EU-US Interaction:
Collaboration European Cystic Fibrosis Society Clinical Trials Network (ECFS-CTN) with US Cystic Fibrosis Foundation –Therapeutics Development Network (CFF-TDN)

Update for EnprEMA Meeting, May 16th, 2017
Tim Lee, ECFS-CTN Director
Establishment of the CF Clinical Trial Networks

- CFF-TDN established 1998
- ECFS-CTN established 2008
  - accelerate pace of development
  - co-ordinated approach
  - reflect research priorities of pwCF
  - optimal study design and safety
  - increase efficiency
- CFF-TDN 82 sites; 27,000 patients
- ECFS-CTN 43 sites; 17,500 patients

Drug Pipeline CFF-TDN website May 2017: CFTR modulator drugs
From the start: Close Collaboration

Michael Boyle, M.D.
Senior Vice President of Therapeutics Development, Cystic Fibrosis Foundation

An Inspirational Message on the Worldwide CF Community Effort to Advance CF Therapies
By Michael Boyle, M.D. | October 28, 2016

There was a clear message in today's second plenary at NACFC: no matter what role you play -- physician, scientist, person living with CF, parent, fundraiser, regulator -- it is going to take a tremendous team effort to advance new therapies as fast as possible and eventually find a cure for CF.

Topics:
North American CF Conference, Our Research Approach

About Michael Boyle, M.D.
Michael Boyle, M.D., is the senior vice president of therapeutics development at the Cystic Fibrosis Foundation and an adjunct professor of medicine at The Johns Hopkins Hospital. Dr. Boyle oversees the clinical development programs of new treatments for the CF Foundation, as well as the Foundation's Therapeutic Development Network of 82 academic research centers.

Dr. Boyle is internationally known for his clinical research in CFTR modulators and difficult-to-treat
US Perspective

• CF is a rare disease – mainly US; Europe; Australia
• The pipeline is massive and the opportunity for progress is now
• In US already widespread availability of first generation CFTR modulators eg Orkambi®, thus some studies more feasible/appropriate in Europe
• There is not the capacity to just conduct these studies in the US
ECFS-CTN – CFF-TDN Practical Interaction

• Monthly teleconferences between Co-ordinating Centres / Senior Team
• Discuss general strategy especially trial design challenges as the “standard care” landscape changes – respond to regulator consultations
• Collaborate on “Global” TDN-ECFS CTN protocol reviews for sponsors
• Develop “Global” standard operating procedures for clinical trials – eg standardised sweat test methods etc
• George attends our Jan Steerco; myself and CTN co-director attend TDN October Steerco
• CFF-TDN team also attend / co-deliver our annual CTN Training Day in June
• Share the same “study weightings” for our site and study metrics reporting system, so we can compare performance
Joint Meetings: ECFS-CTN Annual Steerco Prague
Patients enrolled into CTN approved studies by year
ECFS-CTN
Site Activity
2016

**Median = 6 studies**

**Median = 7% CW-enrolment**
US CFF support to ECFS-CTN

• Helped fund our Trial Metrics Reporting System and Quality Manager
• Responded to our Site Capacity Survey (demonstrating shortage of CF Research Nurses as the major barrier at 20 sites) with substantial further investment to support pump-priming of 20 additional research nurses (or equivalent) across ECFS-CTN network – these are being appointed summer 2017
• NB funding also from European CF patient organisations and income for services to Pharma
• NB ECFS-CTN also interacts closely with National European Research Networks
ECFS-CTN and CFF-TDN co-operation challenges

• Ultimate drug affordability for healthcare may differ between US and some European countries
• Set-up in certain European countries can seem more complex/slower than US
• Some conflicts when we advocate for clinical trials access for our own continent’s patients
• Some differences in regulatory approach
• Some individuals do not have global view (yet)
EU-US Interaction:
Absolutely vital in our field
Many thanks to CFF and CFF-TDN for this productive collaboration