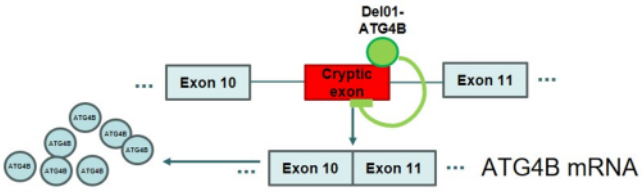
PROJECT NUMBER

18450

Targeted RNA therapy to restore ATG4B function in ALS

Del01-ATG4B is an exon-skipping oligonucleotide that prevents cryptic exon inclusion in ATG4B mRNA – a disease mechanism identified in ALS patients.

Applications: RNA therapy, Oligonucleotide therapy, ALS, neurodegeneration



Features	Benefits
Broad applicability across sporadic and familial ALS	Potential to address a substantially larger patient population than mutation-specific therapies.
Novel RNA-splicing correction mechanism	Targets a fundamental ALS disease mechanism, potentially slowing neurodegeneration.
Selective restoration of ATG4B expression	Restores normal cellular function while minimising effects on healthy cells
Enhanced CNS delivery using peptide-conjugated ASOs	Enables motor neuron targeting, sustained CNS exposure, and potentially less invasive administration
Mechanistically validated target with promising preclinical results	Provides clear rationale and route for therapeutic development

Patented and Available for

- Co-development

- Consulting
- Licensing

To find out more, contact enquiries@innovation.ox.ac.uk and quote the project number.

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