University Medical Center
Utrecht

Date 20 April 2017
Regarding **Application for board member of ECFS**

Dear Sir or Madam:

I hereby present myself as candidate to serve as a member of the board of ECFS. I currently head the CF research laboratory at the UMC Utrecht, and my passion is to solve clinical problems by innovative, multidisciplinary research. I was trained as molecular biologist in an immunological context. Using my molecular background, about 8 years ago I shifted my attention to CF, aiming to develop personalized readouts to better understand individual relations between CFTR genotype, function and responses to therapeutics. My lab has pioneered the personalized application of CFTR modulators via patient-derived cell systems such as intestinal organoids. It represents one of the major breakthroughs in CF research in Europe, and has been awarded with several awards in the past years including the first ECFS Gerd Doring award, and the ECFS award 2017.

I can fully connect to the mission statement of the ECFS, and I think patients are the start and endpoint of research. My largest aim as board member would be to set up infrastructures to increase the translational potential of our basic research efforts in Europe. Such infrastructures are highly needed and will be complementary to the successful ECFS infrastructures such as the patient registry and the clinical trial network that mostly serves the clinical care and research communities.

One such critical infrastructure would be accessible biorepositories of patient tissues that are linked to the registry. ECFS can play a critical role in initiating and organizing high quality biorepositories with transparent governance models. A business model needs to be developed to enable long-term sustainability and support from all key stakeholders.

Another critical need is to further streamline Europe's basic research efforts. The European CF community is already highly interactive and sharing, but I think we can do more. Cross-border prioritization of our aims for basic research, alignment and collaborations with patient foundations, defining the critical experts, and increased access to translational knowhow are all needed. ECFS can further assist in developing infrastructures to stimulate these processes.

Importantly, we need to keep inspiring our younger researchers. It is a tough road for new people who are trying to develop their own research lines in a rare disease field. The above infrastructures should support this new generation by providing scientific networks and high quality research tools and protocols.

Finally, the organoid work enabled a rapid translation of basic research finding to clinical impact in 4 years. I will bring the critical lessons learned in this process to the ECFS board, as well as access to a large network of people active in the CF community as scientists, clinicians, patient representatives, drug

Pediatrics

Dept. of Pediatric Pulmonology

J.M. Beekman
Associate Professor

Tel +31 88 75 542 72
Fax +31 88 75 547 47
jbeekman@ umcutrecht.nl

Visiting address:
Lundlaan 6
3584 EA Utrecht
The Netherlands

Correspondence:
Huispostnummer KH.01.419.0
P.O. Box 85060
3508 AB Utrecht
The Netherlands

www.umcutrecht.nl

University Medical Center
Utrecht

developers, and regulators.

I thus look forward to contribute to the European CF community and hope to help establish new infrastructures to improve our ability to turn new discoveries into impact for patients.

Sincerely yours,

JM Beekman, Ph.D

Department of Pediatric Pulmonology
UMC Utrecht