Life expectancy of people with cystic fibrosis

A US cohort study reported that mortality rate among people with cystic fibrosis decreased by 1.8% a year between 2000 and 2010, and estimated that children born and diagnosed with cystic fibrosis in 2010 would be likely to survive to approximately 56 years if this trend were to continue.

**Overview:** Cystic fibrosis is a genetic condition in which the lungs and digestive system become clogged with thick sticky mucus (NHS Choices 2014). Symptoms usually start in early childhood and include persistent cough, recurring chest and lung infections, and poor weight gain.

People with cystic fibrosis have a reduced life expectancy relative to the general population. However, life expectancy among people with cystic fibrosis has improved over the past few decades as a result of advances in care. In 2013, the median predicted survival of people with cystic fibrosis in the UK was 36.6 years, up from 34.4 years in 2009 (Cystic Fibrosis Trust 2013). In the UK, adults with cystic fibrosis now outnumber children with this condition.

**Current advice:** The Cystic Fibrosis Trust’s Standards for the Clinical Care of Children and Adults with Cystic Fibrosis in the UK recommend that antibiotics are a key part of therapy for people with cystic fibrosis. Antibiotics should be used to manage all stages of airway infections – for prophylaxis, eradication of infection, long-term treatment of chronic infection, and treatment of acute exacerbations. NICE recommends 2 types of dry powder inhaled antibiotic – colistimethate sodium and tobramycin – for treating pseudomonas lung infection in cystic fibrosis.

The standards also recommend lifelong chest physiotherapy with airway clearance techniques and nutritional support. Other treatment options for people with cystic fibrosis include medication with inhaled mucus-clearing treatments, such as nebulised rDNAase and nebulised hypertonic saline. NICE recommends the mucus-clearing treatment mannitol dry powder for inhalation as a possible treatment for some adults with cystic fibrosis.

NICE is currently preparing a clinical guideline on cystic fibrosis, with an anticipated publication date of February 2017.

**New evidence:** A population-based cohort study in the USA by MacKenzie et al. (2014) has estimated mortality and predicted survival among people with cystic fibrosis. The study used data...
from people with a confirmed diagnosis of cystic fibrosis who were in the Cystic Fibrosis Foundation Patient Registry between 2000 and 2010. The registry tracks people with cystic fibrosis at 110 care centres, and as of 2010 included around 85% of all people with cystic fibrosis in the USA.

A total of 34,547 people were included in the registry between 2000 and 2010. Around 12% of those people were excluded from this analysis because of missing data, and 2% a year were lost to follow-up. Analyses were adjusted for gender, ethnicity, mutation of the F508del gene, presence of symptoms at diagnosis, and age at diagnosis.

The mortality rate among people with cystic fibrosis decreased by 1.8% a year between 2000 and 2010 (adjusted hazard ratio [HR]=0.982, 95% confidence interval [CI] 0.972 to 0.993), and by 17% for the whole 10-year period (HR=0.83, 95% CI 0.75 to 0.93). The adjusted risk of death was 19% lower (95% CI 13% to 24%) in males than females. Annual mortality was less than 0.5% until age 10 years, then increased steeply during adolescence and levelled out at age 25 years at 3–4% a year in females and 2–3% a year in males.

The analysis estimated that children born and diagnosed with cystic fibrosis in 2010 would survive to a median age of 39 years (95% CI 38 to 40 years), if age-specific mortality was assumed to remain indefinitely at 2010 levels. If mortality was assumed to decrease at the rate observed between 2000 and 2010, children born and diagnosed with cystic fibrosis in 2010 would be likely to survive to 56 years (95% CI 54 to 58 years).

Limitations of this analysis included that not all people with cystic fibrosis in the USA were recruited, and the projections apply only to people diagnosed in the first year of life and under the care of accredited cystic fibrosis centres. The authors add that some of the improvement in survival of people with cystic fibrosis may be related to diagnosis of more people with a milder phenotype as a result of widespread availability of genotype analysis.

Commentary: “There have been remarkable improvements in clinical outcome for patients with cystic fibrosis over the past few decades. MacKenzie et al. (2014) used data from a large US patient registry to demonstrate a steadily decreasing mortality in cystic fibrosis. They estimated that if the improvements continue at the present rate, patients born in this current decade will survive to more than 50 years.

“These improvements in survival have been achieved through a multidisciplinary model of care at specialist centres, with stepwise introduction of new treatments. New therapies continue to emerge, including for the first time drugs that target the dysfunctional cystic fibrosis transmembrane conductance regulator protein and hence address the basic underlying cause of the disease.

“The number of adults in the UK with cystic fibrosis now exceeds that of children, and continues to rise. However, there needs to be sufficient capacity at specialist adult centres to accommodate the increase in patient numbers. The findings of this study highlight the urgent need to address the capacity issues at adult cystic fibrosis centres. It is important to adequately resource multidisciplinary clinical care and new effective therapies to ensure that the achievements in improved survival continue for the future generations of people with cystic fibrosis.” – Dr Andrew M Jones, Consultant Physician and Honorary Reader, Manchester Adult Cystic Fibrosis Centre, University Hospitals South Manchester NHS Trust

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