



ANNUAL SYMPOSIUM

June 15th and 16th 2022

Congrescentrum De Werelt

Westhofflaan 2, 6741 KH Lunteren



WEDNESDAY JUNE 15th 2022

9.00 Registration and Coffee

9.30 Welcome and Opening Announcements

Victor van Beusechem, President of the NVGCT

9.45 OPENING KEYNOTE LECTURE

KLEOPAS KLEOPA, The Cyprus Institute of Neurology & Genetics, Nicosia

Gene replacement or gene silencing strategies to treat demyelinating CMT neuropathies

10.30 - 12.45 SESSION I Cancer Therapy

Chair: Sabine van der Sanden

10.30 Jürgen Kuball, University Medical Center Utrecht

Translating gdT cells and their receptors to the clinic: 15 years later... go or no-go?

11.00 Coffee break

11.30 Marco Huberts, Erasmus Medical Centre, Rotterdam

Three-dimensional models for pancreatic ductal adenocarcinoma for evaluation of oncolytic virus efficacy

11.45 Tanja de Gruijl, Amsterdam University Medical Center, Amsterdam

Constitutive activation of GSK3beta in the melanoma microenvironment to overcome immune suppression

12.15 Jella van de Laak, Maastricht University

In vitro and *in vivo* validation models for bacteria-mediated cancer therapies

12.30 Inge Jedema, The Netherlands Cancer Institute, Amsterdam

DARE-NL: establishment of a Dutch infrastructure for cancer-specific ATMP
Research of cell and gene therapy

12.45 - 13.15 Greiner Award for Best Thesis 2020 & 2021

Chair: Rob Collin



13.15 - 14.00 LUNCH

14.00 – 15.30 PARALLEL PROGRAM

14.00 - 15.30 SESSION II-A

NVGCT - ZonMW YOUNG INVESTIGATOR SPEED DATE CHALLENGE

Chair: Jan Theys

14.00 Introduction / explanation Speed Date Challenge

14.10 Speed-date sessions & start drafting projects

14.00 - 15.30 SESSION II-B

NVGCT MEETS PATIENT REPRESENTATIVES (in Dutch)

Co-organized by VSOP and Spierziekten Nederland

Chair: Gerard Wagemaker

14:00 Opening

14:05 **Rob Collin, Radboud University Medical Centre, Nijmegen**
The Dutch Center for RNA therapeutics

14.35 **Jan Benedictus, Patientenfederatie Nederland**
Introductie patiënten platform gen- en celtherapie

14:55 **Ria de Haas, Spierziekten Nederland**
De weg naar de eerste gentherapie voor een spierziekte

15.30 *Coffee break*

16.00 JOINT KEYNOTE LECTURE NVGCT, VSOP & SPIERZIEKTEN NEDERLAND

Chair: Enrico Mastrobattista

SABINE FUCHS, University Medical Center Utrecht
PRIME TIME



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**



THURSDAY JUNE 16th 2022

9.00 Announcement finalists Speed Date Challenge to be prepared for a pitch!

9.05 - 9.35 SESSION III IMMUNOTHERAPY

Chair: Max Medina

9.05 **Frédérique de Graaf, Immunetune**
Synthetic linear DNA as a novel platform for the development of personalized neoantigen vaccines

9.20 **Tereza Brachtlova, Amsterdam University Medical Center, Amsterdam**
RNAi library screening to identify target genes for more effective oncolytic immunotherapy of melanoma

9.35 - 10.05 SESSION IV-A GENE DELIVERY

Jerome Cabeau, Thermo Fischer
Designing gene delivery tools to enable cell and gene therapy

10.05 *Coffee break*

10.30 - 11.35 SESSION IV-B

**GENE THERAPY PRODUCT DEVELOPMENT WITHIN INDUSTRY
(sponsored by GENSCRIPT)**

Chair: Rob Collin

10.30 **Jeroen de Vrij, ExoVectory**
ExoVectory (spin-out Erasmus MC): Harnessing the natural power of exosomes for regenerative medicine and delivery of (gene) therapies

10.45 **Di Yu, Elicera**
The journey of Elicera Therapeutics

11.10 **Jeroen van Heijst, Neogene**
Development of the T cell engineering platform for a phase 1 clinical trial of fully-individualized neoantigen-specific TCR therapy to treat advanced solid tumours



11.40 - 12.20 NVGCT - ZonMw Young Investigator Speed Date Challenge

Chair: Jan Theys

- 11.40 **Jan Theys** - Introduction to Speed Date Challenge
- 11.45 **Irene Vázquez Domínguez & Marieke Roefs** (last year's winners)
Project progress and future plans
- 12.00 **Pitches three finalists** (5 minutes pitch, 5 minutes questions)

12.20 - 13.15 Lunch

13.15 - 13.45

- 13.15 **Simona Guidi, ProPharma**
Turning a promising ATMP candidate into a medicinal product

13.45 - 15.30 SESSION V DNA & RNA EDITING

Chair: Jeroen de Vrij

- 13.45 **Irene Vázquez Domínguez, Radboud University Medical Center**
The importance of having a relevant model system to investigate splicing modulation: the unforeseen case of the c.11+5G>A variant in *RPE65*
- 14.00 **Mert Öktem, Utrecht University**
Amphipathic peptide mediated CRISPR-Cas9 ribonucleoprotein delivery for gene knockout and correction in various cell types
- 14.15 **Rick Wansink, Radboud University Medical Center**
Therapeutic approaches against RNA toxicity caused by an expanded microsatellite repeat
- 14.45 **Elena Sendino Garvi, Utrecht University**
GENE SURGERY: a potential CRISPR/Cas9-based treatment option for nephropathic cystinosis *in vitro*
- 15.00 **Dyah Karjosukarso, Radboud University Medical Center**
Towards clinical application of antisense oligonucleotides for the treatment of Stargardt disease
- 15.15 *Coffee break*



15.45 - 16.00 CLOSING CEREMONY

Chair: Victor van Beusechem

15.45 *Announcement winner ESGCT Best Abstract Award*
Announcement winners ZonMW Speed Date

16.00 CLOSING KEYNOTE LECTURE

JOHN VAN DER OOST, Wageningen University, Wageningen
CRISPR-Cas - from bacterial defense to gene & cell therapy

16.45 Meeting Closure
Victor van Beusechem, President of the NVGCT