



CFE/ECFS JOINT SYMPOSIUM, WEDNESDAY JUNE 10

ACCESS TO NEW THERAPIES:

How can patient organizations, researchers, health professionals, authorities and industry contribute?

HALL 400 (LEVEL 4), THE SQUARE, BRUSSELS

09.00-09.15: Access to CF therapies in Europe, Karleen De Rijcke, CF Europe

09:15-10:30: Accelerating access to new therapies: *The Patient, Academic and Industry point of view*

- Ulrike Pypops, CF Belgium, the patient point of view
- Prof. Isabelle Fajac, Physiology Department, Cochin Hospital, Paris, France
- Vertex Pharmaceuticals, Senior Vice President International, Simon Bedson
- Manuela Maronati, VP Marketing & Patient Advocacy, PTC Therapeutics, GmbH,

10.30-11.00: Coffee break

11.00-11.30: The regulatory point of view

- Orphan drug regulation and authorization procedures in Europe (Eurordis, Yann Lecam, tbc)
- What does/can the EU and national authorities do to enhance access? (TBC)

11.30-12.00: Collaboration can accelerate the access to new treatments for CF patients

Models of collaboration:

- ECFS clinical trial network , Dr Tim Lee, Leeds Regional Paediatric CF Centre, UK
- CFF drug pipeline: collaboration with pharmaceutical companies to enhance the development of CFTR- correctors and potentiators, Dr. Preston Campbell, USA

Changing the Reimbursement Decision for 100 Cystic Fibrosis Patients in Ireland

How CF Ireland successfully reversed the decision on the reimbursement of a cystic fibrosis medicine, Philip Watt, CEO, Cystic Fibrosis Ireland

12.00-12.30: Panel discussion (academic, patient, industry, regulatory point of view)

How can all stakeholders collaborate to ensure that CF patients in all European countries have equal access to CF treatments?

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