



EAP Winter Meeting - Rare Diseases | Ethics Friday, 7 December, 2018 | 11:30-13:30

Chair *Liesbeth Siderius*

1. Welcome and approval of the agenda (5 min)
2. **It can be done differently!** (20 min)
Patient organization takes a lead in Orphan Drug development
Kees van der Graaf
past president Unilever Europe

Discussion on orphan drugs
3. Follow up on Spring meeting Sofia, Bulgaria 2018 (5 min)
 - Access to health document
 - What has been happening at national levels
4. UEMS MJC Rare and Undiagnosed Diseases (5 min)
Report
5. Other business (5 min)

Ethics 12.15-13.30

Chair: *Joe Brierly*

1. Round table discussion about purpose of the group
2. Membership discussion
3. Existing projects
4. Possible projects & collaboration
 - a. Possible relevant grant calls
 - b. ELPAT – Ethical, legal and psychological aspects of transplantation paediatric group
 - c. ECHO – European Children’s Hospitals Alliance
 - d. Limits of parental choice – a European study
 - e. Ethics support available in European hospitals caring for children – an online survey
5. Tasks and next meeting



European Academy of Paediatrics
Paediatric Section of U.E.M.S (European Union of Medical Specialists)



Information on Kees van der Graaf

'In 2006 I discovered my deeper purpose in life: Developing a cure for the muscular dystrophy called FSHD. I retired early from Unilever to dedicate the rest of my life to reach this goal. The reason is simple. My eldest son Bart is suffering from this disease. In 1997 I created the Dutch FSHD Stichting (Foundation). This foundation is raising money to fund the research into FSHD. In 1998 I was the co-founder of Spieren voor Spieren (Muscles for Muscles). The goal for this organization is funding research for muscle diseases with a young onset. In 2011 the genetic mechanism of FSHD was discovered. For me the moment to consider the creation of a bio-tech start-up to develop a causal therapy for FSHD. Together with Australian banker Bill Moss I founded in September 2014 the Facio group of companies. Anno 2016 this company has now developed a unique method to measure the level of expression of the bad gene called DUX4. This will enable us to screen for small molecules that could repress DUX4.

All my other engagements are supporting my FSHD activities. My non-executive roles keep me sharp, rooted in the real world, networked and provide me with the funds to do what I need to do for FSHD. Lecturing about leadership, complexity management, alternative business models and the importance of having a corporate and personal purpose help me to spread my strong believes about values and acceptable behavior, and the importance of a triple bottom line/shared value.'