Can CF patient registries identify pulmonary exacerbations?

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Objectives: To test the internal consistency of markers for pulmonary exacerbations in cystic fibrosis patient registry data and to validate findings against clinical opinion.

Methods: The Australian Cystic Fibrosis Data Registry holds data from individual occasions of measurement of lung function, episodes of hospitalisation and home therapy for respiratory indications and results from respiratory cultures. Initial analysis of 2010 to 2012 data using stata software will identify occasions of measurement of lung function (FEV1 per cent of predicted) that are 10 per cent or more below a ‘normal’ level calculated as the patient’s average of highest measures recorded in each of the preceding four quarters. The temporal relationship of such occasions with hospital and home therapy episodes for respiratory indications will permit internal validity checking and sensitivity analysis of the nominated percentage below ‘normal’. The usefulness of recent prior respiratory cultures as a potential additional consistency check will also be examined. A sample of results will be compared with independently formed clinical opinion.

Results: Results are not available at time of abstract submission. They will include findings and an outline of methodology.

Conclusion: The occurrence or frequency of pulmonary exacerbations can be a specified outcome for clinical trials and is of interest in general research into the progress of cystic fibrosis. Use of pulmonary exacerbations as an outcome in the emerging use of CF patient registries for post-market surveillance of long term drug efficacy requires an understanding of the validity of markers that are available in registry data.

Prevalence of Staphylococcus aureus (SA) infection in patients with cystic fibrosis in a reference unit

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Objectives: Epidemiologic, prospective and observational study of infection by Staphylococcus aureus (SA) in Cystic Fibrosis (CF) patients to evaluate prevalence, incidence search for study sensibilities and mechanism resistance and virulence factors of SA.

Methods: A total of 53 respiratory specimens out of 53 patients in a 5 months period were obtained (June 2013 to October 2013). Both adults and pediatric patients were included. Identification of colonies was performed with manual techniques and/or with automatized methods. With the isolation of resulting SA, sensibility was studied through methods in disc-plate or techniques of microdilution. Detection of Panton-Valentine leukocidin (PVL) gene was done using PCR in real time. Phenotypic methods, included coagulase and agglutination tests were performed. Detection of methicillin-resistant SA (MRSA) was observed in 20.8% (PVL 100% negative) and methicillin-sensitive SA (MSSA) in 79.2% (PVL 26.2% positive) of isolated. PVL was only detected in patients with MSSA isolated. Prevalence of SA infection was observed in 18.92% and an incidence in 3.92% in our unit.

Conclusion: We observed a high prevalence of SA in our CF patients unit and highlight the presence of virulence factors, PVL in our analysis, in MSSA strains. Therefore every SA has its pathogenic potential.

Some cystic fibrosis patients do not tolerate tobramycin dry powder inhalation

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Objectives: Tobramycin Inhalation Solutions (TIS) are required in substantial numbers of cystic fibrosis (CF) patients due to intolerability or incapability to manage Dry Powder Inhalation (DPI). A market survey was performed to specify this sub-population.

Methods: This market survey was conducted in Germany (DE) and Italy (IT) with focus on large CF centres with ≥50 CF patients.

Results: DPI was used in 18%, TIS in 82% of patients. DPI is primarily used in adults. Physicians estimated that 1/3 of patients take less DPI treatments than recommended. Main reasons for non-compliance are side effects as cough and bronchoconstriction and incorrect inhalation manoeuvres. Reasons for prescribing TIS are satisfactory tolerance and long-term experience, but also the generalized benefit of liquid inhalation. Young patients and adults with severe disease benefit most from TIS. DPI is preferred due to time saving and lower treatment burden. Adults/workers and patients under time pressure take most advantage of DPI. 90% of centres have to switch patients from DPI to TIS to overcome side effects.

Conclusion: The survey is regarded representative as the surveyed CF centres cover about 20% of the CF population in DE and IT. Side effects after DPI are often rated more serious than a time-consuming TIS treatment, indicating that DPI is not an ideal treatment for all CF patients, particularly children. A medical need was identified for a fast TIS treatment in patients who prefer liquid inhalation for tolerability reasons.

Adults with cystic fibrosis exhibit an increasing prevalence of chronic kidney disease with increasing age, particularly those with cystic fibrosis-associated diabetes mellitus and/or an organ transplant

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Objectives: As more patients with cystic fibrosis (PWCF) survive into adult life the prevalence of other comorbid conditions may increase. Amongst these is chronic kidney disease (CKD). This is uncommon in children, but is reported with increasing frequency in adults.

Methods: Retrospective single-centre observational cohort study of 120 adult PWCF (aged 18−50yrs) over the period 01/01/11−31/12/12. Patient records were interrogated to detect evidence of CKD, as defined by the KDIGO 2012 Clinical Practice Guideline for the Evaluation and Management of Chronic Kidney Disease.

Results: 74 (62%) male. Median (IQR) age 27 (21, 31) yrs. On 01/01/11, 25 (21%) had cystic fibrosis-related diabetes (CFRD). 5 (4%) subsequently developed this. 10 (8%) had received an organ transplant; 4 (3%) received a transplant during the study period. 5 (4%), all male, died.

Conclusion: Of patients who died, one (20%) had CKD. CKD is a common co-morbidity in this cohort. This increases with age, and has specific associated risk factors. This trend may well continue as the global population of patients with cystic fibrosis ages.