Project Sweet Spot

- Therapeutic pseudo-exon activation

Value proposition and Field of application

A platform to identify targets and design antisense oligonucleotides for the treatment of a broad range of diseases caused by, for example, gain-of-function mutations. There are several advantages over existing RNA technologies.

Technological description

This platform technology allows development of splice switching antisense oligonucleotides (ASOs) that can alter or downregulate gene expression, for example in diseases where a mutation leads to an over-active protein.

Our technology has several advantages over current RNA technologies: the risk of off-target effects is extremely low, as the mechanism of action is independent of nucleases and we target unconserved intronic sequences; we insert new sequences as building blocks in the mRNA and allele specific targeting is possible for some targets.

The application potential is extremely broad. We can identify targets with a hidden ”sweet spot” that we accurately target. Binding to this sweet spot results in incorporation in mRNA of a pseudo-exon sequence that would otherwise not be incorporated. The resulting protein will therefore either acquire new characteristics or be unfunctional/quickly degrade.

Many of the targets that we have already identified are in the CNS, eye or liver, which are ideal for ASO drugs based on current delivery technologies. But the potential is much broader than that.

We have proof-of-concept in Parkinson’s Disease where the target LRRK2 currently gets a lot of attention, including a phase 3 study of a small molecule. Our technology has several advantages over the other LRRK2 solutions in development.

We also have proof-of-concept for LRP6, which is relevant as a target for treatment of age-related macular degeneration.

We plan to spin out a company to a) develop own drugs (to a certain point) and b) develop drugs for pharma partners.

Current state of development

We have proof-of-concept in Parkinson's Disease (patient cells) and promising in vitro data in macular degeneration.
The next stage will be in vivo proof-of-concept for the above targets/diseases, optimisation of our LRRK2 lead and development of a high-throughput screening method.

Team

Professor Brage Storstein Andresen, founder-to-be

- 30 years' experience in human genetics, RNA splicing and antisense technology
- >140 scientific papers
- 4 patent applications on SSO-mediated splicing therapy

Future CEO (confidential), founder-to-be

- PhD within a relevant area
- Leadership experience (biotech and pharma)
- Expertise within business development, drug discovery and early development of
- RNA therapeutics
- First-hand experience with relevant pharma deals

SDU business developer: Liv Thomsen

Intellectual property rights

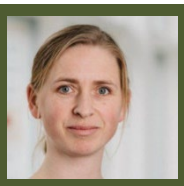
- PCT/EP2022/051790: Splice-switching oligonucleotides targeting pseudo-exons (priority: 26 Jan 2021). Positive preliminary report from EPO.
- PA 2022 70346 : Allele-specific splice switching oligonucleotides targeting pseudo-exons (priority: 29 Jun 2022)
- Proprietary knowledge/insight: PseuFi software and human pseudo-exon database

Business opportunity and Call to action

We are looking for:

- investors that may be interested in supporting the coming spinout company
- pharma companies that may be interested in partnering with us on the development of novel antisense oligonucleotide therapeutics

Contact information
Liv Thomsen, business developer
litho@sdu.dk



SDU