MUKOVISZIDOSE
Mukoviszidose Institut gGmbH

20th Scientific Meeting

CF Registry: Ready for science!? – Value of registry data to improve CF care

09th – 10th September 2021

online

Organization

Scientific advisory board of the German Research Community for Cystic Fibrosis (FGM) & Mukoviszidose Institute gGmbH (MI)

Chairs

Lutz Nährlich (DE/Gießen)

Carsten Schwarz (DE/Potsdam)

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Please not that not all talks have a published abstract.

Program

Thursday, September 9th

12:15-12:30 pm	*Dial In*
12:30-12:45 pm	**Opening of the meeting + short introduction**
	Member of the Federal Executive Board Mukoviszidose e.V. Anna-Maria Dittrich (DE/Hannover)
	Chairs of the meeting Lutz Nährlich (DE/Gießen) / Carsten Schwarz (DE/Potsdam)
12:45-1:45 pm	**Session I: Lessons learned from other disease areas**
12:45-1:15 pm	**German Childhood Cancer Registry: Experinces and lessons learned** Friederike Erdmann (DE/Mainz)
1:15-1:45 pm	**Swedish Rheumatology Quality Registry** Staffan Lindblad (SW/Stockholm)
1:45-2:00 pm	*Break*
2:00-3:00 pm	**Session II: Data structure and quality**
2:00-2:30 pm	**Annual Review or Encounter data in CF Registries – which is best?** Rebecca Cosgriff (UK/London)
2:30-3:00 pm	**Harmonization** Lutz Nährlich (DE/Gießen)
3:00-3:30 pm	*Long break*
3:30-4:30 pm	**Session III: Focus on the patient**
3:30-4:00 pm	**Patient Reported Outcomes** Siobhán Carr (UK/London)
4:00-4:30 pm	**PROM and Real Life data project under CFE involvement** Elise Lammertyn (BE/Brüssel)
4:30-4:45 pm	*Break*
4:45-5:30 pm	**Keynote-Session**
	Bruce Marshall (US/Bethesda)
5:30-6:00 pm	**Open Discussion**

Friday, September 10th

8:30-8:45 am	*Dial In / Opening second day*
8:45-9:45 am	**Session IV: Study design**
8:45-9:15 am	**BEAT-CF - an adaptive platform trial** André Schultz (AU/Nedlands)
9:15-9:45 am	**CF-Start - a randomized registry study** Kevin Southern (UK/Liverpool)
9:45-10:00 am	*Break*
10:00-11:30 am	**Session V: Approval, safety & efficacy – Focus on Pharmacovigilance and HTA**
10:00-10:45 am	**HTA perspective** Beate Wieseler (DE/Köln)
10:45-11:15 am	**Statistical perspective** Tim Friede (DE/Göttingen)
11:15-11:30 am	**EMA perspektiv** Lutz Nährlich (Gießen)
11:30-11:45 am	*Break*
11:45-1:15 pm	**Session VI: Analytic strategies**
11:45-12:15 pm	**Survival analysis** Ruth Keogh (UK/London)
12:15-12:45 pm	**Longitudinal analysis** Daniela Schlüter (UK/Liverpool)
12:45-1:15 pm	*Talk will be announced soon*
1:15-1:45 pm	**Open Discussion**
1:45 pm	**Closing of the Meeting**

Please note that the schedule is given in Central European Summer Time (CEST)

Session I

Better health by science based care and care based science - the Swedish rheumatology example

Staffan Lindblad

SRQ is the Swedish Rheumatology Quality register which is a national database of individual data on patients with rheumatologic diseases and the health outcomes of therapies used. SRQ offers a decision support for patients in their daily life and making decisions together with their doctor on changes in therapy depending on the current health outcomes and the preferences of the patient. At the same time the national database is used for quality improvement purposes to better health care processes and their outcomes on a group level e.g. clinic or region as well as over time. Finally, the national database is used for research which has yielded hundreds of publications on the development of rheumatic diseases and the use and effects of new drugs and other interventions.
The underlying concepts to make all this work is presented together with examples of applications in other disease areas e.g. Cystic Fibrosis.

Session II

Enhancing Real World Data in Cystic Fibrosis: Supporting CFE Member Patient Organisations

Elise Lammertyne

CF Europe has an important role to play supporting its member organisations to spread Real World Data capabilities. While some countries are advanced in collecting RWD, there is variability across Europe. CF Europe's recent survey of patient organisations showed the huge variability in clinical and patient reported outcomes collection and reporting. The survey also highlighted the desire from patient organisations for the latest information and best practice examples of RWD collection.

Three themes emerged from the project's scoping phase:

1. Efficient best practice sharing and dissemination.
2. Operationally support countries that need to build RWD capabilities from scratch, in support of access to new medications also.
3. Aggregate EU level data with the countries that want to empower their country level data with more patients from other countries.

As this project develops, CF Europe will bring in new partners (tech and med tech) and develop an ecosystem approach of working with them to set up pilot areas, led by patient organisations.

Session IV

BEAT CF -A Bayesian Informed Adaptove Platform Trial for CF

André Schultz

Pulmonary exacerbations of CF contribute to progressive, irreversible lung damage. Exacerbations are typically managed with antibiotic regimens and other treatments even though evidence for treatment regimens is scarce. Over the past several decades clinical trials have done little to advance the management of exacerbations. BEAT CF is a Bayesian informed adaptive clinical trial platform for the study of exacerbations. This presentation will describe BEAT CF that is currently recruiting patients across multiple Australian sites and will commence randomization to treatments soon.

Session V

Approval, safety & efficacy - Focus on pharmacovigilance and HTA: the HTA perspective

Beate Wieseler

After a regulatory decision on approval, new drugs become available for use in clinical practice within health care systems. At this point all European health care systems perform a health technology assessment (HTA) aiming to understand the new drugs' position in the therapeutic landscape of the approved indication. The HTA investigates if a new drug has an added benefit for patients compared to the standard of care. The results of the assessment are required to support treatment decisions (both on a system level and individually) and to inform pricing and reimbursement decisions. Often, at the point of market entry only limited evidence is available comparing a new drug to alternative treatment options. This is specifically true for drugs for orphan diseases. Thus, post-approval data collection is required to close the evidence gaps. Patient registries using various study designs could be an option for this data collection.
The talk will describe recent German developments to support post-approval data collection and the role of patient registries in this initiative. Resulting registry and data quality requirements and potential study designs will be discussed.

Session VI

Estimating survival in CF using national registry data: how it works and some challenges

Ruth Keogh

The life expectancy of people with CF has improved substantially over recent decades. However, estimating survival in CF has a number of challenges. In this talk I will outline how CF survival can be estimated, in particular using national registry data. I will discuss how estimates of survival presented in registry reports, such as median survival, should be interpreted and what assumptions are involved. The handling of transplant is an important consideration, and different countries have available different information on transplant and post-transplant follow-up. I will discuss how different ways of incorporating information on transplant affects survival estimates. Examples will be presented based on analyses of the UK CF Registry and the European CF Society Patient Registry. The talk will also look forward briefly to the challenges of estimating what the effects of CFTR modulators will be on life expectancy in CF.

Longitudinal Analysis of CF Registry Data – Benefits, Considerations and Applications

Daniela K. Schlüter

Cystic fibrosis (CF) registries are an incredible resource for research. Due to their longitudinal follow-up of large sections of whole populations of individuals with CF they allow us to assess changes in the population characteristics over time, changes in the disease course with age, and changes due to the impact of time-varying exposures such as treatments and infections. This presentation will give an overview of how we can maximise the insights we can gain from registry data through longitudinal analysis and will highlight the main points for consideration in order to draw valid conclusions. Some example applications will demonstrate what we have learned from longitudinal analyses of CF Registry data and how they help us better understand the disease course which can lead to improvements in CF care.

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