

# Cystic fibrosis: diagnosis and management

## NICE guideline: short version

### Draft for consultation, May 2017

**This guideline covers** the diagnosis and management of cystic fibrosis. It specifies how to monitor the condition and how to manage the symptoms, to improve the quality of life for people with cystic fibrosis. There are also recommendations about treating the most common infections in people with cystic fibrosis, preventing cross infection, service organisation, and information and support.

#### Who is it for?

- Healthcare professionals (primary and secondary care)
- Social care practitioners working with people with cystic fibrosis
- People with cystic fibrosis, families and carers, and the public

This version of the guideline contains the recommendations, context and recommendations for research. Information about how the guideline was developed is on the [guideline's page](#) on the NICE website. This includes the Committee's discussion and the evidence reviews (in the [full guideline](#)), the scope, and details of the Committee and any declarations of interest.

1 **Contents**

2 Recommendations ..... 3

3 1.1 Diagnosis of cystic fibrosis ..... 3

4 1.2 Information and support ..... 4

5 1.3 Service delivery ..... 6

6 1.4 Complications of cystic fibrosis ..... 10

7 1.5 Annual and routine reviews ..... 10

8 1.6 Pulmonary monitoring, assessment and management ..... 11

9 1.7 Other monitoring, assessment and management..... 23

10 1.8 Preventing cross-infection ..... 28

11 Terms used in this guideline ..... 29

12 Putting this guideline into practice ..... 30

13 Context..... 32

14 Recommendations for research ..... 33

15

16

## 1 Recommendations

People have the right to be involved in discussions and make informed decisions about their care, as described in [your care](#).

[Making decisions using NICE guidelines](#) explains how we use words to show the strength (or certainty) of our recommendations, and has information about prescribing medicines (including off-label use), professional guidelines, standards and laws (including on consent and mental capacity), and safeguarding.

2

3 This guideline incorporates recommendations from 2 NICE technology appraisals:

- 4 • [Mannitol dry powder for inhalation for treating cystic fibrosis](#) (NICE technology  
5 appraisal 266)
- 6 • [Colistimethate sodium and tobramycin dry powders for inhalation for treating  
7 \*Pseudomonas\* lung infection in cystic fibrosis](#) (NICE technology appraisal 276)

8 These technology appraisals still apply, and have not been replaced by the  
9 guideline.

### 10 **1.1 *Diagnosis of cystic fibrosis***

11 1.1.1 Be aware that cystic fibrosis can be diagnosed based on:

- 12 • clinical manifestations, supported by sweat or gene test results for  
13 confirmation **or**
- 14 • clinical manifestations alone, for people with symptoms who have  
15 normal sweat or gene test results **or**
- 16 • positive test results in people with no symptoms, for example infant  
17 screening (blood spot immunoreactive trypsin test) followed by sweat  
18 and gene tests for confirmation.

19 1.1.2 Assess for cystic fibrosis and, when clinically appropriate, perform a sweat  
20 test (for children and [young people](#)) or a cystic fibrosis gene test (for  
21 adults) in people with any of the following:

- 1 • congenital intestinal atresia
- 2 • meconium ileus
- 3 • symptoms and signs that suggest distal intestinal obstruction syndrome
- 4 • faltering growth (in infants and young children)
- 5 • undernutrition
- 6 • recurrent and chronic pulmonary disease, such as:
  - 7 – recurrent lower respiratory tract infections
  - 8 – clinical or radiological lung disease (in particular bronchiectasis)
  - 9 – persistent chest X-ray changes
  - 10 – chronic wet or productive cough
- 11 • chronic sinus disease
- 12 • obstructive azoospermia (in young people and adults)
- 13 • acute or chronic pancreatitis
- 14 • malabsorption
- 15 • rectal prolapse (in children)
- 16 • pseudo-Bartter syndrome.

17 1.1.3 Refer people with suspected cystic fibrosis to a specialist cystic fibrosis  
18 centre if:

- 19 • they have a positive or equivocal sweat test result
- 20 • their assessment suggests they have cystic fibrosis but their test results  
21 are normal
- 22 • gene testing reveals 1 or 2 cystic fibrosis mutations.

## 23 **1.2 Information and support**

24 1.2.1 Provide people who are newly diagnosed with cystic fibrosis and their  
25 family members or carers (as appropriate) with opportunities to discuss  
26 their concerns.

27 1.2.2 Information and support should be provided by healthcare professionals  
28 with expertise in cystic fibrosis.

- 1           1.2.3       Provide people with suspected or diagnosed cystic fibrosis and their  
2                   family members or carers (as appropriate) with relevant information in  
3                   clear English and opportunities for discussion on topics such as:
- 4                   • their diagnosis
  - 5                   • monitoring of their condition
  - 6                   • management choices for their condition
  - 7                   • possible or existing complications or comorbidities
  - 8                   • implications for independent living.
- 9           1.2.4       Provide people with cystic fibrosis and their family members or carers (as  
10                   appropriate) with information about their care pathway (for example, by  
11                   providing them with copies of correspondence).
- 12           1.2.5       Give information to people with cystic fibrosis and to family members or  
13                   carers in ways that are individually appropriate. Avoid jargon and use  
14                   formats that they prefer, for example:
- 15                   • face-to-face discussions
  - 16                   • written information (such as leaflets)
  - 17                   • any digital media and reliable internet sources that are available.
- 18           1.2.6       When appropriate, provide people with cystic fibrosis and their family  
19                   members or carers opportunities for discussion with relevant expert  
20                   professionals on:
- 21                   • available resources and support, such as local support and advocacy  
22                    groups, including those aimed specifically at children, young people,  
23                    adults, parents and carers
  - 24                   • implications of the condition for school and education
  - 25                   • career planning
  - 26                   • transition to adult care
  - 27                   • fertility and contraception
  - 28                   • pregnancy and parenting
  - 29                   • organ transplantation.

- 1 1.2.7 Provide people with cystic fibrosis with information about how to contact  
2 other people with cystic fibrosis without risking cross-infection (see  
3 [Preventing cross-infection](#)), for example, by directing them to online  
4 support groups.
- 5 1.2.8 For more information on communication, providing information and shared  
6 decision-making in adult NHS services, see the NICE guideline on [patient  
7 experience in adult NHS services](#).
- 8 1.2.9 Be aware that people with cystic fibrosis and their family members or  
9 carers may need emotional and psychological support (see [Psychological  
10 assessment](#)), in particular:
- 11 • at diagnosis
  - 12 • at times of transition (for example, when moving from education to work  
13 or when living independently for the first time)
  - 14 • during pregnancy
  - 15 • to deal with complications, such as infertility
  - 16 • when approaching the end of life.

## 17 **1.3 Service delivery**

### 18 **Service configuration**

- 19 1.3.1 Care for people with cystic fibrosis should be provided by a specialist  
20 cystic fibrosis multidisciplinary team based at a specialist cystic fibrosis  
21 centre (see [Multidisciplinary teams](#)).
- 22 1.3.2 Specialist cystic fibrosis centres should:
- 23 • plan patient care (including outpatient and inpatient care), taking into  
24 account the risk of cross-infection (see [Preventing cross-infection](#))
  - 25 • maintain local and national registers of patients that include information  
26 about their clinical condition, treatment and outcomes
  - 27 • audit practice and outcomes.

- 1 1.3.3 When a shared-care model is used for children and young people, it  
2 should include:
- 3 • formal arrangements between the local paediatric team at the shared-  
4 care centre and multidisciplinary team at the specialist cystic fibrosis  
5 centre
  - 6 • direct involvement of specialist cystic fibrosis multidisciplinary team  
7 members
  - 8 • an annual assessment and at least one other review per year by the  
9 specialist cystic fibrosis multidisciplinary team in addition to reviews by  
10 the local paediatric team (see Annual and routine reviews).
- 11 1.3.4 If available and when clinically appropriate, outreach care for adults with  
12 cystic fibrosis may be provided by the specialist cystic fibrosis  
13 multidisciplinary team at a local hospital.
- 14 1.3.5 The specialist cystic fibrosis multidisciplinary team should have a member  
15 available for people with cystic fibrosis and their family members or carers  
16 (as appropriate) to contact when they have urgent enquiries at any time  
17 (day and night).
- 18 1.3.6 Consider telemedicine or home visits for routine monitoring where they  
19 are more appropriate than outpatient visits and if the person with cystic  
20 fibrosis prefers it.
- 21 1.3.7 Make arrangements (including providing equipment and expert support)  
22 for people to have intravenous antibiotic therapy at home, when this is  
23 appropriate.

24 **Multidisciplinary team**

- 25 1.3.8 The specialist cystic fibrosis multidisciplinary team should include the  
26 following professionals who have specialist expertise in the condition:
- 27 • specialist paediatrician or adult physician
  - 28 • specialist nurse
  - 29 • specialist physiotherapist

- 1                   • specialist dietitian  
2                   • specialist pharmacist  
3                   • specialist clinical psychologist  
4                   • social worker.
- 5           1.3.9       The specialist cystic fibrosis multidisciplinary team should be led by a  
6                   specialist paediatrician or adult physician.
- 7           1.3.10       Specialist nurses should coordinate care, coordinate communication  
8                   between other members of the team and act as advocates for people with  
9                   cystic fibrosis and their family members or carers (as appropriate).
- 10          1.3.11       The specialist physiotherapist should assess and advise people with  
11                   cystic fibrosis at clinic, at inpatient admissions, during pulmonary  
12                   exacerbations and at their annual review on:
- 13                   • airway clearance  
14                   • nebuliser use  
15                   • musculoskeletal disorders  
16                   • exercise and physical activity  
17                   • urinary incontinence.
- 18          1.3.12       The specialist dietitian should assess and advise people with cystic  
19                   fibrosis about nutrition at outpatient clinic visits, during inpatient  
20                   admissions and at their annual review.
- 21          1.3.13       The specialist pharmacist should advise people with cystic fibrosis on  
22                   medicines optimisation at outpatient clinic visits and at their annual  
23                   review.
- 24          1.3.14       The specialist clinical psychologist should assess and advise people with  
25                   cystic fibrosis at their annual review and when needed at outpatient and  
26                   inpatient clinics.
- 27          1.3.15       The specialist cystic fibrosis multidisciplinary team should either include or  
28                   have access to specialist expertise relevant to cystic fibrosis in the  
29                   following areas:



- 1 • microbiology
- 2 • pulmonary physiology
- 3 • diabetes
- 4 • gastroenterology
- 5 • hepatology
- 6 • rheumatology
- 7 • psychiatry
- 8 • interventional radiology
- 9 • surgery (gastrointestinal and thoracic)
- 10 • obstetrics
- 11 • palliative care.

## 12 **Transition to adult services**

13 1.3.16 Begin the transition process to adult services with young people with  
14 cystic fibrosis when they are 12 years old, and with their family members  
15 or carers (as appropriate).

16 1.3.17 Ensure that young people with cystic fibrosis move to adults' services  
17 between the ages of 16 and 18.

18 1.3.18 All cystic fibrosis services should have a coordinated and documented  
19 pathway for transition from children's to adults' services that includes  
20 plans for managing all cystic-fibrosis-related aspects of care.

21 1.3.19 Provide a named worker to lead the transition for each young person with  
22 cystic fibrosis who is moving to adults' services, and if possible ensure  
23 that the named worker is someone the young person already knows. For  
24 more guidance on named workers see the section on [named workers](#) in  
25 the NICE guideline on transition for young people using health or social  
26 care services.

27 1.3.20 The specialist cystic fibrosis centre should take responsibility for ensuring  
28 transitions are successful.

29 1.3.21 Ask people with cystic fibrosis and their family members or carers (as  
30 appropriate) for feedback on the quality of the transition service.

1 1.3.22 For more guidance on managing transition from children's to adults'  
2 services, see the NICE guideline on [transition for young people using](#)  
3 [health or social care services](#).

#### 4 **1.4 Complications of cystic fibrosis**

5 1.4.1 Be aware that people with cystic fibrosis are at risk of the following  
6 common complications:

- 7 • being underweight
- 8 • meconium ileus (affects 1 in 7 newborn babies)
- 9 • fat-soluble vitamin deficiencies (including vitamins A, D, E and K)
- 10 • distal ileal obstruction syndrome
- 11 • muscle pains and arthralgia
- 12 • male infertility (almost all males with cystic fibrosis are infertile)
- 13 • reduced female fertility
- 14 • upper airway complications, including nasal polyps and sinusitis  
15 (prevalence increases with age)
- 16 • chronic liver disease (the prevalence increases with age until early  
17 adulthood)
- 18 • urinary stress incontinence
- 19 • cystic fibrosis-related diabetes (uncommon in children under 10 years,  
20 but the prevalence increases with age and it affects up to 1 in 2 adults)
- 21 • reduced bone mineral density and osteoporosis.

22 1.4.2 Be aware that people with cystic fibrosis are at risk of the following less  
23 common complications:

- 24 • cystic fibrosis-related arthritis
- 25 • delayed puberty (associated with severe cystic fibrosis)
- 26 • renal calculi (incidence increases with age and 1 in 20 adults are  
27 affected).

#### 28 **1.5 Annual and routine reviews**

29 1.5.1 Be aware that:

- the aim of cystic fibrosis care is to prevent or limit symptoms and complications of the condition
- routine monitoring and annual assessments are crucial in providing effective care.

1.5.2 Offer people with cystic fibrosis a comprehensive annual review that includes the following:

- a pulmonary assessment (see [Pulmonary monitoring](#))
- an assessment of nutrition and intestinal absorption (see [Nutritional interventions and exocrine pancreatic insufficiency](#) 1.7.4)
- an assessment for liver disease (see [Liver disease](#))
- testing for cystic fibrosis-related diabetes, from 10 years of age (see [Cystic-fibrosis-related diabetes](#))
- an assessment for other potential or existing cystic fibrosis complications (see [Complications of cystic fibrosis](#))
- assessments by a specialist physiotherapist, dietician, pharmacist and clinical psychologist (see [Service delivery](#))
- a review of the exercise programme (see [Exercise](#)).

1.5.3 Provide regular routine reviews for people with cystic fibrosis, and do these more frequently immediately after diagnosis and in early life. For example:

- weekly in their first month of life
- every 4 weeks when they are between 1 and 12 months old
- every 6 or 8 weeks when they are between 1 and 5 years old
- every 8 or 12 weeks when they are over 5 years old
- every 3 or 6 months as adults.

## **1.6 Pulmonary monitoring, assessment and management**

### **Pulmonary monitoring**

1.6.1 For people with cystic fibrosis who have lung disease, base the frequency of routine reviews on their clinical condition but review children and young

1 people at least every 8 weeks and adults at least every 3 months. If  
2 appropriate, think about using the review schedules in recommendation  
3 1.5.3.

4 1.6.2 Include the following at each routine review, in relation to pulmonary  
5 assessment, for people with cystic fibrosis:

- 6 • a clinical assessment, including a review of the clinical history and a  
7 physical examination, with measurement of weight and length (if less  
8 than 2 years old) or height
- 9 • measurement of oxygen saturation
- 10 • taking respiratory secretion samples for microbiological investigations,  
11 using sputum samples if possible, or a cough swab or nasal pharyngeal  
12 aspirate (NPA) if not
- 13 • lung function testing with spirometry (including forced expiratory volume  
14 in 1 second [FEV1], forced vital capacity [FVC], and forced expiratory  
15 flow [FEF] 25–75%) in adults, and in children and young people who  
16 can do this.

17 1.6.3 Consider measuring lung clearance index at each routine review if  
18 spirometry is normal.

19 1.6.4 Include the following at each annual review in relation to pulmonary  
20 assessment for people with cystic fibrosis:

- 21 • a clinical assessment, including a review of the clinical history and a  
22 physical examination, with measurement of weight and length (if less  
23 than 2 years old) or height
- 24 • a physiotherapy assessment
- 25 • measurement of oxygen saturation
- 26 • a chest X-ray
- 27 • blood tests, including white cell count, aspergillus serology and serum  
28 IgE
- 29 • taking respiratory secretion samples for microbiological investigations  
30 (including mycobacteria)

- lung function testing (for example with spirometry, including FEV1, FVC, and FEF 25–75%) in adults, and in children and young people who can do this.

1.6.5 Consider measuring lung clearance index at each annual review if spirometry is normal.

1.6.6 For people with cystic fibrosis with lung disease who have symptoms that are concerning them, their family members or carers (as appropriate) or a healthcare professional, decide whether a remote telemedicine (see [telemedicine](#)) or face-to-face assessment is needed and consider which of the following may be useful:

- review of the past history
- physical examination, including measurement of weight and length (if less than 2 years old) or height
- measurement of oxygen saturation
- collection of respiratory secretion samples for microbiological investigations, using sputum samples if possible, or a cough swab or NPA if not
- for adults, blood tests to measure white cell count and inflammatory markers such as C-reactive protein
- lung function testing, for example with spirometry (including FEV1, FVC, and FEF 25–75%) in adults, and in children and young people who can do this
- lung clearance index for people with normal spirometry results.

1.6.7 Think about doing a chest CT scan for children with cystic fibrosis who have not had one before, to detect features that other tests would miss (for example early bronchiectasis).

1.6.8 Think about doing a chest X-ray for people with cystic fibrosis who have had treatment for an exacerbation of lung disease (taking account of severity), if:

- the exacerbation does not respond to treatment **or**

- 1                   • a chest X-ray before treatment showed new radiological abnormalities.

2           1.6.9       Monitor the treatment response during and after an exacerbation of lung  
3                   disease by assessing whether the symptoms and signs have resolved,  
4                   and as appropriate:

- 5                   • take respiratory secretion samples for microbiological investigations,  
6                    using sputum samples if possible, or a cough swab or NPA if not  
7                   • test lung function, for example with spirometry (including FEV1, FVC  
8                    and FEF 25–75%) in adults, and in children and young people who can  
9                    do this  
10                  • measure oxygen saturation.

11          1.6.10     Think about using broncho-alveolar lavage to obtain airway samples for  
12                   microbiological investigation in children and young people if:

- 13                  • they have lung disease that has not responded adequately to treatment  
14                    **and**  
15                  • the cause of the disease cannot be found with non-invasive upper  
16                    airway respiratory secretion sampling.

17           **Airway clearance techniques**

18          1.6.11     Discuss the use of airway clearance techniques with people with cystic  
19                   fibrosis who do not have lung disease and with their parents or carers (as  
20                   appropriate).

21          1.6.12     Offer training in an airway clearance technique to people with cystic  
22                   fibrosis who have lung disease and their parents or carers (as  
23                   appropriate).

24          1.6.13     When choosing an airway clearance technique for people with cystic  
25                   fibrosis:

- 26                  • assess their ability to clear mucus from their lungs, and offer an  
27                    individualised plan to optimise this

- 1                   • take account of the preferences of the person and (if appropriate) their
- 2                    parents and carers
- 3                   • take account of any factors that may influence adherence.

4           1.6.14    Regularly assess the effectiveness of airway clearance techniques, and

5                    modify the technique or use a different one if needed.

6           1.6.15    Do not offer high-frequency chest wall oscillation as an airway clearance

7                    technique for people with cystic fibrosis.

8           1.6.16    Think about using non-invasive ventilation to help with airway clearance

9                    techniques in people who have moderate or severe lung disease.

## 10           **Mucoactive agents**

11           1.6.17    Offer a mucoactive agent to people with cystic fibrosis who have

12                    respiratory symptoms, or other evidence of lung disease.

13           1.6.18    Offer rhDNase (dornase alfa; recombinant human deoxyribonuclease) as

14                    the first choice of mucoactive agent.

15           1.6.19    If clinical evaluation or lung function testing indicates an inadequate

16                    response to rhDNase, consider both rhDNase and hypertonic sodium

17                    chloride or hypertonic sodium chloride alone.

18           1.6.20    Mannitol dry powder for inhalation is recommended as an option for

19                    treating cystic fibrosis in adults:

- 20                   • who cannot use rhDNase because of ineligibility, intolerance or
- 21                    inadequate response to rhDNase **and**
- 22                   • whose lung function is rapidly declining (forced expiratory volume in 1
- 23                    second [FEV1] decline greater than 2% annually) **and**
- 24                   • for whom other osmotic agents are not considered appropriate.

25                    [This recommendation is from [Mannitol dry powder for inhalation for](#)

26                    [treating cystic fibrosis](#) (NICE technology appraisal 266).]

1 1.6.21 People currently receiving mannitol whose cystic fibrosis does not meet  
2 the criteria in 1.6.20 should be able to continue treatment until they and  
3 their clinician consider it appropriate to stop.

4 [This recommendation is from [Mannitol dry powder for inhalation for](#)  
5 [treating cystic fibrosis](#) (NICE technology appraisal 266).]

## 6 **Pulmonary infection**

### 7 ***Staphylococcus Aureus***

8 1.6.22 Offer flucloxacillin as antibiotic prophylaxis against respiratory  
9 staphylococcus aureus infection for children with cystic fibrosis from the  
10 point of diagnosis, and consider continuing up to 6 years of age. Before  
11 starting flucloxacillin, discuss the uncertainties and possible adverse  
12 effects with their parents or carers (as appropriate).

13 1.6.23 For children who are taking antibiotic prophylaxis and have a respiratory  
14 sample culture that is positive for staphylococcus aureus:

- 15 • review prophylaxis adherence and help the child's parents or carers (as  
16 appropriate) with any difficulties they are having
- 17 • start eradication therapy with antibiotics
- 18 • restart prophylaxis after treatment, even if eradication has not been  
19 successful.

20 1.6.24 For people who are not taking prophylaxis and have a new  
21 staphylococcus aureus infection (that is, previous respiratory sample  
22 cultures did not show staphylococcus aureus infection):

- 23 • if they are clinically well, consider an oral anti-staphylococcus aureus  
24 agent
- 25 • if they are clinically unwell and have pulmonary disease, consider oral  
26 or intravenous (depending on infection severity) broad-spectrum  
27 antibiotics that include an oral anti-staphylococcus aureus agent.



- 1 1.6.25 Consider a long-term antibiotic to suppress chronic methicillin-sensitive  
2 staphylococcus aureus (MSSA) respiratory infection in people whose  
3 pulmonary disease is stable.
- 4 1.6.26 For people with chronic MSSA respiratory infection who become clinically  
5 unwell with pulmonary disease, consider oral or intravenous broad-  
6 spectrum antibiotics (depending on infection severity) that include an anti-  
7 staphylococcus aureus agent.
- 8 1.6.27 Do not routinely use antibiotics to suppress methicillin-resistant  
9 staphylococcus aureus (MRSA) in people with stable pulmonary disease.
- 10 1.6.28 If a person with cystic fibrosis and chronic MRSA respiratory infection  
11 becomes unwell with a pulmonary exacerbation or shows a decline in  
12 pulmonary function, seek specialist microbiological advice.
- 13 1.6.29 For guidance on preventing the spread of infection, refer to the NICE  
14 guideline on [healthcare-associated infections](#).

15 ***Pseudomonas aeruginosa***

- 16 1.6.30 If a person with cystic fibrosis develops a new pseudomonas aeruginosa  
17 infection (that is, recent respiratory secretion sample cultures showed no  
18 infection):
- 19 • if they are clinically well:
    - 20 – commence eradication therapy with a course of oral or intravenous
    - 21 antibiotics, together with an inhaled antibiotic
    - 22 – follow this with an extended course of oral and inhaled antibiotics
  - 23 • if they are clinically unwell:
    - 24 – commence eradication therapy with a course of intravenous
    - 25 antibiotics together with an inhaled antibiotic
    - 26 – follow this with an extended course of oral and inhaled antibiotics.
- 27 1.6.31 If eradication treatment is not successful despite treatment as  
28 recommended in 1.6.30, offer sustained treatment with an inhaled  
29 antibiotic. Consider nebulised colistimethate sodium as first-line treatment.

- 1 (See recommendation 1.6.34 on using colistimethate dry powder for  
2 inhalation)
- 3 1.6.32 Depending on infection severity, use either an oral antibiotic or a  
4 combination of 2 intravenous antibiotics of different classes for people:
- 5 • who have chronic pseudomonas aeruginosa infection (when treatment  
6 has not eradicated the infection) **and**
  - 7 • who become clinically unwell with a pulmonary disease exacerbation.
- 8 1.6.33 If a person with chronic pseudomonas aeruginosa infection repeatedly  
9 becomes clinically unwell with pulmonary disease exacerbations, consider  
10 changing the antibiotic regimens used to treat exacerbations.
- 11 1.6.34 Colistimethate sodium dry powder for inhalation (DPI) is recommended as  
12 an option for treating chronic pulmonary infection caused by  
13 pseudomonas aeruginosa in people with cystic fibrosis only if:
- 14 • they would clinically benefit from continued colistimethate sodium but  
15 do not tolerate it in its nebulised form and thus tobramycin therapy  
16 would otherwise be considered **and**
  - 17 • the manufacturer provides colistimethate sodium DPI with the discount  
18 agreed as part of the patient access scheme to primary, secondary and  
19 tertiary care in the NHS.
- 20 [This recommendation is from [Colistimethate sodium and tobramycin dry](#)  
21 [powders for inhalation for treating pseudomonas lung infection in cystic](#)  
22 [fibrosis](#) (NICE technology appraisal 276)]
- 23 1.6.35 For people who are clinically deteriorating despite regular inhaled  
24 colistimethate sodium, consider nebulised aztreonam, nebulised  
25 tobramycin, or tobramycin DPI (see recommendation 1.6.36 on using  
26 [tobramycin DPI](#)).
- 27 1.6.36 Tobramycin DPI is recommended as an option for treating chronic  
28 pulmonary infection caused by pseudomonas aeruginosa in people with  
29 cystic fibrosis only if:

- 1 • nebulised tobramycin is considered an appropriate treatment, that is,  
2 when colistimethate sodium is contraindicated, is not tolerated or has  
3 not produced an adequate clinical response **and**
- 4 • the manufacturer provides tobramycin DPI with the discount agreed as  
5 part of the patient access scheme to primary, secondary and tertiary  
6 care in the NHS.

7 [This recommendation is from [Colistimethate sodium and tobramycin dry](#)  
8 [powders for inhalation for treating pseudomonas lung infection in cystic](#)  
9 [fibrosis](#) (NICE technology appraisal 276)]

10 1.6.37 People currently using tobramycin DPI or colistimethate sodium DPI that  
11 is not recommended according to recommendations 1.6.36 1.6.34 or  
12 1.6.36 should be able to continue treatment until they and their clinician  
13 consider it appropriate to stop. For children and young people this  
14 decision should be made jointly by the clinician, the child or young person  
15 and their parents or carers.

16 [This recommendation is from [Colistimethate sodium and tobramycin dry](#)  
17 [powders for inhalation for treating pseudomonas lung infection in cystic](#)  
18 [fibrosis](#) (NICE technology appraisal 276)]

### 19 ***Burkholderia cepacia complex***

20 1.6.38 For people with cystic fibrosis who develop a new Burkholderia cepacia  
21 complex infection (that is, recent respiratory sample cultures showed no  
22 Burkholderia cepacia infection):

- 23 • whether they are clinically well or not, give antibiotic eradication therapy  
24 using a combination of intravenous antibiotics
- 25 • seek specialist microbiological advice on the choice of antibiotics to  
26 use.

27 1.6.39 Do not use antibiotics to suppress chronic Burkholderia cepacia complex  
28 infection in people with cystic fibrosis who have stable pulmonary status.

1 1.6.40 For people with cystic fibrosis who have chronic *Burkholderia cepacia*  
2 complex infection (when treatment has not eradicated the infection) and  
3 who become clinically unwell with a pulmonary disease exacerbation:

- 4 • give a combination of oral or intravenous antibiotics
- 5 • seek specialist microbiological advice on which antibiotics to use.

6 1.6.41 For people with cystic fibrosis who have chronic *Burkholderia cepacia*  
7 complex infection and declining pulmonary status:

- 8 • consider sustained treatment with an inhaled antibiotic to suppress the  
9 infection
- 10 • seek specialist microbiological advice on which antibiotic to use
- 11 • stop this treatment if there is no observed benefit.

## 12 ***Haemophilus influenzae***

13 1.6.42 For people with cystic fibrosis who develop a *haemophilus influenzae*  
14 infection (diagnosed by a positive respiratory sample culture) but do not  
15 have clinical evidence of pulmonary infection, treat with an appropriate  
16 oral antibiotic.

17 1.6.43 For people with cystic fibrosis who develop a *haemophilus influenzae*  
18 infection (diagnosed by a positive respiratory sample culture) and are  
19 unwell with clinical evidence of pulmonary infection, treat with an  
20 appropriate antibiotic, given orally or intravenously depending on the  
21 severity of the illness.

## 22 ***Non-tuberculous mycobacteria***

23 1.6.44 For people with cystic fibrosis who are clinically well but whose airway  
24 secretions are persistently positive for non-tuberculous mycobacteria,  
25 discuss with them and their family members or carers (as appropriate):

- 26 • the clinical uncertainties about non-tuberculous mycobacterial infection  
27 **and**
- 28 • the possible benefits and risks (for example, drug toxicity) of treating it.

- 1 1.6.45 If a person with cystic fibrosis has a respiratory sample test positive for  
2 new non-tuberculous mycobacteria infection, repeat the test for  
3 confirmation.
- 4 1.6.46 If repeat testing confirms persistent non-tuberculous mycobacteria, do a  
5 chest CT scan to look for changes consistent with non-tuberculous  
6 mycobacteria disease.
- 7 1.6.47 Consider non-tuberculous mycobacterial eradication therapy in people  
8 with cystic fibrosis:
- 9 • whose airway secretions persistently test positive for non-tuberculous  
10 mycobacteria **and**
- 11 • who are clinically unwell with respiratory disease, or who have a chest  
12 CT scan showing changes consistent with non-tuberculous  
13 mycobacteria disease **and**
- 14 • whose pulmonary disease has not responded to other recommended  
15 treatments.
- 16 1.6.48 Consider the following eradication regimen for people with cystic fibrosis  
17 who are having treatment for non-tuberculous mycobacteria:
- 18 • Initial treatment: a combination of appropriate oral and intravenous  
19 antibiotics. Seek specialist microbiological advice on which antibiotics  
20 to use.
- 21 • Continued treatment: prolonged oral and inhaled antibiotics. Seek  
22 specialist microbiological advice on which antibiotics to use.
- 23 ***Aspergillus fumigatus complex***
- 24 1.6.49 Do not routinely use antifungal agents to suppress chronic aspergillus  
25 fumigatus complex respiratory infection (diagnosed by persistently  
26 positive respiratory secretion sample cultures) in people with cystic  
27 fibrosis and stable pulmonary status.
- 28 1.6.50 For people with cystic fibrosis with chronic aspergillus fumigatus complex  
29 respiratory infection and declining pulmonary status:

- 1 • consider sustained treatment with an antifungal agent to suppress the
- 2 infection
- 3 • seek specialist microbiological advice on which antifungal agent to use
- 4 • stop treatment or change to a different agent if there is no benefit.

5 1.6.51 For people with cystic fibrosis with elevated aspergillus serology and  
6 declining pulmonary function despite optimised pulmonary treatment, think  
7 about treating for a possible diagnosis of allergic bronchopulmonary  
8 aspergillosis, especially if there are consistent chest X-ray or CT scan  
9 changes.

### 10 ***Unidentified Infections***

11 1.6.52 For people with cystic fibrosis who have a pulmonary disease  
12 exacerbation and no clear cause in their respiratory secretion samples:

- 13 • use an oral or intravenous (depending on the exacerbation severity)
- 14 broad-spectrum antibiotic
- 15 • continue collecting respiratory secretion samples, and change
- 16 treatments if a pathogen is identified and a more appropriate treatment
- 17 is available.

### 18 **Immunomodulatory agents**

19 1.6.53 For people with cystic fibrosis and deteriorating lung function or repeated  
20 pulmonary exacerbations, offer long-term treatment with azithromycin at  
21 an immunomodulatory dose<sup>1</sup>.

22 1.6.54 For people who have continued deterioration in lung function, or  
23 continuing pulmonary exacerbations while receiving long-term treatment  
24 with azithromycin, stop azithromycin and consider oral corticosteroids at  
25 an immunomodulatory dose.

26 1.6.55 Do not offer inhaled corticosteroids as an immunomodulatory treatment  
27 for people with cystic fibrosis.

---

<sup>1</sup> At the time of publication (October 2017), azithromycin did not have a UK marketing authorisation for this indication. The prescriber should follow relevant professional guidance, taking full responsibility for the decision. Informed consent should be obtained and documented. See the General Medical Council's [Prescribing guidance: prescribing unlicensed medicines for further information](#).

## 1.7 *Other monitoring, assessment and management*

### **Nutritional interventions and exocrine pancreatic insufficiency**

1.7.1 The cystic fibrosis specialist dietitian should offer advice on the benefits of optimal nutrition, and at the annual assessment review the person's:

- total nutritional intake, including energy intake (calories)
- estimated nutritional needs
- pancreatic exocrine replacement therapy, if appropriate (see [Exocrine pancreatic insufficiency](#))

1.7.2 Encourage people to increase calorie intake by increasing portion size and eating high-energy foods if there is concern about their nutrition (including weight loss and inadequate weight gain).

1.7.3 If increased portion size and high-energy foods are not effective, consider a trial of oral nutritional supplements.

1.7.4 If attempts to increase calorie intake are not effective, consider:

- supplementation with enteral tube feeding **or**
- for adults, a short-term trial of an appetite stimulant.

1.7.5 Test for exocrine pancreatic insufficiency in people with cystic fibrosis, using a non-invasive technique such as stool elastase estimation. If the test result is normal, repeat it:

- annually in children and young people
- if symptoms or signs suggesting malabsorption occur.

1.7.6 Offer oral pancreatic enzyme replacement therapy to people with exocrine pancreatic insufficiency. Adjust the dose as needed to minimise any symptoms or signs of malabsorption.

1.7.7 Consider an acid suppression agent (for example an H2 receptor antagonist or a proton pump inhibitor) for people who have persistent

1 symptoms or signs of malabsorption despite optimal pancreatic enzyme  
2 replacement therapy.

### 3 **Distal ileal obstruction syndrome**

4 1.7.8 Be aware that a variety of conditions can cause acute abdominal pain and  
5 resemble distal ileal obstruction syndrome in people with cystic fibrosis,  
6 for example:

- 7 • constipation
- 8 • appendicitis
- 9 • intussusception
- 10 • cholecystitis.

11 1.7.9 Suspect distal ileal obstruction syndrome in people with cystic fibrosis who  
12 have an acute onset of peri-umbilical or right lower quadrant abdominal  
13 pain and any of the following:

- 14 • a palpable mass in the right lower quadrant
- 15 • faecal loading in the right lower quadrant on a plain abdominal  
16 radiograph, especially if associated with small intestine air-fluid levels
- 17 • clinical features of partial or complete intestinal obstruction, such as  
18 vomiting (especially bilious) and abdominal distension.

19 1.7.10 For people who have an acute onset of peri-umbilical abdominal pain but  
20 no other clinical or radiological features of distal ileal obstruction  
21 syndrome, consider further investigation with an:

- 22 • abdominal ultrasound scan **or**
- 23 • abdominal CT scan.

24 1.7.11 Manage suspected distal ileal obstruction syndrome in a specialist cystic  
25 fibrosis centre, with supervision from specialists who have expertise in  
26 recognising and treating the condition and its complications.

27 1.7.12 Offer oral or intravenous fluids to ensure adequate hydration (and  
28 rehydration if needed) for people with distal ileal obstruction syndrome.



- 1 1.7.13 Consider diatrizoate meglumine and diatrizoate sodium solution (orally or  
2 via an enteral tube) as first-line treatment for distal ileal obstruction  
3 syndrome.
- 4 1.7.14 If diatrizoate meglumine and diatrizoate sodium solution is not effective,  
5 consider an iso-osmotic polyethylene glycol and electrolyte (PEG) solution  
6 (orally or via an enteral tube) as a second-line treatment.
- 7 1.7.15 Consider surgery as a last resort, if prolonged treatment with a PEG  
8 solution is not effective.
- 9 1.7.16 To reduce the risk of distal ileal obstruction syndrome recurring:
- 10 • encourage people to drink plenty of fluids
- 11 • optimise pancreatic enzyme replacement therapy (see [Exocrine](#)  
12 [pancreatic insufficiency](#)).
- 13 • consider advising regular treatment with a stool-softening agent such  
14 as lactulose or a PEG solution.

15 **Liver disease**

- 16 1.7.17 Perform a clinical assessment and liver function blood tests at the annual  
17 review for people with cystic fibrosis.
- 18 1.7.18 If liver function blood tests are abnormal, perform a liver ultrasound scan  
19 and consider ursodeoxycholic acid treatment.
- 20 1.7.19 Think about stopping ursodeoxycholic acid if liver function blood tests  
21 return to normal and clinical assessment and liver ultrasound scan show  
22 no liver disease.
- 23 1.7.20 If ursodeoxycholic acid is stopped, monitor for re-emergence of liver  
24 disease using clinical assessment and liver function blood tests.
- 25 1.7.21 Think about referring people with cystic fibrosis to a liver specialist if the  
26 liver function blood test results are persistently abnormal despite  
27 treatment with ursodeoxycholic acid.

1 1.7.22 Refer people with cystic fibrosis to a liver specialist if there is any of the  
2 following:

- 3 • chronic progressive liver disease, based on clinical assessment, liver  
4 function blood tests or the findings on a liver ultrasound scan
- 5 • liver failure, based on clinical assessment and liver function tests
- 6 • portal hypertension, haematemesis, splenomegaly or findings on a liver  
7 ultrasound scan.

### 8 **Cystic-fibrosis-related diabetes**

9 1.7.23 Diagnose cystic-fibrosis-related diabetes using one of the following:

- 10 • continuous glucose monitoring (CGM)
- 11 • serial glucose testing over several days
- 12 • oral glucose tolerance testing (OGTT) – if OGTT is abnormal perform  
13 CGM or serial glucose testing over several days to confirm the  
14 diagnosis
- 15 • HbA1c – if HbA1c is abnormal perform CGM or serial glucose testing  
16 over several days to confirm the diagnosis.

17 1.7.24 Test for cystic-fibrosis-related diabetes (as detailed in recommendation  
18 1.7.23) in people with cystic fibrosis annually from 10 years of age.

19 1.7.25 Test for cystic-fibrosis-related diabetes at the end of the first and second  
20 trimesters of pregnancy, using CGM or OGTT.

21 1.7.26 Test for cystic-fibrosis-related diabetes in people with cystic fibrosis who  
22 are taking long-term systemic corticosteroids or receiving enteral tube  
23 feeding, using CGM or serial glucose monitoring.

24 1.7.27 Think about testing for cystic-fibrosis-related diabetes in people who still  
25 have any of the following despite optimised cystic fibrosis treatment:

- 26 • unexplained weight loss
- 27 • a deterioration in lung function as measured by spirometry
- 28 • frequent pulmonary exacerbations

- 1
- excessive tiredness.

2 **Bone mineral density**

3 1.7.28 Consider dual energy X-ray absorptiometry (DXA) bone density scans for  
4 people with cystic fibrosis who have factors that put them at high risk of  
5 low bone mineral density, such as:

- 6
- frequent or long-term oral corticosteroid use
  - 7 • frequent intravenous antibiotic use
  - 8 • severe lung disease
  - 9 • undernutrition
  - 10 • previous low-impact fractures
  - 11 • previous transplants
  - 12 • post menopause.

13 1.7.29 Seek specialist advice for people with a bone mineral density standard  
14 deviation below -2.0 (Z score) or -2.5 (T score).

15 **Exercise**

16 1.7.30 Advise people with cystic fibrosis and their family members or carers (as  
17 appropriate) that regular exercise improves both lung function and overall  
18 fitness.

19 1.7.31 Offer people with cystic fibrosis an individualised exercise programme,  
20 taking into account their capability and preferences.

21 1.7.32 Regularly review exercise programmes to monitor the person's progress  
22 and ensure that the programme continues to be appropriate for their  
23 needs.

24 1.7.33 Provide people with cystic fibrosis who are having inpatient care with:

- 25
- an assessment of their exercise capacity
  - 26 • the facilities and support to continue their exercise programme (as  
27 appropriate), taking into account the need to prevent cross-infection  
28 (see [Preventing cross-infection](#)) and local infection control guidelines.

1 **Psychological assessment**

2 1.7.34 A specialist cystic fibrosis clinical psychologist (see [Multidisciplinary team](#))  
3 should be available to see people with cystic fibrosis at outpatient clinic  
4 visits and during inpatient admissions.

5 1.7.35 The specialist cystic fibrosis clinical psychologist should assess the needs  
6 of the family members or carers (as appropriate) of people with cystic  
7 fibrosis.

8 1.7.36 At the annual review, the specialist clinical psychologist should include  
9 assessments of:

- 10 • general mental health and wellbeing
- 11 • quality of life
- 12 • any factors that are making treatment adherence difficult
- 13 • psychosocial indicators
- 14 • behaviours that affect health outcomes.

15 1.7.37 If a serious mental health issue is identified at the annual review or at any  
16 assessment performed by the cystic fibrosis clinical psychologist, refer the  
17 person with cystic fibrosis to a mental health practitioner. For more  
18 guidance on treating depression, anxiety or panic disorder in adults, see  
19 the NICE guidelines on [depression in adults](#) and [generalised anxiety](#)  
20 [disorder and panic disorder in adults](#).

21 **1.8 Preventing cross-infection**

22 1.8.1 For recommendations on preventing and controlling infection, see the  
23 NICE guidelines on [infection control in primary and community care](#),  
24 [healthcare-associated infections](#) and the NICE quality standard on  
25 [infection prevention and control](#).

26 1.8.2 To prevent cross-infection among people with cystic fibrosis, use  
27 microbiological surveillance and a local infection control strategy that  
28 covers outpatient and inpatient care.

- 1 1.8.3 Inform people with cystic fibrosis, their family members or carers (as  
2 appropriate) and staff involved in their care about the risk of cross-  
3 infection and how to avoid it.
- 4 1.8.4 Separate people with cystic fibrosis who have transmissible or chronic  
5 pseudomonas aeruginosa or Burkholderia cepacia complex infection, for  
6 example during outpatient clinics.
- 7 1.8.5 Consider separating people with cystic fibrosis who have intermittent  
8 pseudomonas aeruginosa, for example during outpatient clinics.
- 9 1.8.6 All specialist cystic fibrosis clinics should be organised to prevent cross-  
10 infection. Separate people at outpatient clinics, for example by managing:
- 11 • use of communal waiting areas  
12 • attendance at diagnostic and treatment facilities.
- 13 1.8.7 Help people with cystic fibrosis plan their inpatient attendance to avoid  
14 contact with each other, for example when they use:
- 15 • hospital restaurants, schools and recreation areas  
16 • diagnostic and treatment facilities.
- 17 1.8.8 During inpatient care, give people with cystic fibrosis individual rooms with  
18 en-suite facilities.

### 19 ***Terms used in this guideline***

#### 20 **Home Care**

21 Giving care at home instead of a hospital, provided by the relevant cystic fibrosis  
22 specialist (such as a specialist nurse, dietician or psychologist).

#### 23 **Immunomodulatory dose**

24 A dose of a drug that is less than the minimum inhibitory dose.

1       **Outreach care**

2       A model of care in which the specialist multidisciplinary cystic fibrosis team provide  
3       outpatient clinics in local hospitals.

4       **Pulmonary exacerbation**

5       The sudden or recent worsening of clinical symptoms or signs. This is frequently  
6       caused by a respiratory infection.

7       **Pulmonary infection**

8       A sudden or recent respiratory infection. In people with cystic fibrosis, this can be  
9       diagnosed based on new or worsening symptoms or signs, or by identifying  
10      pathogens in respiratory secretion samples.

11      **Shared-care (network cystic fibrosis clinic)**

12      When a local hospital cares for people with cystic fibrosis, with oversight, support  
13      and direct involvement from members of a specialist cystic fibrosis centre.

14      **Young people**

15      Aged 12 to 17 years.

16      **Putting this guideline into practice**

17      NICE has produced [tools and resources](#) to help you put this guideline into practice.

18      Putting recommendations into practice can take time. How long may vary from  
19      guideline to guideline, and depends on how much change in practice or services is  
20      needed. Implementing change is most effective when aligned with local priorities.

21      Changes recommended for clinical practice that can be done quickly – like changes  
22      in prescribing practice – should be shared quickly. This is because healthcare  
23      professionals should use guidelines to guide their work – as is required by  
24      professional regulating bodies such as the General Medical and Nursing and  
25      Midwifery Councils.

26      Changes should be implemented as soon as possible, unless there is a good reason  
27      for not doing so (for example, if it would be better value for money if a package of  
28      recommendations were all implemented at once).

1 Different organisations may need different approaches to implementation, depending  
2 on their size and function. Sometimes individual practitioners may be able to respond  
3 to recommendations to improve their practice more quickly than large organisations.

4 Here are some pointers to help organisations put NICE guidelines into practice:

5 1. **Raise awareness** through routine communication channels, such as email or  
6 newsletters, regular meetings, internal staff briefings and other communications with  
7 all relevant partner organisations. Identify things staff can include in their own  
8 practice straight away.

9 2. **Identify a lead** with an interest in the topic to champion the guideline and motivate  
10 others to support its use and make service changes, and to find out any significant  
11 issues locally.

12 3. **Carry out a baseline assessment** against the recommendations to find out  
13 whether there are gaps in current service provision.

14 4. **Think about what data you need to measure improvement** and plan how you  
15 will collect it. You may want to work with other health and social care organisations  
16 and specialist groups to compare current practice with the recommendations. This  
17 may also help identify local issues that will slow or prevent implementation.

18 5. **Develop an action plan**, with the steps needed to put the guideline into practice,  
19 and make sure it is ready as soon as possible. Big, complex changes may take  
20 longer to implement, but some may be quick and easy to do. An action plan will help  
21 in both cases.

22 6. **For very big changes** include milestones and a business case, which will set out  
23 additional costs, savings and possible areas for disinvestment. A small project group  
24 could develop the action plan. The group might include the guideline champion, a  
25 senior organisational sponsor, staff involved in the associated services, finance and  
26 information professionals.

27 7. **Implement the action plan** with oversight from the lead and the project group.  
28 Big projects may also need project management support.

1 **8. Review and monitor** how well the guideline is being implemented through the  
2 project group. Share progress with those involved in making improvements, as well  
3 as relevant boards and local partners.

4 NICE provides a comprehensive programme of support and resources to maximise  
5 uptake and use of evidence and guidance. See our [into practice](#) pages for more  
6 information.

7 Also see Leng G, Moore V, Abraham S, editors (2014) Achieving high quality care –  
8 practical experience from NICE. Chichester: Wiley.

## 9 **Context**

10 Cystic fibrosis is a multi-system genetic disorder affecting the lungs, pancreas, liver  
11 and intestine. It can have a significant impact on life expectancy and quality of life.  
12 The current median age of those who have died is 28 years and the median  
13 predicted survival is 45.1 years.

14 Diagnosis is primarily made during newborn screening. The median age at diagnosis  
15 is 3 months, and 1 in every 2500 babies born in the UK has cystic fibrosis. More than  
16 57% of people on the UK cystic fibrosis registry are aged over 16 years.

17 Many different mutations are responsible for cystic fibrosis. The UK registry shows  
18 that 90.8% of people with cystic fibrosis have one known genotype; however 8.9% of  
19 people have at least one unknown genotype.

20 Lung function is often reduced in cystic fibrosis. The typical measure of lung function  
21 is forced expiratory volume in 1 second (FEV1). A FEV1 of 50% and above will  
22 enable people to live relatively normal lives and is associated with fewer difficulties in  
23 completing activities of daily living. A FEV1 above 85% indicates normal or near-  
24 normal lung function.

25 Lung infections are a cause of significant morbidity in cystic fibrosis. Chronic  
26 infection (for example with staphylococcus aureus and pseudomonas aeruginosa)  
27 may need long-term use of antibiotics.



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

8 By making robust recommendations based on the available evidence and best  
9 practice in cystic fibrosis care, this guideline will help improve care for this highly  
10 complex condition.

### 11 ***More information***

To find out what NICE has said on topics related to this guideline, see our web  
page on [cystic fibrosis](#) and [healthcare-associated infections](#).

## 13 **Recommendations for research**

14 The Committee has made the following recommendations for research. The  
15 Committee's full set of research recommendations is detailed in the [full guideline](#).

### 16 ***1 Monitoring for liver disease***

17 Should all children with meconium ileus receive ursodeoxycholic acid from  
18 diagnosis?

#### 19 **Why this is important**

20 Liver disease is the third most common cause of mortality in people with cystic  
21 fibrosis, and around 10 to 30% of people with cystic fibrosis will develop cystic-  
22 fibrosis-related liver disease. Children with meconium ileus are at an increased risk  
23 of liver disease, and starting treatment with ursodeoxycholic acid from diagnosis may  
24 reduce this risk. Ursodeoxycholic acid appears safe, is well tolerated and cheap.  
25 Routine use could increase people's overall quality of life and reduce the need for  
26 subsequent treatment for liver disease, but more research is needed into the  
27 effectiveness and safety of this treatment.

1 **2 *Airway clearance techniques***

2 How effective are daily airway clearance techniques in maintaining lung function in  
3 infants and children with cystic fibrosis?

4 **Why this is important**

5 There has been debate about the level of physiotherapy needed to preserve lung  
6 health since healthcare systems started diagnosing cystic fibrosis through newborn  
7 screening. Some clinical teams teach parents airway clearance techniques and  
8 recommend using them daily, but others use alternatives such as parental  
9 respiratory assessment tools with structured exercise. Routine airway clearance from  
10 diagnosis takes up a lot of time and places considerable responsibility on the parents  
11 and carers. These techniques are also difficult to perform, particularly with an infant  
12 or young child who does not understand what is happening. It is important to find out  
13 whether daily airway clearance techniques are helping to maintain lung health or are  
14 creating an unnecessary burden on parents and carers. Future research should look  
15 at the impact on the lives of parents, family members and carers, as well as long-  
16 term clinical outcomes for infants and children with cystic fibrosis.

17 **3 *Monitoring pulmonary disease***

18 Is lung clearance index a useful and cost-effective tool for the routine assessment  
19 and monitoring of changes in pulmonary status in people with cystic fibrosis?

20 **Why this is important**

21 Assessing the severity of lung disease is difficult in younger children, as not all  
22 children under 5 years can do spirometry tests and they are not sufficiently sensitive  
23 in people with good lung function, where CT scans can show pulmonary status  
24 changes before spirometry changes. A simple, sensitive and reproducible  
25 measurement such as lung clearance index allows assessment of respiratory status  
26 in people with cystic fibrosis, and could improve clinical decision-making.

27 **4 *Psychological assessment***

28 What is the most effective measure of psychological functioning to use as a  
29 screening test for thresholds of concern in people with cystic fibrosis?

1 **Why this is important**

2 There are no validated tools to assess psychological and behavioural problems in  
3 people with cystic fibrosis, and these would be useful to validate generic measures  
4 (for example for depression and anxiety). People with a long-term physical health  
5 condition are more likely to present with mental health problems. NHS England  
6 highlights that prevention of mental health problems is the most cost-effective  
7 service that can be provided. To prevent mental health problems, all people with  
8 cystic fibrosis would need to have their mental health status routinely and regularly  
9 assessed. People with cystic fibrosis would benefit, therefore, from having a routine  
10 screening test that would show who needs psychological intervention. This would  
11 allow early intervention to maintain or improve quality of life, prevent mental health  
12 problems from developing, and improve health outcomes through an improvement in  
13 wellbeing.

14 ***5 Monitoring for cystic-fibrosis-related diabetes***

15 What is the most effective screening strategy to detect diabetes in people with cystic  
16 fibrosis?

17 **Why this is important**

18 Diabetes develops and presents very differently in people with cystic fibrosis.  
19 Although annual screening for cystic-fibrosis-related diabetes is recommended in this  
20 guideline for people over 10 with cystic fibrosis, there is a lack of evidence how to  
21 diagnose the condition. There is currently variation in practice, with cystic fibrosis  
22 centres using different combinations of the oral glucose tolerance test, HbA1C, serial  
23 glucose testing, and continuous glucose monitoring systems. Identifying which  
24 strategy is most effective for early identification of cystic-fibrosis-related diabetes  
25 would help teams start prompt treatment and prevent the clinical decline associated  
26 with the condition.