|  |  |
| --- | --- |
| **Titel** | From bed to bench and back, to the future: Huntington’s disease |
| **Datum** | 26 oktober 2018 |
| **Cursusinhoud** | This course provides an overview of future trends in healthcare and therapy of neurodegenerative disorders based on experience in Huntington’s disease gathered over the last 40 years.. Speakers will address the developments that have been made in the care of patients with chronic disease in their final stages and the care of individual patients during the course of their disease. Also the patient within the family system as a network of care will be discussed.    In 1978 the diagnosis of Huntington’s disease was made on the clinical picture of chorea and a positive family history, hopefully confirmed earlier by examination of the morphology of the brain of a clinical certain case. A lot of progress has been made after the finding of the linkage in 1983 and the gene in 1993. From then on, the whole new field of premanifest testing in neurological disorders started. It gave a huge input into basic research with only one aim: to find a useful therapy. The steps made in the lab must be transferred to the clinic. The question is how to do that properly, with the restricted amount of money and clinical available patients. The field is eager to participate so the clinical trials must be structured very rigid so that the maximum result comes out each trial. The rarity of the disease hinders the progress, the other side of the coin is that it made a strong point by organizing a worldwide collaboration possible, which otherwise never could have been reached.    This day will also illustrate how other chronic diseases can learn from these findings and vice versa. |
| **Doelgroep(en)** | Neurologen, klinisch genetici, specialisten ouderengeneeskunde en  i.o. |
| **Leerdoelen** | Achterberg  To have knowledge about the development of clinical studies in a nursing home setting. To learn how to change culture and develop collaboration for studies in a fragile group.  Aziz  To gain knowledge about the laboratory experiments with its pitfalls and how to translate the results to the clinics.  Bloem  To gain knowledge how to organise care for chronic diseases in a multidisciplinary way. To learn how care can be made better and less expensive.  Booij  To gain more knowledge about the Dutch law on euthanasia and physician assisted suicide and the possibilities a patient with a movement disorder has within the scope of this law. To provide aids for physicians on how to use the possibilities provided for in the law when discussing the difficult topic of end of life care with their patient.  Craufurd  To gain knowledge about the system in which premanifest testing is done and learn about the impact of the result of testing for the individual and the family, with Huntington disease as a model.  Durr  To gain knowledge about the genetic background of so-called monogenetic diseases and the interaction with other genes.  Kieburtz  To learn about current trends in US rare disease drug approvals, and to appreciate the diversity of clinical trial designs that may support new drug approvals.  Landwehrmeyer  To gain knowledge how to organise care combined with research for rare disorders in a global network, with Huntington disease as a model.  Reilmann  To gain knowledge how to use animal models in detecting new pathophysiological pathways, with finally the aim to develop new treatments.  Tabrizi  Learning about implementation of new drug developments into clinical practice, illustrated on Huntington’s disease. |
| **Leden cursus -commissie** | * Dr. S.T. Bot, Dept. of Neurology, LUMC, Leiden, The Netherlands * Prof.dr. R.A.C. Roos, Dept. of Neurology, LUMC, Leiden, The Netherlands |
| **Sprekers** | * Prof.dr. W. Achterberg, Dept. PHEG, LUMC, Leiden, The Netherlands * Dr. N.A. Aziz, Dept. of Neurology, LUMC, Leiden, The Netherlands * Prof.dr. B.R. Bloem, Dept. of Neurology, Radboud University Medical Center, Nijmegen, The Netherlands * Dr. S.J. Booij, CWZ Ziekenhuis, Nijmegen * D. Craufurd, Dept. of Genetics University of Manchester, Manchester, UK * Prof.dr. A. Durr, Coordinator of the Reference centre for Rare diseases-Neurogenetics; ICM (Institut du Cerveau et de la Moelle épinière) Pitié-Salpêtrière Hospital, France * Prof. dr. J.J. van Hilten, Dept. of Neurology, LUMC, Leiden, The Netherlands Dr. K. Kieburtz, University Rochester medical Centre, New York, USA * Prof.dr. G. B. Landwehrmeyer, Dept. of Neurology, University Hospital, Ulm, Germany * Dr. R. Reilmann, George-Huntington-Institut GmbH, Münster, Germany * Dr. W.M.C. van Roon-Mom, Dept of Genetics, LUMC, Leiden, The Netherlands * Prof.dr. S. Tabrizi, Director of the Huntington’s Disease Center at University College London, Institute of Neurology, London, UK * Prof. dr. A. Tibben, Dept. of Clinical Genetics, LUMC, Leiden, The Netherlands * Prof.dr. J.J.G.M. Verschuuren, Dept. Of Neurology, LUMC, Leiden The Netherlands |

**Programma**

09.30-09.55 Registration and coffee

chair Willeke van Roon

09.55-10.00 Welcome and Introduction

10.00-10.20 Suzanne Booij Palliative care or euthanasia

10.20-10.40 Wilco Achterberg Top care development in Nursing homes

10.40-11.05 Bas Bloem Can every disease have its own network of care?

11.05-11.20 Tea/coffee break

chair Aad Tibben

11.20-11.45 Bernard Landwehrmeijer Development of mondial platforms: HD as a model

11.45-12.10 Ralf Reilmann Animal models for neurodegeneration

12.10-12.30 Ahmad Aziz How to link the lab to the clinics

12.30-13.20 Lunch

chair Bob van Hilten

13.20-13.40 David Craufurd Psychiatric aspects of at-risk testing

13.40-14.05 Alexandra Durr How monogenetic is Huntington’s disease?

14.05-14.30 Karl Kieburtz How many patients to include in a clinical trial of a neurodegenerative disorder?

14.30 -15.00 Sarah Tabrizi The future clinics of Huntington’s disease

15.00-15.05 Jan Verschuuren closing remarks

15.00-15.30 Tea/coffee break

Walk to the Academiegebouw, Rapenburg 70 (20 min)

16.15-17.00 Farewell Lecture (Academiegebouw)

Raymund Roos

17.00- 18.30 Drinks in the Faculty club