

- ❑ PHARMACEUTICAL

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Patent Type
Patent for invention.

Ownership
Sapienza 100%.

Inventors
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Industrial & Commercial Reference
Pharmaceutical and Biomedical.

Time to Market
Technology validated in relevant environment (TRL_5).

Availability
Licensing.

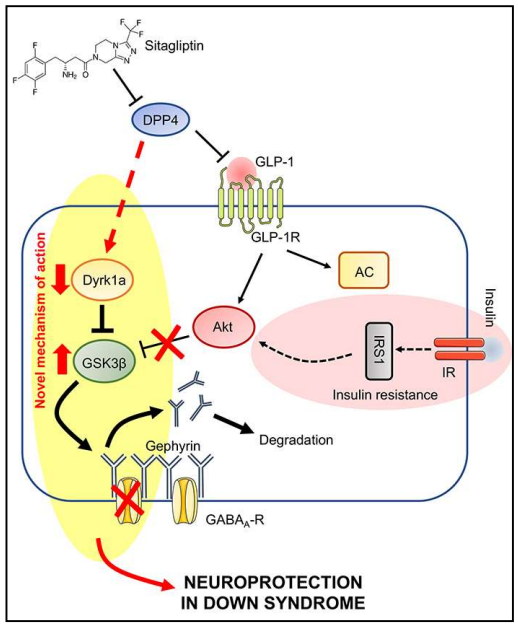

Fig. 1 Novel mechanism of action proposed for sitagliptin in Down syndrome.

Abstract
Down syndrome (DS) is a genetic disorders characterized by a complex phenotype of which cognitive deficit is a relevant aspect. Increased life expectancy in DS also highlighted other co-morbidities, including a high risk to develop Alzheimer-like dementia after the age of fourthy.
Currently, there are no drugs approved for the treatment of intellectual disability or AD-like neurodegeneration in DS. Our invention relies with the repurposing of a known drug, widely used in clinic, and proven to be effective in improving both intellectual disability and the development of AD-like dementia in DS.

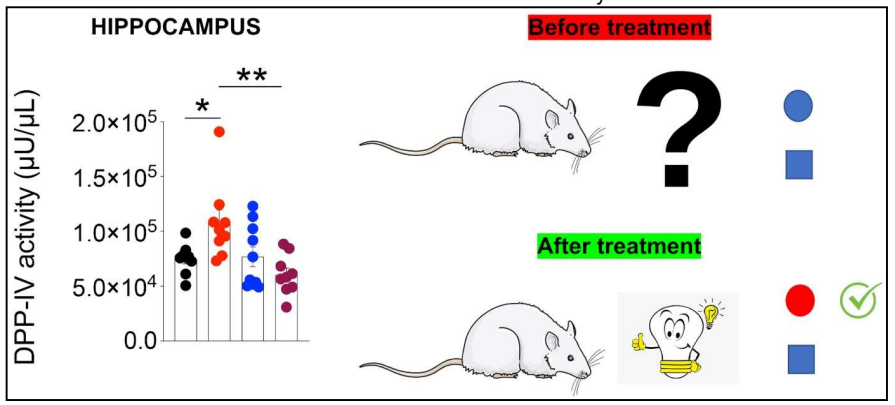

Fig. 2 Intranasal sitagliptin administration reduces DPP4 activity and improve cognitive functions in a mouse model for Down syndrome.

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Technical Description

Data related to the efficacy of the drug have not yet been published and are not protected by patents.

In general, it is a substance that has been shown to have a dual effect when administered in a mouse model of DS:

- correction of the known imbalance between the excitatory / inhibitory circuits in the brain, which is then associated with a significant improvement of cognitive and learning deficits;
- marked reduction and therefore accumulation of neuropathological hallmarks of AD in the brain.

Technologies & Advantages

Currently, there are no drugs available for the treatment of intellectual disability and the development of AD-like neurodegeneration in people with DS.

- repurposing of a drug widely used in the clinic and considered very safe;
- better effects than other molecules tested to act on the same intracellular path but then resulted unsafe when tested in animal models;
- specific and circumscribed effects at the cerebral level;
- restoration of the balance between the activity of the excitatory and inhibitory circuits in the brain;
- slowing of the progression of the neurodegeneration process;
- AD prevention in DS;
- improvement of cognitive and learning deficits.

Applications

The drug can be used to treat intellectual disability and the development of neurodegeneration in individuals with DS. The activity is expressed through a completely new molecular mechanism that modulates an intracellular signaling pathway known to regulate synaptic transmission and found to be altered in DS because the triplication of chromosome 21. The alteration of the pathway has been observed only at cerebral level and consequently the delivery of the drug at the level of the central nervous system allows a site-specific activity, without promoting side effects at the peripheral level.

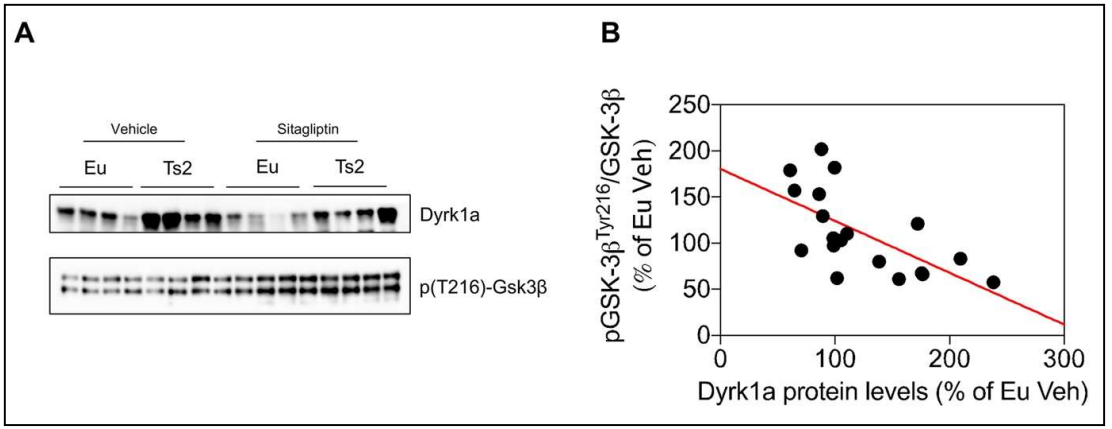

Fig. 3 Intranasal sitagliptin administration reduces Dyrk1a protein levels and promotes GSK3β activation in the hippocampus of a mouse model for Down syndrome.

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