

Use of boron and its derivatives for the treatment of muscular dystrophies

Market sector: Pharmaceutical, musculoskeletal, rare diseases

Type of opportunity: licensing and/ or co-development

Scope of the problem

Boron is an essential metalloid, which plays a key role in plants and animal metabolisms. It has been reported that boron is involved in bone mineralization, has some uses in synthetic chemistry and its potential has been only recently exploited in medicinal chemistry. Little is known about boron homeostasis and function in animal cells. It has been reported that boron is involved in mouse myogenic differentiation. Boron and its derivatives can be used as therapeutic systems for muscular dystrophies for which currently no successful treatment has been obtained, for example, Duchenne muscular dystrophy (DMD) and myotonic dystrophy (DM).

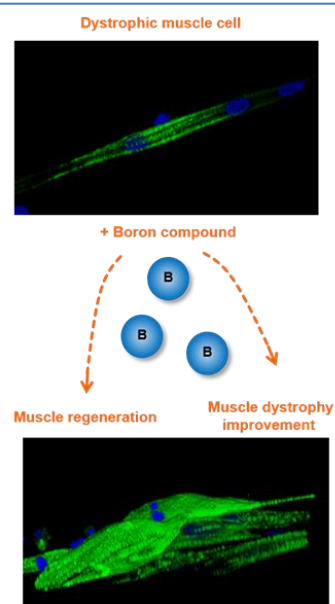
DMD is a progressive and lethal disease, caused by X-linked mutations of the dystrophin encoding gene. The lack of dystrophin leads to weakness, degeneration, and consequent fibrosis in skeletal and cardiac muscles. Nowadays, there is no cure for DMD patients.

DM is a dominantly inherited neuromuscular disorder (rare disease) for which currently there is no cure or effective treatment. Several therapeutic approaches have been tested, although with no clear success.

Patient need addressed: muscular dystrophies

Our innovation:

- New treatment for muscular dystrophies based on the administration of boron or a compound of boron.
- The active compound can be also used in combination with another active principle or with another current therapy (“Combination therapy”).
- The active compound may be administered with an excipient, carrier, and vehicle, commonly used in the pharmacology field, including biodegradable polymeric materials.
- The pharmaceutical composition may be administered orally, parenterally, by inhalation spray, or rectally.
- *In vitro* cellular studies presented similar positive results after addition of boron compound in two different muscular dystrophies, with extremely diverse aetiological origin (DMD and DM1).
- *In vivo* studies showed the repair of muscle atrophy to physiologically healthy levels and also an extension of the life expectancy.



Competitive advantages: The active compound occurs naturally as a mineral or can be prepared easily. The therapeutic effects of boron are expandable and embracing several muscular dystrophies. Boron treatment cannot only allow muscle regeneration at the anatomical level but also at a functional level.

Market size/ opportunity: Muscle-wasting conditions are very rare, affecting just over one in every 1,000 people (Muscular Dystrophy UK, 2018). DMD is the most common form of muscular dystrophy affecting 16 to 20 infants per 100,000 live births, and its market size is expected to reach USD 4.11 billion by 2023, expanding at a CAGR of 41.3% during the period 2018-2023 (Grand View Research, Inc., August 2018).

Intellectual property

International patent application, PCT (September 20, 2019)

Priority date: September 20, 2018