

# Programme MEB Regulatory Science Day 14 February 2019

Advanced therapies, regulation  
and clinical application

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## About

There are numerous developments in the field of Advanced Therapy Medicinal Products (ATMPs), which include products for tissue engineering, gene and somatic cell therapy. Despite their great potential, only relatively few ATMPs have as yet received a marketing authorisation. However, expectations of the ATMPs field have been boosted by the recent success with CAR-T cells in immunotherapy for cancer. The patient's own immune cells are treated outside the body and programmed to fight the cancer cells. The expectations are high, because the first two registered medicinal products are considered game changers in the treatment of certain types of blood cancer. Also the field of gene therapy treatment of gene defects has clearly matured. Some products have recently been approved. Several others show very promising clinical results and are coming close to applying for marketing authorisation.

What can we learn from the successful developments? Also why has for some ATMPs marketing authorisation been withdrawn and what can we learn from the CAR-T cells? How can you ensure that ATMPs become successful and available to patients? Which (scientific/regulatory/financial) hurdles have been encountered, and what can we learn from the solutions? These are the questions that will be addressed during the MEB Regulatory Science Day.

## Registration

Please use the link below to register:

<https://www.mebscienceday.nl/>

After registering, you will receive a mail confirmation in your inbox.

For more information:

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# Programme

**Chair** Dr Marjon Pasmooij, Science Programme Manager MEB

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|---------------|----------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| 12.00 - 13.00 | <b>Registration and lunch</b>                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                  |
| 13.00 - 13.05 | <b>Opening and welcome</b><br>Hugo Hurts, director MEB                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                         |
| 13.05 - 13.20 | <b>Current trends in ATMPs as seen by CAT</b><br>Dr Carla Herberts, senior clinical assessor MEB and alternate CAT member                                                                                                                                                                                                                                                                                                                                                                                                                                                                                      |
| 13.20 - 14.05 | <b>Designing ATMPs with the end in mind</b><br>Prof. Marc Turner, Professor of Cellular Therapy at the University of Edinburgh and Medical Director at the Scottish National Blood Transfusion Service                                                                                                                                                                                                                                                                                                                                                                                                         |
| 14.05 - 14.35 | <b>Lessons learned from Yescarta</b><br>Dr Bethany Dudek, Kite Pharma                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                          |
| 14.35 - 14.50 | <b>Regulatory science poster pitches of PhD candidates and students</b><br>Renske ten Ham, PhD candidate, <i>Challenges in development and marketing authorisation of Advanced Therapy Medicinal Products in Europe</i><br>Delphi Coppens, PhD candidate, <i>A decade of marketing approval of gene and cell based therapies in the United States, European Union, and Japan: A regulatory decision-making evaluation</i><br>Tahira Nakchedi, Master student, <i>Regulatory experience with non-clinical studies of cell-based therapies: An analysis of studies on the biodistribution and tumorigenicity</i> |
| 14.50 - 15.20 | <b>Break and poster session</b>                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                |
| 15.20 - 15.50 | <b>Experiences of the Dutch Health and Youth Care Inspectorate regarding ATMP GMP inspections and Hospital Exemptions</b><br>Dr Christianne Reijnders, Senior inspector IGJ                                                                                                                                                                                                                                                                                                                                                                                                                                    |
| 15.50 - 16.20 | <b>Panel discussion led by Dr Hans Ovelgönne, CAT member</b><br>Panel: Dr Carla Herberts, Prof. Marc Turner, Dr Bethany Dudek, Dr Christianne Reijnders, Dr Yolanda van Kooij                                                                                                                                                                                                                                                                                                                                                                                                                                  |
| 16.20 - 16.30 | <b>Reflection on the day</b><br>Hugo Hurts, director MEB                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                       |
| 16.30 - 18.00 | <b>Drinks and networking</b>                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                   |

Anyone who uses medicines should be able to trust them. The MEB is dedicated to fulfilling this mission every single day, in the Netherlands and in Europe. Good medicines used better.

**Contact**

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