Refinement of antisense oligonucleotide mediated exon skipping as therapy for Duchenne muscular dystrophy

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Abbreviations

2OMePS	2'O-Methyl Phosphorothioate
AON	Antisense oligonucleotide
BMD	Becker muscular dystrophy
BMP4	Bone morphogenetic protein 4
CPP	Cell penetrating peptide
DGC	Dystrophin-glycoprotein complex
DMD	Duchenne muscular dystrophy
ECM	Extracellular matrix
eNOS	Endothelial nitric oxide synthase
GRMD	Golden retriever muscular dystrophy
HDAC-3	Histone deacetylase 2
HGF	Hepatocyte growth factor
IGF-1	Insulin-like growth factor 1
IL-4	Interleukin-4
iNOS	Inducible nitric oxide synthase
IP	Intraperitoneal
IV	Intravenous
MGF	Mechano growth factor
LNA	Locked nucleic acid
MMP	Matrix metalloproteinase
NF-κB	Nuclear factor kappa light chain enhancer of activated B cells
NMJ	Neuromuscular junction
nNOS	Neuronal nitric oxide synthase
NO	Nitric oxide
PDGF	Platelet derived growth factor
PEG	Polyethylene glycol
PMO	Phosphorodiamidate morpholino
PPMO	Peptide phosphorodiamidate morpholino
PNA	Peptide nucleic acid
SC	Satellite cell
TGF-β	Transforming growth factor beta
TRPC	Transient receptor potential cation channels

Curriculum vitae

Hans Heemskerk was born on 14 December 1979 in Haarlem. He attended Lyceum Sancta Maria in Haarlem and received his VWO diploma in 1998. In the same year he started Biomedical Sciences at Leiden University, for which he received his doctoral degree in 2002. The study included three internships, of which the first was to analyse TNF-alpha inhibition as a treatment for Crohn's disease at the department of Gastroenterology and Hepatology under the supervision of Dr. RA van Hogezand. The second internship, at the department of clinical oncology, concerned apoptin in the treatment of cancer, under the supervision of Dr. EM Verdegaal. The last and main internship was conducted at the department of molecular cell biology under supervision of Dr. C Jost and was aimed at the development of a method to track mRNA transport.
After travelling and working as a technician, he worked as a PhD student from 2005 to 2010 at the department of Human Genetics of the LUMC, under the supervision of Prof. Dr. GJB van Ommen, Dr. A Aartsma-Rus and Dr. JCT van Deutekom. The results of this research are presented in this thesis. In 2010 he started a post-doc position at the Institute of Child Health of the University College London. At his new job he continues to develop and improve potential therapies for Duchenne Muscular Dystrophy.

Publication list

Heemskerk H, Morgan J, Aartsma-Rus A. Muscle damage and repair in healthy subjects and Duchenne muscular dystrophy. *Manuscript submitted*

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