

# Why translational medicine fails – and what to do about it

Eureka Institute and  
Science in Transition

Utrecht Summerschool  
Course 2017

July 3<sup>rd</sup> -7<sup>th</sup>  
Utrecht, The Netherlands



UMC Utrecht



**EUREKA  
INSTITUTE**  
for translational medicine

Child health: science for life





Dear participant,

It is with great pleasure that I welcome you to the summerschool course about translational medicine. Translational medicine is at the heart of biomedical research but doesn't always get the appreciation it deserves. UMC Utrecht plays a significant role in fueling the debate about the relative under-appreciation of bench-to-bedside research, which is part of a larger Dutch and international debate about relevance, that is impact and quality, of biomedical research.

With the Science in Transition initiative, rooted in UMC Utrecht and that started in 2013 we pointed to the incentive and reward system of biomedical science that may push researchers into valuing publications in peer-reviewed journals more than efforts to bring results from the laboratory into clinical practice. The challenge of course is to understand why this is and how to change it.

With this summerschool course UMC Utrecht takes its responsibility to aim for the next level in biomedical research and innovation. Like in the clinic we don't stop after making the diagnosis, we are devising therapy and trying treatments as well. As we see it, educating a new generation aspiring translational researchers is part of the solution.

Therefore, with this summerschool you will not only obtain insight in how science really works, but more importantly, you will acquire knowledge on how to be a successful translational researcher yourself. This course is specifically not about learning how to 'play the game', but about understanding that there is a game going on, what the rules of the game are and to be able to transcend it. You will be informed about the latest developments in translational medicine, inspired by rare diseases, and you will develop professional skills that are essential to becoming a translational researcher.

I wish you an inspiring week and I hope the course will be of great importance and guidance for your future as a biomedical professional who wants to impact the lives of patients.

Prof. Frank Miedema  
Dean and vice-chairman of the board, UMC Utrecht  
& one of the founders of Science in Transition





Dear participant,

It is with a mix of pride, great expectations and trepidation that I welcome you to the course “Why translational medicine fails - and what to do about it”, an offering of the Utrecht University as a part of its Summer School and at the same time an initiative which is inspired by and is an integral part of the Eureka Institute for Translational Medicine. Our objective is not to provide you with certainties or burden you with pre-digested knowledge, but rather to inspire you questioning your own notions and hopefully gently shake their foundation.

What we seek is what you seek: to shorten the distance between the unmet need and its solution by inspiring and catalyzing a copernican revolution in Medicine, one which puts patients in the center of the process, one which will truly make a difference in the way we conceive and practice in our respective fields.

The needs are particularly poignant and the opportunities tantalizing in the area of Rare Diseases. Indeed, Rare Diseases are often neglected by Pharma and Science alike, and, too often, also by Society. The tools available are often inadequate, the number of patients insufficient, the critical mass unappealing. Yet, Rare Diseases have contributed to Translational Medicine in a creatively disproportionate fashion. You will have the opportunity to forge a new vision of Translational Medicine, one where study design, clinical and scientific questions, even policies, are molded around the Patient.

We welcome you to the Eureka community. Our best wish is that your enthusiasm, creativity and energy will help propel all of us, together, toward new horizons, in the sole interest of the advancement of Medicine.

Prof. Salvatore Albani, MD, PhD  
President EUREKA Institute  
Director, SingHealth Translational Immunology and  
Inflammation Centre



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## Faculty Roster - July 2017

**Annemieke Aartsma, PhD**

Professor of Translational Genetics,  
Department of Human Genetics, Leiden  
University Medical Center

**Salvatore Albani, MD, PhD,**

Professor, Duke-NUS Medical School  
Singapore, Director, SingHealth  
Translational Immunology and  
Inflammation Centre, UCAN-A Chair

**Petra Baarendse, Ir, PhD**

Senior Expert Improvement & Change  
Management, UMC Utrecht / McKinsey  
& Company program

**Jeffrey Beekman, PhD**

Associate Professor, Dep. Of Paediatric  
Pulmonology/Immunology,  
UMC Utrecht

**Rinze Benedictus, MSc**

Staff advisor UMC Utrecht & Science in  
Transition

**Marloes Bosselaar, MSc**

Project coordinator Research and  
Innovation, Dutch Arthritis Foundation,  
Amsterdam

**Coziana Ciurtin, PhD, FRCP**

Principal Clinical Research Associate/  
Honorary Consultant in Rheumatology  
(Inflammatory Arthritis), University  
College London

**Dirk Elewaut, MD, PhD**

Professor of rheumatology and  
immunology and Chair of the  
Department of Rheumatology at Ghent  
University Hospital, a EULAR and FOCUS  
center of excellence, VIB Inflammation  
Research Center-UGent

**Kors van der Ent, MD, PhD**

Professor in Paediatric Pulmonology and  
director of the Child Health program of  
the UMC Utrecht

**Sabine Fuchs, MD, PhD**

Pediatrician in metabolic diseases at the  
Wilhelmina Children's Hospital /  
UMC Utrecht

**Johan Garssen, PhD**

Professor Immunopharmacology at  
Utrecht University, Head Division of  
Pharmacology at Utrecht  
Institute for Pharmaceutical Sciences,  
Director Immunology Utrecht/  
Singapore, Nutricia Research

**Bert van de Heijning, Ir, PhD**

Principal scientist at Nutricia Research,  
Utrecht

**Rosanne Hertzberger, MSc, PhD**

Writer, free lance microbiologist

**Roos de Jonge, PhD**

Advisor patient participation, Educa-  
tional Centre, UMC Utrecht

**Belinda van 't Land, PhD**

Senior Scientist Immunology at Nutricia  
Research, Utrecht

**Ingrid Lether, MSc**

Manager Research and Innovation,  
Reumafonds

**Jorg van Loosdregt, PhD**

Senior Scientist, Laboratory for  
Translational Immunology,  
UMC Utrecht

**Frank Miedema, PhD**

Dean and Vice Chairman of the Board  
and professor of Immunology,  
UMC Utrecht

**Joris van Montfrans, MD, PhD**

Pediatrician – Immunologist, Division  
Pediatrics, Wilhelmina Children's  
Hospital / UMC Utrecht

**Kiran Nistala, MD, PhD**

Experimental Medicine Physician Glaxo  
Smith Kline & Honorary Consultant in  
Paediatric Rheumatology at Great  
Ormond Street Hospital (GOSH) and  
Scientist based at the University College  
Institute of Child Health (ICH) Centre for  
Rheumatology – London

**Gerard Pasterkamp, MD, PhD**

Professor and head of the Experimental  
Cardiology Laboratory at the  
UMC Utrecht

**Berent Prakken, MD, PhD**

Vice-dean and director of the biomed-  
ical education center at the UMC Utrecht,  
Professor of pediatric immunology at  
the Wilhelmina Children's Hospital

**Norm Rosenblum, MD**

Professor of Pediatrics, Canada Research  
Chair in Developmental Nephrology,  
and Associate Dean, Physician Scientist  
Training, University of Toronto

**Hester den Ruijter, PhD**

Translational scientist at the Laboratory  
of Experimental Cardiology,  
UMC Utrecht

**Casper Schoemaker, PhD**

Senior researcher and project leader at  
the National Institute for Public Health  
and the Environment (RIVM), the  
Netherlands.

**Vicki Seyfert, PhD**

Founder and CEO, My Own Med, Inc.

**Anita Small, MSc, EdD**

Project Manager, Deaf Artists and  
Theatres Toolkit (DATT), Cahoots  
Theatre Company  
Owner/ Founder, small LANGUAGE  
CONNECTIONS

**Rosalind Smyth, CBE, FMedSci**

Director, Institute of Child Health,  
University College London (UCL)

**Ghislaine van Thiel, PhD**

Associate Professor at the Julius Center  
for Health Sciences and Primary Care of  
the UMC Utrecht

**Bas Vastert, MD, PhD**

Pediatric rheumatologist at the  
Wilhelmina Children's Hospital,  
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Senior medical director at Ablynx,  
a Belgian biotech company

**Femke van Wijk, PhD**

Associate Professor, Laboratory of  
Translational Immunology (LTI),  
Wilhelmina Children's Hospital,  
UMC Utrecht

**Nico Wulffraat, MD, PhD**

Professor of Pediatric Immunology,  
UMC Utrecht, Chair Research and  
Education Division Pediatrics, chair  
UCAN CAN-DU

## Organization

EUREKA Institute for Translational Medicine ([www.eurekainstitute.org](http://www.eurekainstitute.org))

Science in Transition ([www.scienceintransition.nl/](http://www.scienceintransition.nl/))

Child Health ([www.umcutrecht.nl/en/Research/Research-programs/Child-Health](http://www.umcutrecht.nl/en/Research/Research-programs/Child-Health))

Apollo Society ([www.apollosociety.eu/](http://www.apollosociety.eu/))

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## Organizing committee

Rinze Benedictus

Sabine Fuchs

Judith de Haan

Gautam Kok

Sabine van der Laan

Berent Prakken

Erica Roks

Norm Rosenblum

Anita Small

Remi Stevelink

Mia Wessels

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## Core Faculty

Rinze Benedictus

Coziana Ciurtin

Sabine Fuchs

Bert van de Heijning

Bea van 't Land

Jorg van Loosdregt

Joris van Montfrans

Kiran Nistala

Gerard Pasterkamp

Berent Prakken

Hester den Ruijter

Vicki Seyfert

Anita Small

Norm Rosenblum

Vicki Seyfert

Bas Vastert

Femke van Wijk

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## Course Coordination

Erica Roks

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## E-learning module

Nienke Verdonk

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## Evaluations

Scott Compton & Jean Xie Huijuan (Duke-NUS Medical School)

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## Utrecht Summer School Central office

Bjorgunn Bretti

Lisanne van Herwaarden

Nadine Nonnekes

Ella Stoffers

Sterre Witte

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**Address UMC Utrecht:** Centre for Education and Training, HB 4.05, Universiteitsweg 98, 3584 CX Utrecht

## Monday July 3<sup>rd</sup>

Location: A.A. Hijmans van den Bergh building  
UMC Utrecht – Class room # 4.33  
Universiteitsweg 98  
3584 CX Utrecht



## Tuesday July 4<sup>th</sup>

Location: Nutricia Research Utrecht  
Utrecht Science Park  
Meeting room Plaza 12  
Uppsalalaan 12  
3584 CT Utrecht



## Wednesday July 5<sup>th</sup>

Location: UMC Utrecht  
meeting room 'Maliebaanzaal' (M 01.1.07)  
Heidelberglaan 100  
3584 CX Utrecht



## Thursday July 6<sup>th</sup>

Location: Landgoed Oostbroek  
meeting room: Janssenzaal  
Bunnikseweg 39  
3732 HV De Bilt



## Friday July 7<sup>th</sup>

Location: UMC Utrecht  
meeting room 'Maliebaanzaal' (M 01.1.07)  
Heidelberglaan 100  
3584 CX Utrecht



## About Translational Medicine

Today, the term “translational medicine” is a buzzword in biomedical sciences with a rapidly increasing number of meetings about it; courses on it; and institutes dedicated to it. At the time of writing, a simple Pubmed search on “translational medicine” generates over 82,000 papers, while a Google search yields nearly 3,000,000 hits. Because of its popularity and its increased use, the meaning of the term translational medicine has become progressively ambiguous and is often used synonymously with clinical testing. In our opinion, translational medicine encompasses the continuum of activities that extend from the concep-

tion of an idea all the way into Phase II/III clinical testing and, ultimately, the development of a tangible product. This itinerary includes multiple and diverse components requiring very different skills and competencies ranging from molecular medicine to pharmacology; from animal testing to clinical trial design; from intellectual property to venture capital.

Translational medicine is therefore the framework needed to ensure the evolution of novel technologies into tangible benefits for patients.

### About the course

**Awareness, inspiration and training** is what this course offers. Key opinion leaders increase your awareness of how science really works and how this shapes translational medicine. International experts provide you with a behind-the-scenes look at some of translational medicine’s success stories. Excellent teachers will train you to become a successful translational researcher yourself, to make a difference in the field, with ample time for personal coaching and mentoring.

## About the research program ‘Child Health’

Child health: science for life



The UMC Utrecht is a center of excellence and has the formal position to deliver specialized care (so-called ‘topreferente zorg’). Continuous improvement of high quality of care can only be achieved by high standards of scientific research and a clear focus on specific disease areas.

All diseases in focus of the Child Health program are characterized by their influence on the individuals’ entire lifespan. These disorders often start at the beginning of life, or even before birth, and can have consequences far into adulthood. Within the Child Health program the ‘Cycle of Life’ approach is strongly intertwined with the so called ‘Cycle of Innovation’. In this ‘Cycle of Innovation’ ambitious interdisciplinary teams of patients, clinicians and investigators – from bench to bedside to society - strive to develop and implement novel approaches for treatment, (early) diagnosis, prognosis and monitoring of children with chronic diseases to fulfill unmet medical and psychosocial needs, to improve the lives of these children and their relatives. Both cycles interact at any moment in our hospital.

The Child Health program links top referent care for pediatric patient groups to interdisciplinary research from fundamental to translational to longitudinal applied medical research. All chronic diseases in focus of the Child Health program share that they start in early beginning of life and can have consequences far into adulthood. These patient-focus areas are: periconceptual, ante- and perinatal damage, congenital and hereditary disorders, severe inflammatory disorders and oncology.



## Apollo Society

Apollo is an international collaborative project between the EUREKA institute and its partner universities with the aim of supporting students to develop the knowledge, skills, and experience that will support a career in the field of translational medicine. Apollo was founded mid-2014 in Utrecht. It was named after the multifaceted Greek god of medicine. Apollo aims to bring together multifaceted medical students and scholars. We want our members to be a group of diverse individuals who stand out by means of their personality, work ethic, research experiences and their aim to be a leader in the field of translational medicine.

[www.apollosociety.eu](http://www.apollosociety.eu)

## Science in Transition



Science is in need of fundamental reform. That is the belief of the initiators of Science in Transition.

Science has become a self-referential system where quality is measured mostly in bibliometric parameters and where societal relevance is undervalued.

The Science in Transition initiators have put forward their ideas in a position paper, organized a series of conferences and participated in many debates. Since 2013 this has added greatly to the debate among researchers and policy makers in The Netherlands and beyond.

Central to the Science in Transition analysis is the realization that we need new rewards and incentives for scientists, and we should involve societal stakeholders in setting the research agenda. Current incentives combined with hypercompetition for limited funds have severe negative effects: many publications of poor quality and limited societal impact; risk aversion and avoiding complex, multidisciplinary problems; systematic under-appreciation of education and other academic duties; very poor career perspectives for young scientists.

[www.scienceintransition.nl/english](http://www.scienceintransition.nl/english)

## Eureka Institute



The Eureka Institute is a non-profit institute founded by an international group of leaders in translational medicine, educators, and institutional administrators. Eureka's mission is to develop a community of translational medicine professionals equipped to catalyze the application of discoveries for the benefit of human health. The institute does this through education and building a community of translational scientists. Among the activities of Eureka are a yearly certificate leaderships course in translational medicine in Siracusa, Italy. The next certificate course will be April 8 – 14, 2018. Eureka partners with major universities worldwide: Duke/NUS, SingHealth; Stanford University, University of Miami, University of Arizona and the UMC Utrecht. Nutricia Research and University College London are supporters of Eureka.

[www.eurekainstitute.org](http://www.eurekainstitute.org)

## Day-to-day program

Eureka's 2<sup>nd</sup> Summer School course "Why translational medicine fails and what to do about it"  
03.07.2017 – 07.07.2017

Course Director: Prof. Dr. Berent Prakken  
Vice-director: Rinze Benedictus  
E-mail: bprakken@umcutrecht.nl

### Monday, July 3<sup>rd</sup>

Location: Hijmans van den Bergh building, UMC Utrecht, class room HB 4.33  
Universiteitsweg 98, 3584 CX Utrecht

On this first day of the course you will get to know each other and start with your group assignment. Also you will be introduced in the sociology of science and discuss the main issues in translational medicine.

Chair of the day: Rinze Benedictus

Time	Title & speakers	Activity
8:30	<b>Coffee and welcome</b>	
9:00	<b>Welcome by the Chair of the Child Health program</b> by <b>Kors van der Ent</b>	Interactive lectures and plenary sessions
9:15-11:00	<b>Introduction and welcome</b> by <b>Norm Rosenblum</b>	Interactive lectures and plenary sessions
11:00-11:15	<b>Coffee break</b>	
11:30-12:15	<b>Eureka and the endangered translational scientist</b> Interactive lecture on the challenges of translational medicine in rare diseases and the role of the translational scientist. Facilitator: <b>Berent Prakken</b>  <b>Learning objectives:</b> <ul style="list-style-type: none"> <li>• Define the components of translational medicine</li> <li>• Analyze the challenges of translational medicine, especially in rare diseases</li> <li>• Discuss the role of the translational scientist</li> </ul>	Interactive lectures and plenary sessions
12:15-13:00	<b>The challenges of Translational Medicine in Orphan diseases</b> Facilitators: <b>Sabine Fuchs</b>  <b>Learning objectives:</b> <ul style="list-style-type: none"> <li>• Importance of collaboration across disciplines and institutes</li> <li>• Sharing contributions in translational research</li> </ul>	Interactive lectures and plenary sessions
13:00-14:00	<b>Lunch</b>	

Time	Title & speakers	Activity
14:00-15:00	<p><b>What drives you?</b> Create with <b>Anita Small</b> your own personal manifesto “Be committed to your truth” and start your own “Leadership and Innovation Toolkit” within the context of translational medicine.</p>	Professional skills
15:00-15:30	<p><b>Understanding how science really works</b> Lecture by <b>Frank Miedema</b>, UMC Utrecht dean and initiator of Science in Transition. A ‘view from the trenches’ about the scientific method and associated myths.</p> <p><b>Abstract</b> Science in Transition is a Dutch initiative in which four distinguished scientists gave a piercing analysis of the current state of Dutch universities. This received a lot of attention from public, policy makers and scientists. It added greatly to the debate in The Netherlands about scientific quality and was felt beyond the borders. Central to the Science in Transition analysis is the realization that we need new rewards and incentives for scientists, and we should involve societal stakeholders in setting the research agenda. Current incentives combined with hyper competition for limited funds have severe negative effects: many publications of poor quality and limited societal impact; risk aversion and avoiding complex, multidisciplinary problems; systematic under-appreciation of education and other academic duties; very poor career perspectives for young scientists. <a href="http://www.scienceintransition.nl/english">http://www.scienceintransition.nl/english</a></p> <p><b>Learning objectives</b></p> <ul style="list-style-type: none"> <li>• Students will get insight into how modern science works as a social system.</li> <li>• Students will be able to reflect upon their own role, expectations and the potential actions they may want to make to achieve their personal goals as a scientist.</li> </ul>	Interactive lectures and plenary sessions
15:45-16:00	<b>Coffee break</b>	
16:00-16:20	<p><b>Introduction to Project Prometheus: From culturing cells to culturing paediatric health</b> Facilitator: <b>Sabine Fuchs</b></p> <p><b>Learning objectives:</b></p> <ul style="list-style-type: none"> <li>• Get to know the road from bench to bedside guided by a real-life case in a rare disease</li> <li>• Practice collaborating over disciplines and cultures</li> </ul>	Group assignment
16:30-18:00	<p><b>Project Prometheus part 1: Start a translational medicine research proposal</b> Facilitators: <b>Sabine Fuchs, Rinze Benedictus</b></p>	Group assignment

## Day-to-day program

### Tuesday, July 4<sup>th</sup>

Location: Nutricia Research, Utrecht Science Park,  
Uppsalalaan 12, 3584 CT Utrecht

Today you will learn to appreciate the challenges of translational medicine, reflect on your personal leadership style and contemplate the opportunities of public private partnerships.

**Chair of the day: Bea van 't Land & Bert van de Heijning**

Time	Title & speakers	Activity
8:30	<b>Get together at meeting point at the entrance Hall of the Nutricia Research facility</b>	
8:45	<b>Introduction to the Research Facility</b> by <b>Bea van 't Land</b>	Interactive lectures and plenary sessions
9:00-9:30	<b>Feedback and reflection</b> Personal and interactive session, led by <b>Anita Small</b> and <b>Joris van Montfrans</b>	Professional skills
9:30-9:35	<b>Welcome</b> by professor Johan Garssen	Interactive lectures and plenary sessions
9:45-10:30	<b>Protect what's yours</b> Why protecting intellectual property is crucial to successful innovation and translational medicine. By <b>Gerard Pasterkamp</b> , professor of experimental cardiology  <b>Learning objectives:</b> <ul style="list-style-type: none"> <li>• Protected knowledge may be of interest for investors or companies since it will allow an exclusive right to market the product.</li> <li>• Understand the statement of an investor: "what you publish too fast may be too bad for the patient"</li> <li>• Understand what you report where and how</li> <li>• Be able to delineate what is the definition of "public domain"</li> </ul>	Interactive lectures and plenary sessions
10.45-11:00	<b>Coffee</b>	
11:15-12:15	<b>Discover your style</b> Discover your personal communication style and reflect on working in teams. Begin your "leadership and innovation toolkit". By <b>Anita Small</b>	Professional skills
12:15-13:30	Lunch and tour of the facility – <b>Bea van 't Land and Bert van de Heijning</b>	
13:30-14:15	<b>Improving the health of children with a food supplement? Not so fast.</b> Interactive debate with <b>Bert van de Heijning, Hester den Ruijter, Bea van't Land, Rosanne Hertzberger</b> moderated by <b>Rinze Benedictus</b>  <b>Learning objectives:</b> <ul style="list-style-type: none"> <li>• Learn to appreciate and align different interests of different stakeholders</li> <li>• Realize how media may shape 'scientific' debate</li> </ul>	Interactive lectures and plenary sessions

Time	Title & speakers	Activity
14:30-15:00	<b>Personal experiences in navigating innovation between academia and industry</b> By Johan Garsen – Nutricia Research	Interactive lectures and plenary sessions
15:00-15:15	<b>Coffee / Tea</b>	
15:15-16:30	<b>Project Prometheus part 2</b> Set the next step on the translational road and come up with a good design for a clinical trial	Group assignment
16:30-18:00	<b>Personal mentoring</b> Take the time to share and discuss personal dilemmas with trusted and experienced senior researchers	Professional skills



The 2016 participants and core faculty at Nutricia Research

## Day-to-day program

### Wednesday, July 5<sup>th</sup>

Location: UMC Utrecht – room 'Maliebaanzaal (M.01.1.07)' – Heidelberglaan 100 - 3584 CX Utrecht

This day you will be stimulated to critically contemplate the merits of animal research and you will be invited for dialogue with upcoming and distinguished researchers about academic leadership. You will also be introduced to the powers of collaboration.

**Chair of the day: Coziana Ciurtin**

Time	Interactive lectures and plenary sessions	Activity
8:30-9:00	<b>Coffee</b>	
9:00-9:30	<b>Feedback and reflection</b>	Professional skills
9.30-10.15	<b>Pro's and cons of animal models for Orphan Diseases</b> A guided quest by <b>Norm Rosenblum</b>  <b>Learning objectives:</b> <ul style="list-style-type: none"> <li>• To develop an awareness of the challenges in applying animal models to human translational research, especially in Orphan Diseases.</li> <li>• To understand factors that determine 'validity' of results arising from animal studies and the applicability of these results to humans.</li> <li>• To identify ways to improve the utility of animal models in human translational research.</li> </ul>	Interactive lectures and plenary sessions
10.30-11.00	<b>Coffee Break</b>	
11:00-12:00	Science is Team work. By <b>Anita Small</b> and <b>Berent Prakken</b>	Professional skills
12:15-13:15	<b>Lunch</b>	
13:15-14:15	<b>Developing leadership in translational medicine</b> Interactive lecture by <b>Rosalind Smyth</b> , professor of Child Health and director of the Institute of Child Health, University College London  <b>Learning objectives:</b> <ul style="list-style-type: none"> <li>• To learn the principles of leadership development, and how essential it is to successful academic careers.</li> <li>• To understand the principles of how to develop a strategy.</li> </ul>	Interactive lectures and plenary sessions
14:15-14:45	<b>Coffee Break</b>	
15:00-16:30	<b>Inspirational speed counseling</b> with a panel of established and upcoming clinical scientists	Professional skills
16:30-17:30	<b>Beer and Bites &amp; Keynote meeting with Vicki Seyfert on The future of Health care</b>	
19:00	Speakers dinner	

## Thursday, July 6<sup>th</sup>

Location: 'Landgoed Oostbroek', meeting room: Janssenzaal,  
Bunnikseweg 39, 3732 HV De Bilt

Learn how introducing the patient's perspective can steer the direction of a complete research program and learn the importance of patient participation in crafting a socially robust and ethically sound research proposal.

### Chair of the day: Hester den Ruijter

Time	Title & speakers	Activity
8:30-9:00	<b>Coffee</b>	
9:00-9:30	<b>Feedback and reflection</b> Personal and interactive session led by <b>Anita Small</b> and <b>Joris van Montfrans</b>	Professional skills
9.30-10.15	<b>From cure to lifelong care</b> <b>Nico Wulffraat</b> (pediatrician) with an introduction by <b>Kors van der Ent</b> , chairman of the Child Health research program  <b>Learning objectives:</b> <ul style="list-style-type: none"><li>• Which health related topics are relevant for children and parents?</li><li>• What are the challenges for a children's hospital to address these topics in a meaningful way?</li><li>• Which tools are available for the dialogue between children/parents and health professionals?</li></ul>	Interactive lectures and plenary sessions
10:15-10:30	<b>Coffee Break</b>	
10:45-12:00	<b>Moderated panel debate "Giving a voice to patients"</b> <b>Facilitator: Petra Baarendse</b> A patient's perspective on translational medicine. Are you ready to involve patients in your own research projects? Really? Will you ask them to help draw conclusions in your next paper? Why not? <b>Panelists:</b> <b>Ingrid Lether</b> , Manager Research and Innovation, Dutch Arthritis Foundation <b>Marloes Bosselaar</b> , Project Assistant patient participation, Dutch Arthritis Foundation <b>Roos de Jonge</b> , Advisor patient participation, Educational Centre, UMC Utrecht <b>Casper Schoemaker</b> , Senior researcher / patient advocate, UMC Utrecht  <b>Learning objectives:</b> <ul style="list-style-type: none"><li>• There is a mismatch between what patients and health professionals want to see researched and the research that is actually done. Can research priorities generated by patients solve this problem?</li><li>• To know and understand the need to involve patients in all stages of doing research and learn practical ways and means to actively involve patients in your research, to improve your clinical and societal impact.</li></ul>	Interactive lectures and plenary sessions
12:00-13:00	<b>Lunch</b>	
13:00-14:30	<b>Project Prometheus part 3: Stand and deliver</b> Present your final research proposal to the other course participants in a moving debate. Incorporate the patient perspective.	Group assignment
14:30-15:15	<b>From care to cure in Muscular Dystrophy.</b> By <b>Annemieke Aartsma</b> , Professor of Translational Genetics, Department of Human Genetics, Leiden University Medical Center <ul style="list-style-type: none"><li>• Understand that patients should be involved every step of the way of therapy development</li><li>• Have ideas on how to involve patients in the different steps of therapy development</li></ul>	Interactive lectures and plenary sessions
15:15-17:15	<b>Personal mentoring</b> Discuss personal issues in a safe environment (Includes coffee break)	Professional skills

## Day-to-day program

### Friday, July 7<sup>th</sup>

Location: UMC Utrecht – room ‘Maliebaanzaal (M.01.1.07)’ – Heidelberglaan 100 - 3584 CX Utrecht

Get a behind-the-scenes story of working at a Big Pharma company.  
And prepare yourself for your future in Translational Medicine.

**Chair of the day: Kiran Nistala**

Time	Title & speakers	Activity
8:30-9:00	<b>Coffee</b>	
9:00-9:30	<b>Feedback and reflection</b> Personal and interactive session, led by <b>Anita Small</b> and <b>Joris van Montfrans</b>	Professional skills
9:30-10:30	<b>Thou shalt not...</b> Interactive lecture on research integrity and ethics by <b>Ghislaine van Thiel</b> (associate professor in medical ethics, UMC Utrecht)  <b>Learning objectives</b> , participants will: <ul style="list-style-type: none"> <li>• Gain basic knowledge of research ethics, specifically on clinical trials involving children</li> <li>• Learn to assess ethical complexities of contemporary pediatric research</li> </ul>	Interactive lectures and plenary sessions
10:30-10:45	<b>Coffee Break</b>	
11.00-11:45	<b>How commercial and scientific operatives can drive drug discovery</b> Interactive lecture by <b>Kiran Nistala</b> , Experimental Medicine Physician at GSK  <b>Learning objectives:</b> <ul style="list-style-type: none"> <li>• Students will understand how Industry makes the transition from molecular target to disease indication by integrating, biology, commercial and clinical operatives</li> <li>• Students will appreciate the specific opportunities and challenges that apply to drug discovery in rare diseases.</li> </ul>	Interactive lectures and plenary sessions
11.45-12:45	<b>A stimulated dialogue about opportunities and pitfalls in public private partnerships</b> With <b>Hilde de Winter</b> (biotech company Ablynx) and <b>Dirk Elewaut</b> (Flemish Institute for Biotechnology, Ghent University)  <b>Learning objectives:</b> <ul style="list-style-type: none"> <li>• Understand how to set up a meaningful relationship between a translational scientist and industry</li> <li>• Discuss potential pitfalls and risks</li> <li>• Explore how to work together in a respectful, unbiased and meaningful way</li> </ul>	Interactive lectures and plenary sessions
12:45-13:45	<b>Lunch</b>	

Time	Title & speakers	Activity
13:45-14:30	<b>What should I do?</b> Dilemma game about research integrity and publication pressure. By <b>Norm Rosenblum &amp; Rinze Benedictus</b>	
14:30-15:30	<b>Mind the gap</b> Interactive dialogue with <b>Vicki Seyfert, Hester den Ruijter</b> and <b>Coziana Ciurtin</b>  <b>Learning objectives:</b> <ul style="list-style-type: none"> <li>• How to bridge the gap between goals and reality in the real-world</li> <li>• Reflect on the role of a translational scientist in relation to society</li> </ul>	Group discussion
15:30-16:00	<b>Coffee Break</b>	
16:00-16:45	<b>A translational medicine success story: How organoids help solve clinical issues in cystic fibrosis</b> <b>Jeffrey Beekman</b> , associate professor at UMC Utrecht  <b>Abstract:</b> Translational medicine encompasses the translation of ideas into research and subsequent applications. Moving from concept to application requires a multidisciplinary approach, and usually takes a long breath. This lecture focusses on recent breakthroughs for the treatment of cystic fibrosis (CF), a monogenetic disease caused by dysfunction of a chloride channel in mucosal epithelia. Treatment of CF is shifting from symptom-targeted therapies to therapies that target the CF-causing mutant protein. Forbes magazine recognized the first CF-protein-targeting drug as 'most important new drug in 2012', and many new drugs are currently in development. Beekman and colleagues established and patented a novel individual test for CFTR modulators using individual adult stem cell cultures termed organoids. A collaborative effort enabled progression in 4 years from initial discovery to reimbursed 'off-label' treatments for the first people with CF. Starting as a principal investigator in 2010, Jeffrey Beekman will share some of the challenges encountered during this 'translational success story', particularly emphasizing on the process rather than the science.  <b>Learning objectives:</b> <ul style="list-style-type: none"> <li>• To develop awareness of various stakeholders in a translational process</li> <li>• To identify personal challenges in a translational process.</li> </ul>	Interactive lectures and plenary sessions
16:45-17:00	<b>Wrap up</b> <b>Berent Prakken</b> and <b>Norm Rosenblum</b>	
17:00-17:30	<b>Evaluations</b> <b>Anita Small</b> and <b>Joris van Montfrans</b>	
19:00	<b>Beer and bites</b> at brewery 'Stadskasteel Oudaen' and subsequent <b>Conference Dinner</b> Students receive their certificates	

## Faculty biographies

### About the speakers and moderators



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## About the speakers and moderators

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**Annemieke Aartsma-Rus** is a professor of translational genetics at the Leiden University Medical Center. She played an important role in the development of the antisense mediated exon skipping therapy for Duchenne muscular dystrophy. Her work currently focuses on developing antisense-mediated exon skipping as a therapy for Duchenne muscular dystrophy. Her research involves work in cell and animal models to improve delivery and efficiency of exon skipping compounds, studies in muscle regeneration and pathology, the identification of biomarkers, studying the basics of pre-mRNA splicing and transcript processing and the generation of mouse models. On a networking level she tries to bring together stakeholders in drug development to discuss challenges. She was involved in organizing multiple seminal workshops and meetings, e.g. a stakeholder meeting on antisense-mediated exon skipping development for DMD and SMA involving academics, industry, regulators and patient

representatives hosted by the European Medicine Agencies (April 2015 and November 2016), a training school to educate young scientists in stakeholder communication (April 2015) and the annual ExPRESS Training school for patient representatives and researchers (together with EURORDIS).

Thus far, she has published over 140 peer-reviewed papers and 9 book chapters, as well as 14 patents and has edited one book. She has given many invited lectures at meetings, symposiums and workshops as well as patient/parent organizations meetings, where she is known for her ability to present science in a clear and understandable way. She has created and maintains multiple websites on therapeutic approaches for aimed at patients and parents. In 2011 she received the Duchenne Award from the Dutch Duchenne Parent Project in recognition of this work and her dedication to the Duchenne field.



**Salvatore Albani (MD, PhD)** is an internationally renowned rheumatologist and immunologist. He is currently Professor of Medicine at Duke-NUS Medical School in Singapore, and the Director of the Translational Immunology Centre at SingHealth/ Duke. Before joining Duke-NUS, Dr. Albani served as director of the Translational Medicine Unit at the Clinical Investigation Institute at the University of California, San Diego, where he also was professor of Medicine and Pediatrics. His fundamental research interest is in understanding human immunity and contributing the knowledge to therapeutic and diagnostic advancements. He has developed several innovative approaches in the area of induction and maintenance of immune tolerance in humans, being responsible for the whole translational process from idea to the conclusion of a Phase II clinical trial in autoimmune inflammatory diseases, which have a large impact on society and individuals. He has been responsible for conception and execution of each of the stages of this complex itinerary, which spans a wide and diverse gradient of technologies and challenges. These include molecular immunology, lead identification and validation, intellectual property, CMC

(Chemistry, Manufacturing and Controls), IND (Investigational New Drug filings), trial design, data management and analysis, interfacing with Venture Capital and Pharma, leading complex groups in a multi-center setting, etc. The technology platform has applications in diseases that could benefit from a restoration of immune tolerance. This translational research itinerary has been the original backbone of my career, as witnessed by a rich publication trail (among others Nature Medicine, Lancet, JCI, PNAS, Nature Rheumatology, A&R, ARD, etc, H factor 34) and by approximately 100 patents. Development of high throughput technology platforms is also part of his scientific career. These platforms aim to provide tools for knowledge-based diagnostic and therapeutic decisions. In his role as an educator, it has been his privilege to mentor many talented individuals, and to provide the right challenges and learning opportunities to help them grow and advance. He seeks to expand this even further by helping to create and nurture the next generation of translational scientists. An important step is cultivating in translational professionals the necessary awareness, knowledge and experience

## About the speakers and moderators



**Petra J.J. Baarendse (1979) (PhD)** guides and advises, together with McKinsey & Company, departments and divisions within the UMC Utrecht with the implementation of a large improvement and transformation program called “Together for the Patient”. She has a background in neuroscience, research policy and management. After finishing her master’s in behavioral biology with an extracurricular specialization in neuroscience (cum laude), she obtained a PhD degree in neuroscience at the Rudolf Magnus Institute of Neuroscience. During her PhD, she focused on the role of prefrontal monoamine neuro-transmission in social-cognitive development in preclinical models. She spent a year at the Department of Functional Genomics of the Center for Neurogenomics and Cognitive Research (CNCR) Amsterdam. At the CNCR, she worked on in vivo phenotyping and characterizing the contribution of dorsohippocampal NMDA receptors in emotional learning. During her PhD program, she was member of the educational committee of the Graduate School of Neuroscience (2010-2012), member of the PhD representative committee of the Rudolf Magnus Institute of

Neuroscience (2008-2012) and representative at the re-accreditation of RMI at the Royal Netherlands Academy of Arts and Sciences (2011). In 2010, she completed a training in entrepreneurship in bio-industry. After completing her PhD, she worked as a postdoctoral fellow at the Department of Developmental Psychology at the VU University Medical Center Amsterdam. Here, she focused on neurocognitive development and identification of biomarkers of neuropsychiatric disorders in childhood. From 2013 till 2016 she was head of the Research & Education Office and program manager of the Child Health Program, an integrated framework for child-centered interdisciplinary research to improve the lives of children during childhood and thereafter. January 2016 she was appointed as program leader of Patient Centered WKZ. With this program the Children’s Hospital aims to develop an integrated strategy and working policy of effective care, research and education defined by the patient and family needs. As of February 2017, she has the role of Senior Expert to continue to work on this ambition in a UMC Utrecht-broader perspective.



**Jeffrey Beekman** leads a translational research group in the UMC Utrecht that focuses on the development of individual readouts for disease progression and therapy response in cystic fibrosis (CF). He has been trained in molecular and cellular biology, and became principle investigator in 2010, focusing on CF. His lab developed an innovative functional assay in intestinal organoids that provides a patient-specific platform for analysis of CFTR residual function and CFTR-targeting drugs. This platform facilitates preclinical drug

efficacy screening in an individual manner, and is highly suited for drug development and patient stratification. His current work focusses on the implementation of this intestinal model to enable precision or personalized care for people with cystic fibrosis. Currently, the first patients have received treatments based on this personalized test, for which he was rewarded CF person-of-the-year 2017 by the European CF society.



**Rinze Benedictus (MSc)** works as a staff advisor at the UMC Utrecht, The Netherlands, and is involved in the Science in Transition initiative. This initiative, that gained significant political traction in The Netherlands, brought the debate about quality, reproducibility and relevance of scientific research in the limelight. Benedictus was trained a biologist, and after working as a science journalist, is now active in

science policy and management. At the UMC Utrecht he is developing and implementing new incentives and rewards for researchers. Benedictus is also doing PhD research about the formative effects of these new incentives and rewards at the Centre for Science and Technology Studies, Leiden University, Leiden, the Netherlands.

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## About the speakers and moderators

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**Marloes Bosselaar (MSc)** is project coordinator Research and Innovation at the Dutch Arthritis Foundation (DAF). After a bachelor Biomedical Sciences at the Leiden University, she obtained a Master's degree in Management, Policy Analysis and Entrepreneurship in Health and Life Sciences at the VU University in 2014. During her studies, she did fundamental research on rheumatoid arthritis at the Department of

Rheumatology at the LUMC. In 2016, she joined the DAF as a project coordinator. In this function she coordinates the programme that aims to increase patient participation in research. The DAF stimulates researchers to involve patients in their projects and patients participate in the Scientific Advisory Council involved in ranking project applications in the grant procedures of the DAF.



**Coziana Ciurtin (PhD, FRCP)** is senior lecturer, consultant rheumatologist and clinical trial lead within the department of rheumatology at University College London (UCL). Since 2015, she is director of medicine studies portfolio at University College London Hospital (UCLH), currently leading on the largest portfolio of clinical trials within the division of medicine. Coziana trained in Bucharest (Romania), and in Leeds and Oxford (UK). She established a young translational research lab that is interested in stratified medicine approaches in autoimmune rheumatic diseases, in particular

inflammatory arthritis, lupus and Sjögren's syndrome. In addition, she developed an inflammatory arthritis service supporting translational medicine initiatives at UCL, enabled by her expertise in musculoskeletal ultrasound and synovial biopsies. She has won several national international awards during her training and more recently with the PhD projects coordinated in her lab, and published papers, book chapters and books in the field of biologic treatment in rheumatology, clinical trial methodology, inflammatory arthritis and Sjögren's syndrome.



**Dirk Elewaut (MD)** is a full professor of rheumatology and immunology and Chair of the Department of Rheumatology at Ghent University Hospital, a EULAR and FOCIS center of excellence. He obtained his MD at Ghent University in 1991 and his PhD in 1997 at the same institution. Following postdoctoral research at the University of California San Diego and the La Jolla Institute for Allergy and Immunology he joined the faculty of the Department of Rheumatology at Ghent University Hospital in 2001. He has published more than 200 scientific publications, and is heading a team of 20

researchers of the Laboratory of Molecular Immunology and Inflammation at the same department. The team has recently joined the Inflammation Research Center (IRC) of the Flanders Research Institute for Biotechnology (VIB) where Dirk Elewaut has been appointed as principal investigator. His research interests are centered around translational aspects of immune regulation to combat inflammatory arthritis and associated joint damage, with special focus on spondyloarthritis.

## About the speakers and moderators



**Kors van der Ent (MD, PhD)** (1962) is paediatrician, Professor in Paediatric Pulmonology and chair of the Child Health program. After medical training in Rotterdam and Utrecht he became a research fellow at the department of Paediatric Respiratory Diseases where he graduated in 1997 on a thesis on clinical and physiological aspects of tidal breathing analysis as a measure for airway obstruction in young infants. At the same year he founded the Utrecht Cystic Fibrosis Center, being the largest in the country, focusing on multidisciplinary and long-term centralised treatment of patients with CF. In 2000

he became head of the department of Paediatric Respiratory Diseases and Allergology and was appointed as professor since 2007. Van der Ent is the leader of the national HIT-CF program which focuses on the development and personalized application of new drugs influencing the basis genetic defect in patients with CF. This program includes both mechanistic studies in collaboration with the Hubrecht lab and the Byvoet Center as well as studies into host pathogen interaction in collaboration with the veterinary faculty of the Utrecht University.



**Sabine Fuchs (MD, PhD)** is a pediatrician in metabolic diseases at the Wilhelmina Children's Hospital / UMC Utrecht. She studied pharmacy (cum laude) and medicine (cum laude) at the University of Utrecht. She combined her pediatric training with a PhD (ZonMW AGIKO) in the department of metabolic diseases in the Wilhelmina Children's Hospital / UMC Utrecht. She now combines clinical work with research in the field of genetic/ metabolic liver diseases. In collaboration with the Hubrecht Institute (prof. Clevers), she uses liver

organoids to improve therapeutic strategies for patients with liver diseases – both as a unique in vitro model to develop and test novel treatment strategies and as a new source for liver stem cell transplantations. She further aims to improve pediatric care as a board member of TULIPS (Training Upcoming Leaders In Pediatric Science), an organization devoted to improving child health by empowering young clinician scientists to achieve high quality research.



**Johan Garssen** is Full Professor of Immunopharmacology at Utrecht University, where he's also Head of the Division Pharmacology at the Utrecht Institute for Pharmaceutical Sciences. At Danone/Nutricia Research, after several functions as from 2002, Johan is currently R&I Director Immune in the Global ELN Centre of Excellence Utrecht. He studied medicine and biology at the Free University, Amsterdam, Netherlands. He specialised in immunology, pharmacology and biochemistry and finished both studies in 1987 cum laude. He finished his PhD thesis at the University of Utrecht in 1991 on the role of T cells in respiratory allergy, an immunopharmacological approach. This PhD programme and a postdoc period was partly performed at Yale University, New Haven, USA. After the postdoc period he became senior scientist at the National Institute of Public Health in the Netherlands. There he coached many research projects,

both preclinical as well as clinical research, in the field of immunomodulation induced by a.o. nutritional ingredients, drugs and environmental agents. Johan Garssen published over 500 peer reviewed papers in the field of "immunomodulation". He is editorial board member for Elsevier "Pharma Nutrition" and co-editor of "Pharma-Nutrition, An Overview" (2014-Volume 12 of Springer Series AAPS Advances in the Pharmaceutical Sciences), examining the effects of natural products and functional/medical foods (nutritional programming) on disease management. He has contributed to various grants, a.o. NWO, EU, NIH, and recently the STW Nutrall Grant, nutrition-based approach to support antigen-specific oral immunotherapy for food allergy and the RAAKPRO DiAgRaMs Grant, validate exercise as challenge model to test health products on intestinal condition and immune system.

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## About the speakers and moderators

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**Bert van de Heijning (MSc, PhD)** (1961) Main fields of interest and expertise are nutrition and metabolism, in particular digestive physiology and gut biology; by training zoologist and neuroendocrinologist. Education and professional tenure: Biology (MSc) at Wageningen University (NL). Graduation subjects were Zoology and Animal Physiology (Neuroendocrinology), and teaching qualification. The MSc program included a 6-mo traineeship in Cambridge (UK). PhD-student at the Rudolf Magnus Institute for Neurosciences; PhD degree at Utrecht University (NL) in 1991. 9-mo PhD-

traineeship at McGill university in Montreal (Canada). 5-yr post-doc in the Gastroenterology dept. of the Utrecht Medical School, followed by a short (2 yr) post-doc in Dairy science in Ede (NL). In 1998 assistant professorship at Wageningen University (NL) in the Human and Animal Physiology group, lecturing Physiology, supervising graduate and PhD-students. In 2006 'switched to industry' as senior scientist, and since 2015 principal scientist within Nutricia Research.



**Rosanne Hertzberger (MSc, PhD)** is a freelance microbiologist and a writer. She studies carbohydrate metabolism of vaginal lactobacilli at the Free University of Amsterdam. She writes weekly columns for Dutch national newspaper NRC Handelsblad and published a book "Ode aan de E-nummers" (In Defense of Processed Foods) in April this year.

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## About the speakers and moderators

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**Roos de Jonge (PhD)** was trained as a Medical Biologist and obtained her PhD in neurogenetics at AMC, Amsterdam, The Netherlands. After a year of postdoctoral fellowship at the Silvius Laboratorium in Leiden, she decided to quit basic science and study philosophy at UvA, the Netherlands. She aimed for a job in science education or journalism but ended up as the scientific coordinator at Prinses Beatrix Foundation, a big charity foundation specialized in muscle and motor

neuron diseases. In 2006 her daughter was born with a severe congenital heart defect and forced her to switch to Duchenne Parent Project which was at walking distance of her house. She started combining the care for her daughter with patient advocacy and got involved in patient participation at Stichting Kind&Ziekenhuis. Nowadays works as Advisor Patient Participation in education at the UMCUtrecht combining her personal experience with training.



**Belinda van't Land (PhD)** is a Sr. Scientist within Nutricia Research and affiliated to the Wilhelmina's Children's Hospital, UMC Utrecht. She currently leads a cross-functional research team (consisting of a Post-doc, 3 PhD students and a Sr. Assistant Scientist) studying the complex interplay between nutrition and immunological development in early life. She obtained her PhD at the Radboud University of Nijmegen which focused on the immunopathology involved in mucosal barrier injury with the impact of nutrition as key

study aspect. With increasing interest in immunological challenges and the passion for translational research she took the role as scientist within Nutricia Research and performed clinical as well as preclinical research leading to a fast amount of publications, patents as well as granted research collaborations. With the passion for early life immune development and believe that nutrition can make the difference she is dedicated to increase the translational capacity of current research collaboration.



**Ingrid Lether (MSc)** Manager Research and Innovation, Reumafonds studied biology at Utrecht University and obtaining her Master's degree in 1987. She has worked for several pharmaceutical companies, being involved in phase I-III clinical research. In 2001 she joined the Dutch Arthritis Foundation as manager Research and Innovation. In this

function she has developed the current grant procedures (on project grants, program grants, fellowships, and on international collaborations and consortia) and research policy of the DAF. The DAF is actively bringing (international) researchers and funders together, with the aim to help bring forward translational research into arthritis.

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## About the speakers and moderators

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Eureka Alumnus **Jorg van Loosdregt** (1981) obtained his PhD at the Molecular Immunology laboratory, UMC Utrecht, The Netherlands. Here, he studied the regulation of the transcription factor FOXP3, which is crucial for both the differentiation and function of Regulatory T cells, and therefore for maintaining immune homeostasis. After completing his PhD program in 2011 (cum laude) Jorg moved to the Department of Translational Research, at the Sanford-Burnham Medical Institute in San Diego, USA, on a fellowship from the Dutch Arthritis Foundation. In San Diego he

investigated the role of autophagy in orchestrating effector T cell responses, with a focus on rheumatoid arthritis. Since December 2013, van Loosdregt is working in the Center for Molecular Medicine (CMM) and laboratory for translational immunology (LTI) where he is supported by a VENI fellowship. His research group uses a translational approach to study the epigenetic and molecular pathways that are deregulated in autoimmune diseases with the aim of identifying novel therapeutic strategies to treat these diseases.



**Frank Miedema** (1954) studied biochemistry at the University of Groningen, specializing in immunology, with a minor in Philosophy of Science. He obtained a PhD from the University of Amsterdam at the Central Laboratory of the Blood Transfusion Service (CLB), now Sanquin. There, from 1983 he was project leader immunovirology of HIV/AIDS as part of the Amsterdam Cohort Studies. In 1996 he was appointed full professor at AMC/University of Amsterdam and in became Director of Sanquin Research in 1998. In 2004 he became head of the Immunology department at the UMC Utrecht. From

2009 he is dean and vice chairman of the Executive Board at the UMC Utrecht. He is one of the initiators of [www.scienceintransition.nl/english](http://www.scienceintransition.nl/english). Science in Transition believes that the scientific incentive and reward system is in need of fundamental reform. Next to Science for Science (articles in journals), the added value for society needs to be more appreciation and societal stakeholders should be involved more integrally in the production of knowledge.



**Joris van Montfrans (MD, PhD)** is a clinician-scientist in the Wilhelmina Childrens Hospital. His clinical work consists of taking care of children with immunodeficiencies and auto immune diseases. His research focusses on children and adults with primary immunodeficiencies and particularly looks into new or improved diagnostic tests, genetics causes of

immunodeficiencies, and improved treatments. Joris is active in the national working party for immunodeficiencies (secretary) and in the ESID registry steering committee. Other activities: he is part time architect specializing in modern houses.

## About the speakers and moderators



**Kiran Nistala** is an experimental medicine physician at GSK specialising in Drug Discovery Project Leadership and Early Phase Clinical Trial Design in the Complement, Chemokine and Cytokine Drug Performance Unit. His additional roles include leadership of the GSK Physician Community Global, membership of the GSK Immunotoxicology panel and Experimental Medicine Initiative steering committee, a collaboration between GSK, Astra Zeneca and University of Cambridge. His clinical activities are based at Great Ormond Street Hospital as an Honorary Consultant in Paediatric

Rheumatology. Prior to joining GSK, Kiran trained as an academic paediatric rheumatologist. During his PhD at Institute of Child Health, UCL, Kiran was the first to show that IL-17 secreting T cells played a pathogenic role in childhood arthritis (Juvenile Idiopathic Arthritis (JIA)). This was followed by a Wellcome Intermediate Clinical Fellowship working in the group of Prof Mauri, investigating the molecular control of B cell differentiation using a mouse model of autoimmunity. Four years of murine research was enough to convince him of the importance of researching medicines in man!



**Gerard Pasterkamp (MD, PhD)** is Professor of Experimental Cardiology and laboratory of clinical chemistry, UMC Utrecht. The laboratory houses 60 researchers and technicians that cover a broad range of activities: biomarker discovery in cardiovascular research, large animal drug testing and thrombosis and hemostasis. His research interests are in the field of cardiovascular biology and more specifically innovation in biomarkers and drug targets. The research group houses the largest atherosclerotic plaque biobank worldwide: Arhero Express including >4000 patients. This biobank has generated new insights into determinants of plaque destabilisation. For example, it has been demonstrated that local plaque characteristics are strongly associated with long term outcome but also that plaque characteristics have rapidly changed in the last decade. The laboratory now invests in the excavation of genetic

determinants of atherosclerotic plaque characteristics. Cells (cohort of 700 patients where all cell fractions have been isolated) and Aneurysm Express. Private/public research projects are one of the main core-activities within the laboratory of experimental cardiology. In the UMC Utrecht he is a member of the focus theme "Innovation and Valorisation". Recently he initiated with 6 other founders from 3 Universities an inter-academic office that will act as a science broker specialized in the field of cardiovascular biomarkers and drug targets ([www.cardiolaborate.org](http://www.cardiolaborate.org)). Within the laboratories spin off activities are stimulated. He is co-founder of the biomarker company CAVADIS and another spin off from the group is ENCARE. The company ENCARE is currently executing the last pig studies to test a biological entity that prevents heart failure after myocardial infarction before entering in studies in humans.



**Berent Prakken (MD, PhD)** is vice-dean and director of the biomedical education centre at the UMC Utrecht, the Netherlands. He is also professor of pediatric immunology at the UMC Utrecht and honorary professor at the University of Ghent, Belgium. Berent Prakken has built a translational research lab that focuses on regulation of inflammation and biomarker development in human inflammatory diseases. He received numerous national and international awards and grants for his work. The work of his group is published in all major international journals, including Nature Medicine, PNAS, Immunity, the Lancet, JCI and in Nature Reviews journals. Over the years he successfully mentored more than 40 PhD

students. Prakken serves in various national and international research advisory boards and was (until 2015) member of the Dutch National Health Council ('Gezondheidsraad'). He was vice-chair of Medical Ethical Review Board (IRB) of the UMC Utrecht. Berent Prakken is president of the Pediatric Rheumatology European Society (PRES) and member of the EULAR executive committee. He is member of the steering committee of UCAN (international federation facilitating biological research in arthritis) and has set up the first international platform for biological studies in arthritis (UCAN-U, [www.ucan-u.org](http://www.ucan-u.org)). Berent Prakken's personal commitment is to collaboration, training and education.

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## About the speakers and moderators

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**Dr. Norman Rosenblum (MD)** is Professor of Paediatrics, Physiology, and Laboratory Medicine and Pathobiology at the University of Toronto, and a Paediatric Nephrologist and Senior Scientist in the Research Institute, the Hospital for Sick Children. He is the recipient of a Tier I Canada Research Chair in Developmental Nephrology. Dr. Rosenblum is a MD graduate of Dalhousie University. He completed a Pediatric residency and a fellowship in Pediatric Nephrology at the Children's Hospital, Boston followed by a postdoctoral fellowship in the laboratory of Bjorn Olsen in the Department of Anatomy and Cell Biology, Harvard Medical School. Dr. Rosenblum was recruited in 1993 as a clinician scientist to the Hospital for Sick Children and University of Toronto. Since then, the focus of his research has been to elucidate molecular mechanisms that control normal and malformed kidney development in genetic mouse models with a focus on signaling by bone morphogenetic, WNT and Hedgehog proteins. His lab has generated several models of human kidney-urinary tract malformation. He has published over

110 peer-reviewed original manuscripts and book chapters. Dr. Rosenblum is deeply engaged in developing and managing career development programs for clinician scientists. He founded and led the Canadian Child Health Clinician Scientist Program from 2001-2012. In his present role as Associate Dean, Physician Scientist Training in the Faculty of Medicine, University of Toronto, he is Director of both the MD/PhD and Clinician Investigator Programs. Dr. Rosenblum is immediate Past-President of the Canadian Society for Clinical Investigation and a current member of the Council of the American Pediatric Society. Norm is a founding member of the EUREKA Institute for Translational Medicine and served on the Board of Directors and the Faculty of the Certificate Course (2008-2017). Dr. Rosenblum is the recipient of the 2004 Aventis Pasteur Research Award, the American Pediatric Society inaugural 2006 Norman J. Siegel New Member Outstanding Science Award, the Society for Pediatric Research 2010 Maureen Andrew Award in Mentoring, and the Kidney Foundation of Canada 2011 Medal for Research Excellence.



**Hester den Ruijter (PhD)** is a translational scientist at the Laboratory of Experimental Cardiology in the UMC Utrecht. Her research focusses on sex differences in cardiovascular diseases. She has been trained in fundamental research as well as in clinical epidemiology. Currently, she coordinates the Dutch Heart Foundation funded consortium Queen of Hearts, a consortium that works on biomarker discovery - a clinical test - to improve the diagnosis of cardiovascular disease in

women. One of our challenges is how to involve stakeholders in the process of translating fundamental knowledge to the patient. Next to her scientific career, she values the communication towards our society and she is an active member of Women Inc, an organization that raises national awareness that important sex differences in medical research and care are currently neglected.



**Casper Schoemaker (PhD)** is a senior researcher and project leader at the National Institute for Public Health and the Environment (RIVM), the Netherlands. He is the father of a JIA patient. One day a week he works as a patient representative / researcher at the Wilhelmina Children's Hospital. He is representing the Dutch JIA Association in several research projects and in the Dutch JIA-guideline panel. As a member of the JIA Patient Council Children's Hospital UMC Utrecht, he works closely together with several research groups at the Children's Hospital UMC Utrecht. He is co-principal investigator of 'Erratic and yet reliable'. This funded research project is about ways to overcome social consequences in

school, work and sports of erratic chronic diseases (including JIA) in adolescents and young adults. As a patient advocate at the Lung Foundation of the Netherlands, Casper appraises research proposals from a patient perspective. In the process of patient involvement and engagement he can build on his professional experience with stakeholder participation (including patients) in the Dutch Public Health Status and Forecast 2014 report. He was a member of the editorial board of the leading Dutch mental health journal, including patients. In these years he experienced how patients can make a valid and valued contribution to scientific discussions.

## About the speakers and moderators



**Vicki Seyfert-Margolis (PhD)** founded My Own Med in January 2013, based on over two years of work on a database, web and mobile application platform technology for family based co-management of health. Previously, Dr. Seyfert-Margolis was the Senior Advisor for Science Innovation and Policy in the Office of the Commissioner of the US Food and Drug Administration. While at the FDA, she oversaw the development and execution of an agency wide strategic plan for regulatory science. Prior to the FDA, she served as Chief Scientific Officer at the Immune Tolerance Network (ITN), a non-profit consortium of researchers seeking new treatments for diseases of the immune system. At ITN, Dr. Seyfert-Margolis oversaw the development of over 20 leading edge assay development and centralized laboratory facilities, bringing

them to GLP and CLIA compliance. She designed and implemented biomarker discovery studies for over 25 Phase II clinical trials across a broad array of immunologically mediated diseases including autoimmune disorders, allergy, and solid organ transplantation. Prior to this, she served as Director of the Office of Innovative Scientific Research Technologies at the National Institute of Allergy and Infectious Diseases at NIH, where she worked to integrate emerging technologies into existing immunology and infectious disease programs. Dr. Seyfert-Margolis completed her PhD in immunology at the University of Pennsylvania's School of Medicine, and her post-doctoral fellowship work at Harvard University and the National Cancer Institute.



**Dr. Anita Small**, sociolinguist, educator and researcher, is owner of small LANGUAGE CONNECTIONS. Her work focuses on co-creating empowering cultural environments and programs that engage and “give voice” to diverse populations. Clients include non-profit organizations, museums, theatre companies, television broadcasters and educational institutions. She is Co-Founder and past Co-Director of the award winning DEAF CULTURE CENTRE, Canada, the first of it's kind internationally, featuring a museum, art gallery, archives and multi-media production studio. She has obtained over eight million dollars in language and culture project grants. She spearheaded and served as content manager and co-creator of numerous award winning educational productions (over 20 children's books, videos, DVD's, television and website productions). She is most known for her international award winning innovative program development in the Deaf community including a United Nations/ UNESCO World Summit Award (2013) as co-creator of the Deafplanet educational television series and website for best e-content and creativity and the International W3 Award

(2012) as co-creator of the first Animated American Sign Language (ASL) Dictionary for Kids. Dr. Small established the Canadian Literary and Deaf Arts Awards of Excellence through the Canadian Cultural Society of the Deaf and the International Deaf Documentary Awards through Deaf History International. Dr. Small has her Master of Science, Communication Disorders (1981), her Doctorate of Education, Sociolinguistics (1986) and Cultural Mediation Training and Staff Dispute Resolution Training (1992). She taught in the Faculty of Education, York University for 12 years and in the Linguistics Department, University of Toronto. She is cross-appointed to Hogeschool Utrecht University, Institute for Sign Language & Deaf Studies and Knowledge Centre, Netherlands. She has taught, consulted and provided cross cultural mediation training in the U.S., Canada, Japan, Italy and the Netherlands with Deaf and hearing personnel for 25 years. Dr. Small is recipient of the Kathy Dolby Award (2006), the singular national award from the Canadian Deaf community given to a hearing individual. [www.anitasmall.com](http://www.anitasmall.com)

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## About the speakers and moderators

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**Rosalind Smyth (CBE, FMedSci)** is Director of the UCL Institute of Child Health and Honorary Consultant Respiratory Paediatrician and non-executive director of Great Ormond Street Hospital NHS Foundation Trust. She graduated in medicine from Clare College, Cambridge and Westminster Medical School and trained in paediatrics in London, Cambridge and Liverpool. Until September 2012, she was Professor of Paediatric Medicine in at the University of

Liverpool and was Director of the NIHR Medicines for Children Research Network, which supported all clinical research with children in England, from 2005-2012. Her research interests include viral/host interactions in the airways, drug safety and efficacy in children. She is a fellow and recent council member of the Academy of Medical Sciences and was awarded CBE in 2015 for services to the regulation of medicines for children.



**Ghislaine van Thiel (PhD)** obtained a Master's degree of Health Sciences at Maastricht University. She specialized in Medical Ethics and Philosophy of Science. Currently she is Associate Professor at the Julius Center for Health Sciences and Primary Care of the UMC Utrecht. Her main research

topics are ethics of drug regulation and development, and clinical ethics. Ghislaine is a member of the Dutch Health Council, a chair of the Medical Research Ethics committee at the UMC Utrecht and she teaches medical ethics at the Faculty of Medicine.



Eureka alumnus **Bas Vastert (MD, PhD)** is a pediatric rheumatologist in the Wilhelmina Children's Hospital Utrecht. He obtained his PhD on mechanisms of disease and therapy in severe Juvenile Idiopathic Arthritis in 2013. From 2013, he started a translational research line in the laboratory of translational immunology and from March 2017 he is co-heading the v Loosdregt-Vastert group within the Laboratory of Translational Immunology in UMC Utrecht, focusing on novel therapeutic strategies in JIA through translation of cutting edge basic science into clinical benefit. He is currently leading a multi-center prospective trial

implementing a biomarker driven stop-strategy for rIL-1RA therapy in systemic JIA in the Netherlands and setting up a clinical trial studying the potential of Nicotinamide (vitamin B3) in therapeutic strategies for non-systemic JIA. In 2016 he organized, together with EUREKA alumnus Klaus Tenbrock ((Aachen, Germany) a fund raising bicycling tour, from Utrecht (Netherlands) to Genua (Italy). The UCAN Ride for Arthritis (UCANR4A.eu) boosted international research collaboration, raised > 30 000 euro on private donations and received major media attention (local as well as national) in the Netherlands, Germany, Switzerland and Italy.



**Hilde De Winter (MD)** is sr. medical director at Ablynx, a Belgian biotech company, and is the medical lead on the the anti-vWF Nanobody caplacizumab program in acquired TTP. Hilde has more than 10 years of experience in clinical development and medical affairs in affiliate, regional and global roles at Amgen, OncoMethylome and Innogenetics.

Most recently, Hilde was intl. development medical director, European medical lead for inflammation pipeline products at Amgen. Hilde obtained her MD degree at the University of Ghent, Belgium. She is board-certified in internal medicine, and conducted postdoctoral research training in immunology at the La Jolla Institute for Allergy and Immunology.

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## About the speakers and moderators

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**Femke van Wijk (PhD)** was trained as a biologist and obtained her PhD in immunology at Utrecht University, the Netherlands. She completed her postdoctoral fellowships at the UMC Utrecht and the La Jolla Institute for Allergy and Immunology, USA. Currently she is an Associate Professor in Translational Immunology at the UMC Utrecht, and visiting Professor at the Guangzhou Medical University, China. Her research focuses on understanding the mechanisms of human

T-cell development, plasticity and regulation in chronic inflammation. She is a recipient of the Veni and Vidi career development Awards and she is an active member in several international networks, including UCAN-U and Eureka, that aim to share data and resources for human research and training of translational scientists. In 2017 she received the PhD daily supervisor of the year award by the Utrecht Graduate School of Life Sciences.



**Nico Wulffraat (MD, PhD)** is professor of pediatric Rheumatology and chair Research and Education at the department of pediatrics, UMC Utrecht, The Netherlands. He obtained his MD and PhD (1987) at the Free University Hospital Amsterdam. He was trained as a pediatrician from 1988 until 1993 in Utrecht. He was fellow pediatric immunology and rheumatology from 1993 to 1995 in the WKZ. Since 1995 he worked as a consultant in pediatric immunology-rheumatology. Since 2010 he is the head of this subunit. From 1997-2004 he was also coordinator of the pediatric allogeneic and autologous stem cell transplantations for immune deficiencies, metabolic disorders and autoimmune diseases. Field of Study in Pediatric Rheumatology are interventions in the regulation of chronic inflammation (medication, immunisation and cellular therapies). He is PI of several investigator initiated trials of immunisations in children with rheumatic diseases including MMR, HPV and Men C,

prevention of MTX side effects, and application of Mesenchymal Stem cells in refractory GvHD and JIA. He is coordinator of a FP7 consortium for pharmacovigilance of biologics used in JIA (PHARMACHILD). Also He leads the EHC project SHARE that aims at documenting standards of care and treatment recommendations for pediatric rheumatic diseases throughout Europe. Past memberships are Board of Dutch pediatric rheumatology society, Scientific Advisory Council of the Dutch League for Rheumatology, ZONMW programme committee Translational Adult Stem cell research, ZONMW programme committee Priority medicines for Children, European Medicines Agency (EMA) expert group for Pediatric rheumatology. Currently he is council member of PRES and PRINTO. Dr Wulffraat has co-authored over 240 Pubmed cited papers in international journals on pediatric rheumatology and immunology and 12 chapters in books. Hirsch index is 44.



inspired by the positive energy and commitment shown by everybody in this course to change the current paradigm in research into a better and translational kind of way” Yogi Prawira

“Working together with this amazing inspiring group this week gives me a lot of new positive energy” Judith

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“It all starts with an idea”

Gautam Kok

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“This Summer School has inspired me to perform only research that actually makes a difference, rather than just being interesting or advancing my career”

Remi Stevelink

“For anybody who wants to become a translational researcher, the first and critical step to become one is to participate in this course. Thank you so much for the amazing and inspiring week”

Janneke Peeters

“A Summer course that changes your life, not only professionally but also on personal basis!”

Sabine van der Laan

“This Summer School has made me aware of translational medicine and it’s transition. I’ve not only learned much about this topic, I also learned more about myself as a translational researcher / MD. Thank you so much for this inspiring week!”

Lynne Rumping

“I was inspired by the fact that established and successful scientists are taking the responsibility to help us change the current paradigm of science”

Arthur van der Boog

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“This course has challenged my understanding of true translational research. I am honored to carry the torch to Miami and promote translational medicine and the Apollo program to my peers.”

Michelle Trojanowsky

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“Be brave. Take risks. Nothing can substitute experience”