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NETWORKING TOWARDS CLINICAL APPLICATION OF ANTISENSE-MEDIATED EXON SKIPPING

This COST Action aims to advance the development of antisense-mediated exon skipping for rare

diseases, focusing on Duchenne muscular dystrophy for which this approach is currently assessed in phase 3 clinical trials.

The Action involved all key stake holders (scientists, clinicians, regulators, industry and patients) this COST Action aims to overcome challenges through networking to allow clinical implementation of antisense-mediated exon skipping for as many rare disease patients as possible.

Several challenges hamper its development to wide clinical application:

- There is no standardized protocol for important biological outcome measures, such as dystrophin restoration
- The approach is mutation specific; development for

This COST Action will address the described issues through:

- Meetings and training to standardize outcome measures
- Meetings with regulatory authorities to discuss alternatives to develop this approach for small

patient subgroups is challenging as most mutations are rare

- Fragmentation: several European groups work on preclinical optimization
- There is therapeutic misconception amongst patients and unrealistic expectations
- patients groups
- Networking workshops where unpublished data are shared confidentially between Parties to foster synergistic work and avoid duplication
- Training of young scientists in unbiased and clear communication to patients

For more details, contact any of our current members or visit the website www.exonskipping.eu

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