Kandidaat initiatieven te presenteren op de eerste Healthy Ideas, Healthy Returns bijeenkomst van 25 april 2016 bij het Nederlands Kanker Instituut

AMC: geen
LUMC: ????
VUMC: ????
RadboudMC: ????
UMCG: ????

HUBRECHT LABORATORIUM

NTrans Technologies

Title: iTOP, a backdoor into the cell
Name presenter: Niels Geijsen

potential for spin-off company?	Yes
Stage:	Company founded Nov. 2015
business plan ready	yes
SWOT analysis	yes
patent application filed/in preparation	yes
FTO analysis	yes
prior art analysis	yes
market research ready	yes

Level of development/validation:
NTrans technologies has developed several kits for the genetic modification of specific target cell types including human pluripotent stem cells, dendritic cells and mesenchymal stem cells. These are expected to hit the market before the summer off 2016. Kits for transduction and gene editing of immune cells (T-cells, Hematopoietic stem cells and NK-cells) are underway. In addition to facilitating biomedical research, target identification and drug discovery, our protein-based gene editing technologies provide a unique and safe method for personalized medicine applications in immune- and gene therapies

10 or 30 min presentation*	30 minutes

NKI-AVL

Scenic Biosciences

Title: Cell-seq, the power of DNA sequencing to cell biology
Name presenter: Thijn Brummelkamp

potential for spin-off company?	Yes
Stage:	under development
business plan ready	no
SWOT analysis	yes
patent application filed/in preparation	yes
FTO analysis	no
prior art analysis	no
market research ready	yes

Level of development/validation: Around 40 different screens have been performed, with the screen hits from each screen fully recapitulating the known biology plus identifying novel drug targets.

10 or 30 min presentation*	30 minutes

Qameleon Therapeutics

Title: Qameleon Therapeutics: rescuing abandoned drugs.
Name presenter: Rene Bernards

potential for spin-off company?	yes, already incorporated
Stage: date foundation company,	14-4-2015, EUR 150,000 seed funding EU
business plan ready	yes
SWOT analysis available	yes
patent application(s) filed	yes
FTO analysis performed	yes
prior art analysis performed	yes
market research ready	yes

Level of product development/validation:
First phase 1 clinical study to start in May 2016

10 or 30 min presentation:	30 minutes.

MAASTRICHT UMC

CiMaas

Presenter: Yvo Graus en/of Gerars Bos.

business plan ready	yes
SWOT analysis	yes
patent application filed/in preparation	yes
FTO analysis	yes
prior art analysis	yes/no
market research ready	yes

Level of development/validation: Three products in pipeline. Clinical validation for first product (dendritic cells) starts end 2016 early 2017. Clinical validation second product (Natural Killer cells) is expected to start end 2017. Third product (antibiotics) is at pre-clinical level.

ERASMUS MC

Title:	Targeting senescence against age-related diseases and cancer
Presenter:	Peter L.J. de Keizer
Potential for spin-off company?	Yes
10 or 30 minute presentation?	30 minutes
Stage:	
Business Plan ready?	No
SWOT analysis?	No
Patent application filed/in preparation?	Yes (filed)
FTO analysis?	No
Prior art analysis	Yes
Market research ready?	No

Level of development/validation? So called “senescent cells” in our bodies are a negative influence on aging, a wide range of age-related diseases and cancer development. Removal of these senescent cells was shown to be beneficial to health-span, but to date there are no therapeutic compounds that can do so effectively and safely. We set out to develop such compounds, which generated several hits, out of which we optimized three lead compounds. One of these is especially potent in clearing senescent

cells in human cells. In mice, this anti-senescence compound, TASC1, counteracted age-related decline in organ function, hair and muscle loss and improved overall fitness.

On top of that, TASC1 proved to be very potent in overcoming therapy resistance of several lethal cancer cell types, including metastatic melanoma, breast cancer and Glioblastoma multiforme.

Thus, TASC1 is validated as a potent therapeutic agent for counteracting senescence and the associated decline in healthspan and shows promise against difficult to treat types of cancer.

Title:	Antisense oligonucleotides for splicing correction of lysosomal storage diseases
Presenter:	Pim Pijnappel
Potential for spin-off company?	Yes
10 or 30 minute presentation?	10 minutes
Stage:	
Business Plan ready?	No
SWOT analysis?	No
Patent application filed/in preparation?	Yes (filed)
FTO analysis?	Yes
Prior art analysis	Yes
Martket research ready?	No
Level of development/validation?	Preclinical development using cell-based assays employing primary patient-derived cells

UMC UTRECHT

Synerkine: An innovative treatment of chronic pain and inflammation
Summary: The UMCU has discovered and patented a new approach to treat chronic pain, which differs from all currently used therapies. This novel approach is to normalize pain sensation by administering synerkine, a fusion protein of two anti-inflammatory cytokines. Thus synerkine is based on human protein sequences and therefore is expected to have a unique safety profile. Proof of concept for efficacy has been obtained in multiple models for inflammatory, neuropathic and osteoarthritic pain in mice and dogs. CHO cell lines producing synerkine have been made, the UMCU is currently seeking partners to fund process development for manufacturing of clinical batches for clinical studies. Phase 1 studies are planned within 2 years from start of process development.
Medical need: Chronic pain is the dominant symptom in medical practice nowadays affecting >20% of all people worldwide. Causes of chronic pain are multiple and include diseases such as diabetic neuropathy, osteoarthritis and chronic inflammatory diseases. Current treatments are mainly nonsteroidal anti-inflammatory drugs (NSAIDs), corticosteroids, and opioids, also known as narcotic analgesics. These drugs all have significant safety issues, which is one of the reasons why 50-60% of the people with chronic pain have insufficient pain control.
Rationale: Cytokines are hormone-like proteins that are produced by many cells in the body and that orchestrate inflammatory responses of the body to infection and injury. Cytokines are involved in pain at multiple levels including in the peripheral tissues, the primary sensory nerves and the spinal chord. Anti-inflammatory cytokines have potential as pain killers, but clinical development is hampered by pharmacokinetic limitations and their use as a stand-alone drugs. Synerkine has been developed by UMCU to take away these limitations. Indeed in various animal models synerkine was superior to therapy with stand-alone cytokines, and even to combination therapy with cytokines, highlighting its unique synergistic and pharmacokinetic properties.
Intellectual property: A patent application claiming the sequence of synerkine and its application has been filed.
Clinical development: Clinical development trajectory will be agreed with partner. Currently we foresee a phase 1/2 study on the safety, tolerability and preliminary efficacy of intra-thecal administration of synerkine in patients with chronic post-herpetic neuralgia as a first step.
Status of the project: Research batches of synerkine have been made and characterized in HEK293 cells and in CHO cells (the latter using a platform that allows the cells to be used for clinical manufacturing). Synerkine has been evaluated extensively *in vitro*. Proof of concept for efficacy in

various human *ex vivo* models for inflammatory diseases such as osteoarthritis, rheumatoid arthritis and inflammatory bowel disease has been obtained. Pharmacokinetics of synerkine have been evaluated in rats, and showed improved half-life compared to wild-type cytokines. Proof of concept of efficacy to treat pain has been obtained in multiple mouse models for inflammatory, neuropathic and chemotherapy induced pain, as well as in a dog model for osteoarthritis. Currently mechanistic studies on the effects of synerkine in these models are underway. In none of the animal studies safety issues occurred.

Opportunity: The UMCU intends to spin out a new company dedicated to develop synerkine for clinical use. Capital need of the new company includes costs for process development and production of clinical batches, preclinical toxicity studies, clinical trial design, and management.