Children with cystic fibrosis have better lung function but comparable growth with age- and sex-matched peers with PCD

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Objectives: CF multidisciplinary care has been associated with dramatic improvements in lung function and nutritional status. In view of the less established service for primary ciliary dyskinesia (PCD), we compared lung function and growth of children with CF with PCD matches to assess comparative clinical outcomes.

Sample: 37 PCD patients were identified through diagnostic cilia brushing results. CF patients were matched by age and sex. The set was 59% male and mean age was 10.08 years (range 0.87 to 17.08).

Methods: Height and weight measurements were used to calculate BMI and z scores. The best lung function and any positive microbiology on sputum samples from the preceding 12 months were compared.

Results: Percent predicted FVC was significantly better in patients with CF compared with those with PCD (98.0 vs 80.7; p = 0.001). There was no significant difference in percent predicted FEV1 (82 vs 78; p = 0.42). Z scores for weight were −0.55 and −0.37 (p = 0.51), height −0.59 and −0.59 (p = 0.99) and BMI −0.15 and −0.23 (p = 0.76) respectively. In 19% of the CF group, all cultures were negative (P = 0.51), height −0.59 and −0.59 (p = 0.99) and BMI −0.15 and −0.23 (p = 0.76) respectively. In 19% of the CF group, all cultures were negative.

Conclusion: Despite normal absorption there was evidence of suboptimal nutrition in children with PCD, comparable to that of children with CF. This, together with significantly better lung function in CF, supports the inclusion of dietitians and physiotherapists in MDTs caring for patients with PCD and a centralized coordinated approach to their care.

The German multidimensional project “Fit for Life” to optimize the cystic fibrosis course in children with critical trends: first results regarding the initial 12 months period

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Objective: Life expectancy with CF increased considerably in central Europe from preschool age three decades ago to presently 39.6 years. Principle aim of the project is to stabilize children who do not appear to profit from this trend applying a multidimensional interdisciplinary program together with the centres and patients.

Methods: The German registry data resource was scanned to identify patients aged 8 to 18 years with critical courses defined as FEV1 below 80% predicted during 3 consecutive years. Interventions were supported by the German CF-foundation including the below mentioned modules and monitoring by controlling tools.

Results: Between 2005 and 2007, inclusion criteria were fulfilled by 274 children from 85 centres. 87 patients (32%) from 39 centres joined the program with durations of 12 to 36 months. First results are presented from 52 patients (mean age 14.5yrs; 61.5%) who completed the first interventional period of 12 months. In this time FEV1 could be maintained or improved in 61% and BMI-percentile in 46% of patients. Altogether, 350 measures were applied, including social work activities (28%), local physiotherapy (18%), sports (15%), nutritional issues (17%), therapy compliance (15%), empowerment and other activities (7%).

Conclusion: First experiences applying the multidimensional interdisciplinary purposive program, which exceeds the chances of the national healthcare systems, are promising. First results suggest that many patients could be stabilized. However for evaluation final data from all participants after 36 months are required and comparison to the patients who did not join the project despite fulfilling inclusion criteria will be of special interest.

Variability of clinical outcomes and impact of risk factors in cystic fibrosis (CF) patients in a regional CF center in a developing country

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Early diagnosis, specialized care are critical in improving life span in cystic fibrosis. Clinical outcomes may vary according to genotype-phenotype correlation and due to influence of risk factors as socio-economic, health-care related.

Aim: Determination of impact of RF on variability of clinical outcomes.

Methods: Review of clinical file of CF patients (0–26 years) followed up over a 12 year study period (1998–2011) in a Children's Hospital. Risk factors: poor socioeconomic status (PSS), late diagnosis (LD), growth failure at diagnosis (GF), related conditions (CFRC), lack of CF funding (LCFF), genotype (GT) were correlated with clinical outcomes.

Results: 45 patients were followed up, 10/45 had meconium ileus (MI); 15 deaths encountered (67% before 2004). Mean age at diagnosis was 1.3 years, 88% before 1 year of age. 35/45 patients were genotyped, prevalence of del F508 was 66.6%, 60% of patients had GF at diagnosis, the majority being diagnosed before the age of 6 months. In 55% of patients GF was correlated with PSS. Early CFRLD (4/45), ABPA (3/45) and surgical complications of MI (8/10) adversely impacted outcomes. Pulmonary exacerbations, more hospital days were needed in patients with PSS, LD, and GF. Lung function, nutrition were poorer in patients with LD, PSS, CFRC.

Conclusions: Phenotype expression was adversely impacted by certain RF, mostly by PSS. Outcomes were poorer before availability of CF funding and organized care. Early diagnosis could have been beneficial in most cases. Proper funding of CF Centers, staffed units, developing of educational programs for specialists and families could change the outlook for CF patients in developing countries as well.