SKIP-NMD

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Research Areas

Product Development

Drugs

At a Glance

- Status: **Active Consortium**
- Year Launched: **2012**
- Initiating Organization: **SKIP-NMD**
- Initiator Type: **Government**
- **Rare** disease
- Location: **International**

Abstract

Duchenne muscular dystrophy (DMD) is an incurable progressive muscle-wasting disorder presenting within the first few years of life. It arises from the absence of the protein dystrophin from muscle. Without medical intervention, affected children become unable to walk beyond about 12 years of age. SKIP-NMD is an EU FP7-funded collaborative grant involving 10 partners from Europe and the United States, whose aim is to restore dystrophin production in a subset of DMD boys. This will be achieved by developing a drug that “skips” the mutations causing DMD, so as to restore dystrophin protein expression.

Mission

The SKIP-NMD project is designing a drug called an antisense oligonucleotide (AON) to “skip” exon 53. Skipping exon 53 will restore dystrophin production in DMD boys with deletions spanning exons 52, 45-52, 47-52, 48-52, 49-52, and 50-52. Therefore, only a subset of DMD boys will be eligible for this treatment. The drug will first undergo pre-clinical tests, followed by a phase I/IIa clinical trial. The project will also develop new outcome measures and biomarkers to ascertain the drug’s effectiveness.

The project follows from Prof. Muntoni's previous work in the MDEX consortium with Sarepta Therapeutics involving another AON previously shown to safely restore dystrophin production in another subset of DMD boys, by "skipping" exon 51 (Kinali et al, 2009; Cirak et al, 2011). However, different AON are required to skip different exons, so ultimately a panel of such AON drugs is required to ensure as many DMD boys as possible can be treated.

The objectives of the SKIP-NMD grant are to:

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Homepage http://www.skip-nmd.eu/

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