# NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

# Guideline scope

# Cystic fibrosis: diagnosis and management of cystic fibrosis

## *Short title*

Cystic fibrosis

## *Topic*

The Department of Health in England has asked NICE to 'prepare a clinical guideline on the diagnosis and management of cystic fibrosis'.

## *Who the guideline is for*

Who should take action:

- healthcare professionals in primary care
- healthcare professionals in secondary care
- providers of cystic fibrosis services
- practitioners in cystic fibrosis.

It may also be relevant to:

- people with cystic fibrosis, families and carers and the public.

NICE guidelines cover health and care in England. Decisions on how they apply in other UK countries are made by ministers in the Welsh Government, Scottish Government, and Northern Ireland Executive.

## *Equality considerations*

NICE has carried out an equality impact assessment during scoping. The assessment:

- lists equality issues identified, and how they have been addressed

- explains why any groups are excluded from the scope, if this was done.

# 1 What the guideline is about

## *1.1 Who is the focus?*

### Groups that will be covered

- Children, young people and adults with cystic fibrosis including those who have non-classic cystic fibrosis, and those who have had an organ transplant.

## *1.2 Settings*

### Settings that will be covered

- All settings in which NHS-commissioned health and social care is provided.

## *1.3 Activities, services or aspects of care*

### Key areas that will be covered

1 The clinical manifestations of cystic fibrosis at the time of diagnosis in children, young people and adults.

2 The complications of cystic fibrosis, including their prevalence.

3 Routine monitoring of lung disease, including microbiological surveillance, radiological imaging and pulmonary function testing.

4 Antibiotic management in cystic fibrosis to:
   - prevent bacterial colonisation
   - treat acute pulmonary infection
   - treat chronic pulmonary infection, including clinical exacerbations and colonisation.

5 Management with mucoactive or mucolytic agents.

6 Chest physiotherapy.

7 Monitoring nutritional status.

8 Management of exocrine pancreatic insufficiency.

9 Management of distal intestinal obstruction syndrome (previously known as meconium ileus equivalent) in children, young people and adults.

10 Surveillance for cystic-fibrosis-related diabetes.
11 Surveillance for cystic-fibrosis-related liver disease.
12 Prevention of cystic-fibrosis-related liver disease progression.
13 Surveillance for reduced bone mineral density.
14 Recognising psychological and behavioural problems.
15 Models for delivery of care and multidisciplinary teams.

**Areas that will not be covered**

1 Specialist management of cystic-fibrosis-related diabetes.
2 Specialist management of cystic-fibrosis-related fertility problems.
3 Specialist management of cystic-fibrosis-related liver disease.
4 Specialist management of cystic-fibrosis-related bone disease.
5 Specialist management of cystic-fibrosis-related ENT disorders.
6 Specialist management of cystic-fibrosis-related renal disease.
7 Surgical management of gastrointestinal complications.
8 Transplantation.
9 Management of specific psychological conditions.
10 Management of specific behavioural problems.

## *1.4 Economic aspects*

We will take economic aspects into account when making recommendations. We will develop an economic plan that states for each review question (or key area in the scope) whether economic considerations are relevant, and if so whether this is an area that should be prioritised for economic modelling and analysis. We will review the economic evidence and carry out economic analyses, using an NHS and PSS perspective, as appropriate.

## *1.5 Key issues and questions*

While writing this scope, we have identified the following key issues, and review questions related to them:

1 Diagnosis and complications:
   - What are the clinical manifestations of cystic fibrosis at the time of diagnosis in children, young people and adults?

2 What is the age-related prevalence of the following complications of cystic fibrosis?

- Malnutrition
- Impaired growth
- Cystic fibrosis related renal disease
- Delayed puberty
- Distal intestinal obstruction syndrome (DIOS)
- Intussusception
- Volvulus
- Rectal prolapse
- Cystic fibrosis related diabetes
- Nasal polyps
- Chronic sinusitis
- Cystic fibrosis related musculoskeletal disorders,
- Urinary stress incontinence
- Reduced bone mineral density
- Pseudo-Bartter's syndrome
- Cystic fibrosis related liver disease
- Exocrine pancreatic insufficiency
- Infertility
- Psychological disorders
- Behavioural difficulties.

3 Management of pulmonary disease:

- How and when should microbiological specimens be collected?
- What is the effectiveness of the following in monitoring pulmonary disease?
  - ⇒ Microbiological investigation, including techniques such as bronchoscopy and lavage
  - ⇒ Chest X-ray
  - ⇒ Chest computed tomography (CT) scan
  - ⇒ Lung function testing, including lung clearance index and forced expiratory volume in 1 second ($FEV_1$).
- What is the effectiveness of antibiotic treatment:

- ⇒ To prevent bacterial colonisation
- ⇒ To treat acute pulmonary infection
- ⇒ To treat chronic pulmonary infection, including clinical exacerbations and colonisation?

– What is the effectiveness of mucoactive or mucolytic agents, including rhDNAse (Pulmozyme), saline (normal and hypertonic) and mannitol?

– What is the effectiveness of chest physiotherapy in people with cystic fibrosis including techniques aimed at airway clearance?

4 Gastrointestinal manifestations:

– What is the effectiveness of enzyme replacement therapy with or without gastric acid suppression in the treatment of exocrine pancreatic insufficiency?

– What are the effective strategies for prevention and treatment of distal intestinal obstruction syndrome?

– How should people with cystic fibrosis be monitored to make sure they have the best nutritional status possible?

5 Cystic fibrosis related diabetes:

– How should people with cystic fibrosis be monitored for the onset of cystic fibrosis related diabetes?

6 Liver disease:

– What is the effectiveness of ultrasound scanning to detect clinically important cystic fibrosis related liver disease?

7 What is the effectiveness of ursodeoxycholic acid for preventing liver disease progression in people with cystic fibrosis?

8 Bone disease:

– How should monitoring be carried out to identify reduced bone mineral density?

9 Psychological problems:

– How should people with cystic fibrosis be monitored for psychological or behavioural problems?

10 Delivery of care:

– What is the best set-up for multidisciplinary teams to provide care for children, young people and adults with cystic fibrosis?

- What is the most effective model for delivery of care for people with cystic fibrosis (including shared care, centre care, community care, home care and telemedicine)?
- How can services be organised to minimise the risk of cross-infection?
- What parts of the transition from children's to adult services are most important for young people with cystic fibrosis and their family members and carers?

### *1.6 Main outcomes*

The main outcomes that will be considered when searching for and assessing the evidence are:

1 Health-related quality of life.
2 Height, weight and BMI.
3 Survival rates.
4 Lung function (for example, $FEV_1$).
5 Number of acute pulmonary infections including those needing hospitalisation.
6 Prevalence of infection with specific bacterial pathogens.

## 2 Links with other NICE guidance

- Dyspepsia and gastro-oesophageal reflux disease (2014) NICE guideline 184
- Constipation in children and young people (2010) NICE guideline 99
- Depression in adults with a chronic physical health problem (2009) NICE guideline 91
- Living-donor lung transplantation for end-stage lung disease (2006) NICE interventional procedure guidance 170

### NICE guidance that may be updated or incorporated unchanged in this guideline

Depending on the outcome of a NICE technology appraisal review proposal this guideline will either; update and replace, or incorporate, the following NICE guidance:

- Colistimethate sodium and tobramycin dry powders for inhalation for treating pseudomonas lung infection in cystic fibrosis (2013) NICE technology appraisal guidance 276
- Mannitol dry powder for inhalation for treating cystic fibrosis (2012) NICE technology appraisal guidance 266

### NICE guidance about the experience of people using NHS services

NICE has produced the following guidance on the experience of people using the NHS. This guideline will not include additional recommendations on these topics unless there are specific issues related to cystic fibrosis:

- Patient experience in adult NHS services (2012) NICE guideline CG138
- Service user experience in adult mental health (2011) NICE guideline CG136
- Medicines adherence (2009) NICE guideline CG76

### NICE guidance in development that is closely related to this guideline

NICE is currently developing the following guidance that is closely related to this guideline:

- Gastro-oesophageal reflux in children and young people. NICE clinical guideline. Publication expected January 2015.
- End of life care for infants, children and young people. Publication expected 2016.
- Care of the dying adult. Publication expected 2016.

### Other related NHS guidance

- NHS Commissioning Board Clinical Commissioning Policy: Ivacaftor for Cystic Fibrosis, March 2012. Reference: NHSCB/A01/P/b.

## 2.1 Key facts and figures

2.1.1 Cystic fibrosis is a genetic disorder affecting the lungs, pancreas, liver and intestine. It can have a significant impact on life expectancy and quality of life.

2.1.2 Cystic fibrosis is associated with a reduced life expectancy. The current median age at death is 29 and the median predicted survival is 36.6 years.

2.1.3 Diagnosis is primarily made during newborn screening. The median age at diagnosis is 3 months, and 1 in every 2,500 babies born in the UK has cystic fibrosis.

2.1.4 More than 57% of people on the cystic fibrosis registry were over 16 years old (Cystic Fibrosis Trust).

2.1.5 Many different mutations are responsible for cystic fibrosis. The UK registry shows 90.8% of cases are associated with a single genotype; however 8.9% of cases have at least one unknown genotype.

2.1.6 Lung function is often reduced in cystic fibrosis. The typical measure of lung function is forced expiratory volume in 1 second ($FEV_1$). A $FEV_1$ of 50% and above will enable people to live relatively normal lives, and is associated with fewer difficulties in completing activities of daily living. A $FEV_1$ above 85% indicates normal or near-normal lung function.

2.1.7 Lung infections are a cause of significant morbidity in cystic fibrosis. Chronic infection by *Staphyloccus aureus* and *Pseudomonas aeruginosa* may need long-term use of antibiotics.

## 2.2 Current practice

2.2.1 Best practice for cystic fibrosis suggests that people with the condition benefit from a multidisciplinary team approach (MDT). Such teams include physicians or paediatricians with sufficient time

in their job plans allocated to the disease, supported by specialist nurses, dietitians, physiotherapists, pharmacists, social workers and psychologists.

2.2.2 To provide sufficient multidisciplinary team experience for the management of a complex disease, people with cystic fibrosis are grouped together into specialist centres for treatment. In these centres, outpatient care is the basis of management and patients should be monitored at least 4 times a year by the MDT, including an annual screen to assess their progress (NHS England, service specifications 2013/14, Clinical Reference Group cystic fibrosis).

2.2.3 Children with cystic fibrosis may be seen in conjunction with local paediatricians and their multidisciplinary teams (a shared-care model) for the convenience of their carers. Such shared-care arrangements are not supported in the adult sector, where everyone attends specialist centres except when population density and geography make travel a problem.

2.2.4 For inpatient care, it is considered that all people with cystic fibrosis should be admitted to single rooms with en-suite facilities on wards run by experienced cystic fibrosis staff (NHS England, service specifications 2013/14, Clinical Reference Group cystic fibrosis). Cross-infection between people with cystic fibrosis is a serious risk, and all centres and clinics should have robust protocols in place aimed at preventing it.

2.2.5 There is variation both in the multidisciplinary team structures and arrangements for providing care, and in the resources available to support services. Particular problems may arise with smaller shared care clinic arrangements. In some centres both inpatient and outpatient facilities are limited. For example, there may be problems in arranging admission to single rooms with en-suite facilities. If adequate protocols are not in place, then there is a risk of cross-infection.

2.2.6 By producing a robust evidence-based approach to defining best practice in cystic fibrosis care, this guideline will help improve healthcare for this highly complex condition.

# 3 Further information

This is the draft scope for consultation with registered stakeholders. The consultation dates are 20 November to 18 December 2014.

The guideline is expected to be published in February 2017.

You can follow progress of the guideline.

Our website has information about how NICE guidelines are developed.