TECHNOLOGY OFFERING

CYCLOVECTOR – RNA DRUG DELIVERY BY CYCLODEXTRIN VECTORS

Modified Cyclodextrins (CDs) represent a major advance over current non-viral delivery vectors for siRNA. The Cyclodextrin vectors developed at UCC/UCD have a favourable toxicity profile and do not evoke an immune response *in vivo*. Furthermore, proof-of-concept of the efficacy and specificity of siRNA delivery by modified Cyclodextrins has been demonstrated *in vivo* for several disease indications such as prostate cancer, IBD and Huntingdon's Disease.

VALUE PROPOSITION

Renewed investor interest in RNAi therapies has been sparked by recent positive clinical trial data which has demonstrated the safety and efficacy of gene-silencing in modifying disease outcomes. The CycloVector technology developed at UCC/UCD offers a unique opportunity for drug discovery companies wishing to gain entry into this therapeutic area or for established siRNA companies seeking to de-risk their pipelines by investing in novel siRNA vectors with proven safety and efficacy *in vivo.*

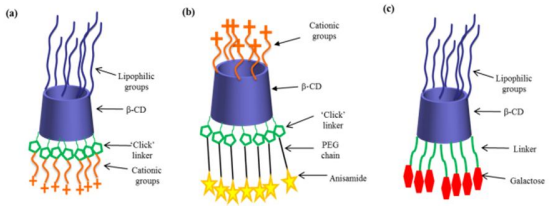

Figure 1. Schematic representation of selected CD vectors. (a) Amphiphilic and cationic modified CD, (b) Anisamide targeted PEGylated CD and (c) Galactose targeted amphiphilic CD (O'Mahony *et al*., Pharm. Nanotechnology, 2013).

THE TECHNOLOGY

A library of vectors based on modified cyclodextrins has been designed and synthesised, with lipophilic, charged or neutral PEG groups, as well as cell-targeting or biolabile groups and peptide- and glyco-conjugates. *In vivo* proof-of-concept data for gene knockdown by CD-complexed siRNA has been obtained in several small animal disease models including Huntingdon's, IBD and prostate cancer. The CD-complexed siRNA is resistant to degradation by serum nucleases and remain stable when stored for up to six months. The modified CDs have a favourable toxicity profile *in vitro* and *in vivo*. In a mouse toxicology study, systemic administration of high doses of targeted CD-complexed siRNA nanoparticles was not associated with any adverse effects on liver enzymes and body weight.

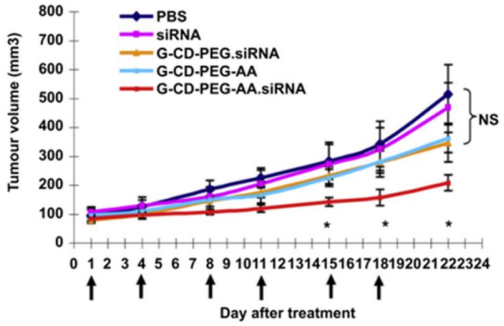

Figure 2. Targeted CD.siRNA complexes successfully inhibit tumour growth *in vivo* in the mouse TRAMPC1 tumour model (O'Driscoll *et al*., Biomaterials 2012).

DEVELOPMENT STATUS

- Lead optimization

FIELD OF APPLICATION

- Delivery of siRNA therapeutics
- Validated in Oncology/CNS/IBD

CONTACT

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