



Program NVGCT Annual symposium March 11-13, 2020

Wednesday March 11th 2020

Afternoon will be In DUTCH

- 13.00-17.10 **Middag over Genterapie voor patiënten en patiënten organisaties**
[Voorzitter: Prof Dr. G Wagemaker](#)
- 13.00-13.25 **Registratie en Welkom**
- 13.25-13.30 **Opening door Prof. dr. G. Wagemaker**
- 13.30-14.15 **Dr. C.A. Herberts, College ter Beoordeling Geneesmiddelen, lid CAT/EMA**
Genterapie, hoe is dat geregeld?
- 14.15-15.00 **Dr. M. Jak, UMCU**
Hit the target; 'Super' cellen van de patiënt maken genezing van kanker mogelijk
- 15.00-15.30 **koffie-thee pauze**
- 15.30-16.15 **Prof. dr. F.W.G. Leebeek, ErasmusMC**
Genterapie voor hemofilie: Genezing nabij!
- 16.15-17.00 **Paneldiscussie o.l.v. Dr. M. Driessens, (VSOP) m.m.v. mw. B. Verhoeven (Hematon), Dr. L.Timmers (Zorginstituut), Drs. M. van den Bosch (VWS) en Drs. F. Logman (Menzis)**
Wat is er nodig om genterapie bij de patiënt te krijgen?
- 17:00-17.10 **Presentatie publieksinformatie Genterapie & Patiënten folders**
- 17:10-17.15 **Sluiting door Prof. Dr. G. Wagemaker**

Thursday March 12th 2020

9.00 - 10.00 **Registration and Coffee**

10.00-10.15 **Welcome and Opening Announcements**
[Victor van Beusechem](#), President of the NVGCT

10.15 - 11.00 **SESSION I GENOME EDITING**

[Chair: Piter Bosma](#)

10.15 **Danny Wilbie, Utrecht University**

Influence of the cell cycle phase on Cas9-mediated gene-knockout efficiency in HEK293T cells

10.30 **Siwei Chen, University of Groningen**

A high-throughput screening platform for identifying potential small molecular inhibitors of CRISPR/Cas9-mediated gene editing

10.45 **Johanna Walther, Utrecht University**

Direct in vivo delivery of CRISPR/Cas9 via biomimetic lipid nanoparticles for gene therapy

11.00 - 11.30 **SESSION II EXHIBITOR PITCHES**

[Chair: Jeroen de Vrij](#)

The following exhibitors present their company's activities in a pitch:

Lonza, CellGenix, Miltenyi Biotec, tebu-bio and Eurogentec

11.30 - 12.45 **NVGCT-ZonMw Young Investigator Speed Date Challenge (I)**

[Chairs: Ingrid de Visser & Jan Theys](#)



11.30 Introduction / explanation Speed Date Challenge

11.40 2019 winners: Alexandra Mowday and Heleen van der Veen

11.55 Speed-date sessions & start drafting projects

12.45 - 13.45 **LUNCH**

13.45 - 14.45 **SESSION III VECTOROLOGY & DELIVERY**

[Chair: Sabine van der Sanden](#)

13.45 **Iris Dautzenberg, Leiden University Medical Center**

Experimental validation of the reduction of the envelope-pseudotyped lentiviral vectors titers in cell-culture medium

- 14.00 **Johan van der Plas, Centre for Human Drug Research**
- First-in-human, randomized study to assess the safety, tolerability, immunogenicity and shedding of a live-attenuated respiratory syncytial virus vaccine*
- 14.30 **Irene Vázquez Domínguez, Radboud University Medical Center**
- First steps towards a comprehensive comparison of chemically modified AONs within the retina*
- KEYNOTE LECTURE**
- [Chair: Rob Collin](#)
- 14.45 **KLEOPAS KLEOPA**
- The Cyprus Institute of Neurology and Genetics and Cyprus School of Molecular Medicine**
- Cell-targeted gene therapy for demyelinating inherited neuropathies and leukodystrophies*
- 15.30 **COFFEE BREAK**
- 16.00-17.00 **SESSION IV THERAPY DEVELOPMENT AND REGULATIONS**
- [Chair: Hidde Haisma](#)
- 16.00 **Harm Hermsen, ProPharma**
- Why you need a multidisciplinary approach to be successful in cell and gene therapy development?*
- 16.30 **Marco Gielkens, Gene Therapy Office**
- Regulations in a rapidly emerging field*
- 17.00 **NVGCT General Assembly**
- 17.30 **Leisure time / draft projects Speed Date**
- Proposals need to be handed in at 22.30 hrs ultimately
- 19.00 **Dinner**
- 20.30 **Famous NVGCT Pubquiz**
- [Quizmaster Jan Theys](#)

Friday March 13th 2020

8.55 **Announcement top-three Speed Date Projects to be prepared for a pitch!**

9.00 - 10.45 **SESSION V ONCOTHERAPY**

[Chair:](#) Toos Daemen

9.00 **Tereza Brachtlova, Amsterdam UMC**

RNA library screening to identify target genes for more effective oncolytic immunotherapy of melanoma

9.15 **Alexandra Mowday, Maastricht University**

Bugs as drugs to target necrotic tumours

9.30 **Tanja de Gruijl, Amsterdam UMC**

Activation of GSK3-beta in the melanoma microenvironment renders dendritic cells refractory to immune suppression and induces T cell activation and oncolysis

10.00 **Frédérique de Graaf, Erasmuc MC**

Designing a safe and effective oncolytic viral therapy with Newcastle Disease Virus

10.15 **Jing Li, Amsterdam UMC**

Targeting core RNA spliceosome proteins as a novel treatment for non-small cell lung cancer

10.30 **COFFEE BREAK**

11.00 **Florence van Tienen, Maastricht University Medical Centre**

Development of the first in man clinical application of autologous mesoangioblasts as cell therapy medicinal product

11.15 - 12.00 **Greiner Award for Best Thesis 2019**

[Chair:](#) Rob Collin



12.00- 13.00 **LUNCH**



13.00 **NVGCT-ZonMw Young Investigator Speed Date Challenge (II)**

Pitches (5 minutes each) by three selected couples

[Chairs:](#) Jan Theys & Ingrid de Visser

13.20

NVGCT OUTSTANDING ACHIEVEMENT AWARD 2020

[Chair: Victor van Beusechem](#)

DIRK GRIMM

Heidelberg University

The fast and the curious - high-throughput interrogation of AAV vector libraries in vitro and in vivo

14.15 - 15.45

SESSION VI RNA THERAPIES

[Chair: Enrico Mastrobattista](#)

14.15

Jim Swildens, ProQR Therapeutics

Development of antisense oligonucleotides for the treatment of inherited retinal diseases

14.45

Tomasz Tomkiewicz, Radboud University Medical Center

Antisense oligonucleotide-based therapy for a common ABCA4 mutation underlying Stargardt disease

15.00

Nuria Suárez Herrera, Radboud University Medical Center

Antisense oligonucleotides cloned into an adeno-associated viral vector for the rescue of aberrant splicing of ABCA4 in Stargardt disease

15.15

Willeke Mom – van Roon, Leiden University Medical Center

Antisense oligonucleotide therapy development for Spinocerebellar Ataxia type 1 and -3

15.45

Award winning ceremony

EUROPEAN SOCIETY OF
GENE & CELL THERAPY



*Announcement winner ESGCT Best Abstract Award
Announcement winners ZonMW Speed Date
Closing remarks*

16.00

Meeting Closure by Prof. Dr. V van Beusechem, President of the NVGCT



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