Cochrane Cystic Fibrosis & Genetic Disorders Group

Newsletter

February 2012

Issue 44

Editorial: Updating Policy Change

Tracey Remmington, Managing Editor

The **Cochrane Editorial Unit (CEU)** has advised that all updated Cochrane Reviews (CRs) are to be given a new citation. This is so the wording of abstracts is always consistent between the *Cochrane Database of Systematic reviews* (*CDSR)* & other databases, such as MEDLINE.

The National Library of Medicine highlighted user problems caused by inconsistencies between abstracts published in the *CDSR* and in PubMed. These arose because the data feed to PubMed with each new issue of the *CDSR* includes **only** information about articles with new citations. Not all updates of CRs have new citations, and therefore the abstracts for updated CRs without a new citation were not being sent to PubMed.

Note: When a CR receives a new citation, a new licence for publication form is required for each author prior to publication.

Concerns that citing all updates might lead to a reduced impact factor (IF) are recognised. The CEU has modelled the effects of what would have happened to the 2010 IF, had all CR updates been cited from 2008 onwards. The actual 2010 IF was 6.186, the CEUs most confident estimate for the modelled IF was 5.926 (worst-case estimate being 4.440). For more information: http://www.editorial-unit.cochrane.org/citation-updated-reviews.

- Current Titles Registered -

- Anti Ig E and immunotherapy for allergic bronchopulmonary aspergillosis in people with CF
- Antibiotic treatment for non-tuberculous mycobacteria lung infection in people with CF
- Cystic fibrosis transmembrane conductance regulator correctors for cystic fibrosis
- Cystic fibrosis transmembrane conductance regulator potentiators for CF
- Desmopressin acetate (DDAVP) for preventing acute bleeds during pregnancy in women with congenital bleeding disorders
- Enzyme replacement and substrate reduction therapy for Gaucher disease
- Enzyme replacement therapy with galsulfase for mucopolysaccharidosis type VI
- Eradication therapy for Burkholderia cepacia complex (BCC) in people with cystic fibrosis
- Interventions for caregivers for the recognition of disease-related complications in children with sickle cell anaemia
- Interventions to reduce the utilization of transfusion therapy in people with thalassaemia major and intermedia
- Non-surgical interventions for treating menorrhagia in women with bleeding disorders
- Psychological therapies for people with hemophilia and their families
- Splenectomy for people with thalassaemia major and intermedia
- Treatment for osteoporosis in people with beta thalassaemia
- Treatment for preventing bleeding in people with congenital bleeding disorders undergoing surgery
- Vasodilator therapy for pulmonary vascular complications in people with sickle cell disease

What's Inside:

A REVIEW GROUP OF THE COCHRANE COLLABORATION®

Output of the CFGD Group

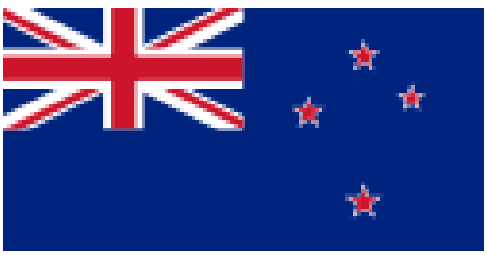
108 reviews & 22 protocols published on Issue 3 2012 of *The Cochrane Library*

New Director of UK Cochrane Centre, Martin Burton

Nikki Jahnke
Asst Managing Editor

Martin Burton has been the new Director of the UK Cochrane Centre, since September 2011. He is the first practising clinician to be appointed in the role and will continue to be an Ear, Nose and Throat Consultant at the Oxford Radcliffe Hospitals NHS Trust. Mr Burton is also a Senior Clinical Lecturer in the Nuffield Department of Surgery at the University of Oxford and has been a member of the Cochrane Collaboration since 1998 when he helped establish the Cochrane Ear, Nose and Throat Disorders Group.

As part of his role as Director of the UK Cochrane Centre, Mr Burton has been visiting all the UK-based Cochrane Groups. He describes himself as “an enthusiastic advocate for evidence-based medicine” and is pleased that his new role allows him “to work with others who are equally passionate and committed to improving the quality of healthcare for patients in the UK and beyond.” He has a vision for the future of the UK Cochrane Centre which “centres around engaging with patients, healthcare workers – of all sorts – and researchers, to ensure that patients receive the best and most effective treatments and avoid or minimise the harms that some therapies may cause."



20th Cochrane Colloquium Auckland, New Zealand 30th Sept – 3rd Oct 2012

Tracey Remmington
Managing Editor

Originally it had been planned to hold the 2012 Cochrane Colloquium in China. However, recent changes in Chinese government policy governing scientific meetings have made it impossible for the Chinese Cochrane Centre to secure government approval for a meeting of international delegates on the scale of a Colloquium with such short notice.

The Chinese Cochrane Centre has been working since 2009 to organize the 2012 Colloquium, and has engaged extensively in negotiations with the relevant government ministries, so it is with great disappointment that it has concluded that it will not be possible to host a Chinese Colloquium at this time. The Steering Group is exceptionally grateful to all the staff of the Centre, and in particular Youping Li and Mingming Zhang, as well as the contributors from Nanning (Guangxi), for all the preparations made for the Colloquium on behalf of the Collaboration. It is also grateful to Phil Wiffen and Steve McDonald for support they have provided to the Chinese Cochrane Centre.

The Collaboration’s annual Colloquium is a unique opportunity for Cochrane contributors and those interested in evidence-based health care to meet together in person. The Steering Group were therefore very pleased to announce that they have accepted the generous offer of the New Zealand Branch of the Australasian Cochrane Centre, co-directed by Cindy Farquhar and Mark Jeffery, to host the 2012 Colloquium in Auckland, New Zealand, 30 September to 3 October.

Registration opens on 8th March 2012 and more information can be found at http://colloquium.cochrane.org/.

The Cochrane Collaboration: Reflecting on 2011and looking forward to 2012

Nikki Jahnke
Asst Managing Editor

2011 was an extremely successful year for The Cochrane Collaboration and all of its contributors:

• The fourth consecutive year that updated reviews (464 published) outnumbered new reviews (414 published), demonstrating the commitment of author teams to keeping their reviews relevant and based on the best available evidence.

• A 16.8% increase in global usage of The Cochrane Library.

• The establishment of Official Relations with the World Health Organization (the public health arm of the United Nations) providing the opportunity for the Collaboration to influence the way in which research evidence is created and used by the WHO.

• The launch of summaries.cochrane.org, a consumer-friendly site for Cochrane evidence which received a Web Award at the 2011 Plain English Campaign's annual awards.

Read more about our achievements in 2011 in the Annual Report (http://annualreport.cochrane.org/).

In 2012 the focus will be on ensuring the quality, stability and efficiency of the Collaboration's organisational structure and of its products, to support you better and to continue to meet users' needs by:

• A review of the Collaboration's publishing arrangements for its products and, based on this, conducting a process to engage a publishing partner from 2014, when our current publishing contract expires.

• A strategic session on 'Cochrane Content' at the mid-year meetings of the Collaboration's management committees to inform the development of The Cochrane Library over the next five years (see www.editorial-unit.cochrane.org/collaboration-strategic-session-2012-cochrane-content).

• Seeking and developing alternative funding sources for the Collaboration.

• Enhancing the organisation's monitoring and management functions, particularly of individuals, groups and functions which receive core Collaboration funding and/or use the Cochrane brand.

The Cochrane Collaboration relies on your support, enthusiasm and commitment to our vision and principles and welcomes your input at all stages. There is a discussion forum to ask a question or post a suggestion about any topic at any time: www.cochrane.org/forums/general/collaboration-questions.

UK Cochrane Contributors Meeting

The 17th Annual Meeting of UK- and Ireland-based contributors to The Cochrane Collaboration will take place on 20 – 21 March 2012 at Burleigh Court, University of Loughborough.

The meeting provides an opportunity for UK- and Ireland-based members actively involved with The Cochrane Collaboration to get together to learn, debate and network. This year the plenary sessions will focus on looking forward and the impact and sustainability of The Cochrane Collaboration.

The website for the UK and Ireland-based Contributors meeting is now live. For further information and to register go to: www.cochrane.ac.uk.

Alessandro Liberati

Tracey Remmington & Nikki Jahnke
Managing Editors

We would like to pay our respects to our Cochrane colleague, Alessandro Liberati, who died on 01 January 2012 after a prolonged battle with multiple myeloma.

His sense of irony, affability, smiles, communication skills, enthusiasm, energy, vision, and hard work were central to the launch, diffusion and growth of evidence-based medicine in Italy. He passed this to thousands of students during his years as a university professor and to vast numbers of others through his research, writing and presentations.

Alessandro became involved in clinical research during a spell of voluntary service in the early 1980s. His interest in evidence-based medicine increased as he became closer to Prof Thomas C. Chalmers and Sir Iain Chalmers. When Iain invited Alessandro to take up the task of establishing the Italian Cochrane Centre in 1994, as one of the first Centres in the young Cochrane Collaboration, Alessandro accepted with enthusiasm. His systematic reviews of chemotherapy for breast cancer and antibiotic prophylaxis in intensive care were key-milestones of research in their fields. But, after his diagnosis of multiple myeloma, Alessandro started to look at research on a broader basis. He strove to reduce distortions in the prioritization of research questions, to make them more relevant to patients' needs. In 2004, he wrote a BMJ personal view talking of his disease and the uncertainties he faced (see below). In the same year, Alessandro was appointed to drive an integrated research system, bringing together medical schools and public hospitals in Emilia Romagna. This was his last appointment as a research officer, in a long career which spanned decades and involved a dedication to helping people make the best possible choices in health care and healthcare research.

In October 2011 at the Madrid Colloquium, Alessandro sat down with writer Alan Cassels and shared his thoughts on the accomplishments of The Cochrane Collaboration and explained why he became involved and committed to the work of The Collaboration. The full interview was recorded and edited by Richard Davis with photos supplied by Jini Hetherington and Kay Dickersin and can be viewed at www.cochrane.org/features/farewell-professor-alessandro-liberati. In May 2011, Alessandro began a blog, his colleagues from the Italian Cochrane Centre have translated his final post into English for publication on the Cochrane Blog (www.cochrane.org/news/blog/all-my-friends-and%E2%80%A6-fellow-travellers).

Alessandro served not only his country but colleagues across the world, and countless others who never met him but who benefit from his work. His death has left a large hole in the lives of many friends and colleagues both outside and within The Cochrane Collaboration.

An interesting article ……

Liberati A.
Need to re-align patient-oriented and commercial and academic research [editorial]. *The Cochrane Library* 2012 (1 Jan).
http://www.thecochranelibrary.com/details/editorial/1431131/Need-to-re-align-patient-oriented-and-commercial-and-academic-research.html

A podcast of this review is available at:
www.cochrane.org/podcasts/issue-1-2-january-february-2012/pycnogenol-treatment-chronic-disorders

Pycnogenol for the treatment of chronic disorders

Reviewers: Schoonees A, Visser J, Musekiwa A, Volmink J

Abstract

Background

Oxidative stress has been implicated in the development of a number of conditions including cancer, arthritic disorders and cardiovascular disease. Pycnogenol®, a herbal dietary supplement derived from French maritime pine bark extract, is standardised to contain 70% procyanidin which is a powerful antioxidant. Pycnogenol® is marketed as a supplement for preventing or treating a wide range of chronic conditions.

Objectives

To assess the efficacy and safety of Pycnogenol® for the treatment of chronic disorders.

Search strategy

We searched CENTRAL (until 18 September 2010), MEDLINE (until 18 September 2010) and EMBASE (until 13 October 2010) as well as three trial registries. We also contacted the manufacturer of Pycnogenol® and hand-searched bibliographies of included studies.

Selection criteria

Randomised controlled trials evaluating the effectiveness of Pycnogenol® in adults or children with any chronic disorder were included. We assessed clinical outcomes directly related to the disorder (stratified as participant- and investigator-reported) and all-cause mortality as primary outcomes. We also assessed adverse events and biomarkers of oxidative stress.

Data collection & analysis

Two authors independently assessed trial eligibility, extracted all data and assessed risk of bias. A third author additionally extracted information on outcomes and results. With three exceptions, results for outcomes across studies could not be pooled.

Main results

This review includes 15 trials with a total of 791 participants that have evaluated Pycnogenol® for the treatment of seven different chronic disorders. These included asthma (two studies; N = 86), attention deficit hyperactivity disorder (one study; N = 61), chronic venous insufficiency (two studies; N = 60), diabetes mellitus (four studies; N = 201), erectile dysfunction (one study; N = 21), hypertension (two studies; N = 69) and osteoarthritis (three studies; N = 293). Two of the studies were conducted exclusively in children; the others involved adults.

Due to small sample size, limited numbers of trials per condition, variation in outcomes evaluated and outcome measures used, as well as the risk of bias in the included studies, no definitive conclusions regarding the efficacy or safety of Pycnogenol® are possible.

Authors' conclusions

Current evidence is insufficient to support Pycnogenol® use for the treatment of any chronic disorder. Well-designed, adequately powered trials are needed to establish the value of this treatment.

Timing of hypertonic saline inhalation in cystic fibrosis

Reviewers: Elkins M, Dentice R

Abstract

Background

Inhalation of hypertonic saline improves sputum rheology, accelerates mucociliary clearance and improves clinical outcomes of people with cystic fibrosis.

Objectives

To determine whether the timing of hypertonic saline inhalation (in relation to airway clearance techniques or in relation to time of day) has an impact on its clinical efficacy in people with cystic fibrosis.

Search strategy

We identified relevant randomised and quasi-randomised controlled trials from the Cochrane Cystic Fibrosis Trials Register, the Physiotherapy Evidence Database (PEDro), and international cystic fibrosis conference proceedings.

Date of the last search of the Group's Cystic Fibrosis Trials Register: 6 December 2011.

Selection criteria

Any trial of hypertonic saline in people with cystic fibrosis where timing of inhalation was the randomised element in the study protocol with either: inhalation up to six hours before airway clearance techniques compared to inhalation during airway clearance techniques compared to inhalation up to six hours after airway clearance techniques; or morning compared to evening inhalation with any definition provided by the author.

Data collection & analysis

Both authors independently assessed the trials identified by the search for potential inclusion in the review.

Main results

The search strategy identified 50 trial reports which represented 24 unique studies. One study, published only as an abstract, is awaiting further assessment. None of the other studies compared timing regimens for the inhalation of hypertonic saline and we excluded these from the review.

Authors' conclusions

This review did not identify any evidence comparing the timing of hypertonic saline inhalation in relation to airway clearance physiotherapy. Until such evidence becomes available, clinicians could advise patients to inhale hypertonic saline before airway clearance, because this is the only regimen evaluated in the studies that established the efficacy of the use of hypertonic saline. This review also did not identify any evidence comparing the timing of hypertonic saline inhalation in relation to time of day. Until such evidence becomes available, clinicians could advise patients to inhale hypertonic saline morning and evening; but if only one dose per day is tolerated, the time of day at which it is inhaled could be based on convenience or tolerability.

Given the competing theoretical rationales about why hypertonic saline could be more effective if inhaled at certain times, a trial comparing these various timing regimens should be conducted.

Deferasirox for iron chelation in people with transfusion-dependent thalassaemia

Reviewers: Meerpohl JJ, Antes G, Rücker G, Fleeman N, Motschall E, Niemeyer CM, Bassler D

Abstract

Background

Thalassemia is a hereditary anaemia due to ineffective erythropoiesis. In particular, people with thalassaemia major develop secondary iron overload resulting from regular red blood cell transfusion. Iron chelation therapy is needed to prevent long-term complications. Both deferoxamine and deferiprone have been found to be efficacious. However, a systematic review of the effectiveness and safety of the new oral chelator deferasirox in people with thalassaemia is needed.

Objectives

To assess the effectiveness and safety of oral deferasirox in people with thalassaemia and secondary iron overload.

Search strategy

We searched the Cystic Fibrosis and Genetic Disorders Group's Haemoglobinopathies Trials Register. We also searched MEDLINE, EMBASE, EBMR, Biosis Previews, Web of Science, Derwent Drug File, XTOXLINE and three trial registries: www.controlled-trials.com; www.clinicaltrials.gov; www.who.int./ictrp/en/. Date of the most recent searches of these databases: 24 June 2010.

Date of the most recent search of the Group's Haemoglobinopathies Trials Register: 03 November 2011.

Selection criteria

Randomised controlled trials comparing deferasirox with no therapy or placebo or with another iron chelating treatment.

Data collection & analysis

Two authors independently assessed risk of bias and extracted data. We contacted study authors for additional information.

Main results

Four studies met the inclusion criteria. Two studies compared deferasirox to placebo or standard therapy of deferoxamine (n = 47). The placebo-controlled studies, a pharmacokinetic and a dose escalation study, showed that deferasirox leads to net iron excretion in transfusion-dependent thalassaemia patients. In these studies, safety was acceptable and further investigation in phase II and phase III trials was warranted. Two studies, one phase II study (n = 71) and one phase III study (n = 586) compared deferasirox to standard treatment with deferoxamine. Data suggest that a similar efficacy can be achieved depending on the ratio of doses of deferoxamine and deferasirox being compared; in the phase III trial, similar or superior efficacy for surrogate parameters of ferritin and liver iron concentration could only be achieved in the highly iron-overloaded subgroup at a mean ratio of 1 mg of deferasirox to 1.8 mg of deferoxamine corresponding to a mean dose of 28.2 mg/d and 51.6 mg/d respectively. Data on safety at the presumably required doses for effective chelation therapy are limited. Patient satisfaction was significantly better with deferasirox, while rate of discontinuations was similar for both drugs.

Authors' conclusions

Deferasirox offers an important alternative line of treatment for people with thalassaemia and secondary iron overload. Based on the available data, deferasirox does not seem to be superior to deferoxamine at the usually recommended ratio of 1 mg of deferasirox to 2 mg of deferoxamine. However, similar efficacy seems to be achievable depending on the dose and ratio of deferasirox compared to deferoxamine. Whether this will result in similar efficacy in the long run and will translate to similar benefits as has been shown for deferoxamine, needs to be confirmed. Data on safety, particularly on rare toxicities and long-term safety, are still limited.

Therefore, we think that deferasirox should be offered as an alternative to all patients with thalassaemia who either show intolerance to deferoxamine or poor compliance with deferoxamine. In our opinion, data are still too limited to support the general recommendation of deferasirox as first-line treatment instead of deferoxamine. If a strong preference for deferasirox is expressed, it could be offered as first-line option to individual patients after a detailed discussion of the potential benefits and risks.

2012 Timetable for Cochrane Workshops

Asia-Pacific Region Workshops

For more information see: http://acc.cochrane.org/timetable-registration

Australasian Cochrane Centre

Date	Location	Type of Workshop
14 – 16 March 2012	Melbourne	Introduction to writing a Cochrane systematic review
14 – 18 May 2012	Melbourne	Review completion workshop
26 – 28 June 2012	Adelaide	Introduction to writing a Cochrane systematic review
04 – 06 July 2012	Sydney	Introduction to writing a Cochrane systematic review

Brazilian Cochrane Centre

For more information see: http://www.centrocochranedobrasil.org.br/

Canadian Cochrane Centre

Date	Location	Type of Workshop
07 – 08 July 2012	Hamilton, ON	Two-Day Standard Author Training Workshop

Dutch Cochrane Centre

Date	Location	Type of Workshop
20 Mar 2012	Amsterdam	Developing a Cochrane protocol of interventions
04 Jun 2012	Amsterdam	Developing a Cochrane review of interventions

German Cochrane Centre

Date	Location	Type of Workshop
22 Mar 2012	Freiburg	Workshop Systematic Reviews in Medicine
23 – 24 Mar 2012	Freiburg	GRADE Workshop - Grading Evidence and Recommendation
23 – 24 Mar 2012	Freiburg	Advanced GRADE Workshop - Grading Evidence and Recommendation

Iberoamerican Cochrane Centre

For more information see: http://www.cochrane.es/

Nordic Cochrane Centre

Date	Location	Type of Workshop
24 May 2012	Copenhagen	Developing a protocol for a Cochrane review
31 Oct 2012	Copenhagen	Developing a protocol for a Cochrane review

South African Cochrane Centre

For more information, please see http://www.mrc.ac.za/cochrane/workshops.htm

UK Cochrane Centre

Date	Location	Type of Workshop
06 Mar 2012	Oxford	Analysis for systematic reviews
07 Mar 2012	Oxford	Advanced Topics in the Analysis and Reporting of Systematic Reviews
22 – 23 March 2012	Loughborough	Annual Methods Training Event: Assessing risk of bias in Cochrane Reviews
09 May 2012	York	Understanding Searching Techniques to Inform HTA, Systematic Reviews and Guideline Development Training Course

US Cochrane Centre

Date	Location	Type of Workshop
29 Mar – 01 Apr 2012	Baltimore	Research Methods Training in Complementary and Integrative Medicine

Cochrane Centres

Centres share a responsibility for helping to co-ordinate and support the Cochrane Collaboration. The shared responsibility of the Cochrane Centres includes organising workshops, seminars and colloquia to support and guide the development of the Cochrane Collaboration.

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- Contact Details -

Please photocopy, complete and return the following section if :

- Your contact details have changed & you wish to be kept informed about the Cystic Fibrosis and Genetic Disorders Group

- You are not on our mailing list and you would like to receive information about the Group in the future

- You would like to be removed from the Group's mailing list

Name :
Job title / position :
Address :
Telephone :
Fax :
E-mail address :
Proposed contribution to Cystic Fibrosis and Genetic Disorders Group, if any (e.g. undertaking a review (give interested area) , hand searching, refereeing, etc) : **I would like to receive future mailings: Yes / No**

Cochrane Cystic Fibrosis and Genetic Disorders Group

Summary sheet (Feb 2012)

September 1995	Registered with the Cochrane Collaboration as the Cystic Fibrosis Group
December 1997	Scope of group expanded to include other genetic diseases

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Editors Emeritus: Prof Deborah Ashby (UK)
Dr Gerard Ryan (Australia)

Group Website: http://www.liv.ac.uk/cfgd

Current funding: NHS R&D Programme, UK

Trial Registers

The register of randomised controlled trials (RCTs) for **cystic fibrosis** contains 1875 references to 1109 RCTs. This is compiled from electronic searches of the Cochrane Central Register of Controlled Trials (updated each new issue), quarterly searches of MEDLINE, a search of EMBASE to 1995 and the prospective handsearching of two journals: *Pediatric Pulmonology*; and the *Journal of Cystic Fibrosis*. Unpublished work is identified by searching the abstract books of three major cystic fibrosis conferences: the International Cystic Fibrosis Conference; the European Cystic Fibrosis Conference and the North American Cystic Fibrosis Conference.

The **haemoglobinopathies** register holds 642 references to 334 trials, the **coagulopathies** register has 276 references to 191 trials, and there are also 146 references for **phenylketonuria** and 661 references for **hyperlipoproteinaemia** (subsets on the **inborn errors of metabolism** register). As well as the electronic searching described above the following are searched for trials to include in the genetic disorders registers: the journals: *Haemophilia* and the *Journal of Inherited Metabolic Disease*; and the proceedings of the European Haematology Association conference; the American Society of Hematology conference; the Caribbean Health Research Council Meetings; the National Sickle Cell Disease Program Annual Meeting; the European Haematology Association conference; the American Society of Hematology conference; and the Society for the Study of Inborn Errors of Metabolism conference.

Cystic fibrosis reviews

Active cycle of breathing technique for cystic fibrosis
Antibiotic adjuvant therapy for pulmonary infection in cystic fibrosis
Antibiotic strategies for eradicating *Pseudomonas aeruginosa* in people with cystic fibrosis
Antifungal therapies for allergic bronchopulmonary aspergillosis in people with cystic fibrosis
Anti-inflammatory drugs and analgesics for managing symptoms in people with cystic fibrosis -related arthritis
Antioxidant micronutrients for inflammation and oxidation in cystic fibrosis lung disease
Bisphosphonates for osteoporosis in people with cystic fibrosis
Chemical pleurodesis versus surgical intervention for persistent and recurrent pneumothoraces in cystic fibrosis
Chest physiotherapy compared to no chest physiotherapy for cystic fibrosis
Combination antimicrobial susceptibility testing for acute exacerbations in chronic infection of *Pseudomonas aeruginosa* in cystic fibrosis
Conventional chest physiotherapy compared to any form of chest physiotherapy for cystic fibrosis
Disease modifying anti-rheumatic drugs in people with cystic fibrosis -related arthritis
Dornase alfa for cystic fibrosis
Drug therapies for reducing gastric acidity in cystic fibrosis
Duration of IV antibiotic therapy for people with cystic fibrosis
Elective versus symptomatic intravenous antibiotic therapy for cystic fibrosis
Enteral tube feeding for cystic fibrosis
Home intravenous antibiotics for cystic fibrosis
Inhaled bronchodilators for cystic fibrosis
Inhaled corticosteroids for cystic fibrosis
Inspiratory muscle training for cystic fibrosis
Insulin and oral agents for managing cystic fibrosis-related diabetes
Macrolide antibiotics for cystic fibrosis
Nebulized and oral thiol derivatives for pulmonary disease in cystic fibrosis
Nebulised anti-pseudomonal antibiotic therapy for cystic fibrosis
Nebulised hypertonic saline for cystic fibrosis
Neuraminidase inhibitors for the treatment of influenza infection in people with cystic fibrosis
Newborn screening for cystic fibrosis
Non-invasive ventilation for cystic fibrosis
Omega-3 fatty acids for cystic fibrosis
Once daily versus multiple daily dosing with intravenous aminoglycosides for cystic fibrosis
Oral anti-pseudomonal antibiotics for cystic fibrosis
Oral calorie supplements for cystic fibrosis
Oral non-steroidal anti-inflammatory drugs for cystic fibrosis
Oral steroids for cystic fibrosis
Oscillating devices for airway clearance in people with CF
Oxygen therapy for cystic fibrosis
Palivizumab for prophylaxis against respiratory syncytial virus infection in children with cystic fibrosis
PEP physiotherapy for airway clearance in cystic fibrosis
Percutaneous long lines for administering intravenous antibiotics in people with cystic fibrosis
Physical training for cystic fibrosis
Prophylactic anti-staphylococcal antibiotics for cystic fibrosis
Psychological interventions for people with cystic fibrosis and their families
Self-management education for cystic fibrosis
Singing for children and adults with cystic fibrosis
Single versus combination intravenous antibiotic therapy for people with cystic fibrosis
Sodium channel blockers for cystic fibrosis
Timing of dornase alfa inhalation for cystic fibrosis
Timing of hypertonic saline inhalation in cystic fibrosis
Topical cystic fibrosis transmembrane conductance regulator gene replacement for CF-related lung disease
Topical nasal steroids for treating nasal polyposis in people with cystic fibrosis
Totally implantable vascular access devices for cystic fibrosis
Ursodeoxycholic acid for cystic fibrosis -related liver disease
Vaccines for preventing infection with *Pseudomonas aeruginosa* in people with cystic fibrosis
Vaccines for preventing influenza in people with cystic fibrosis

Cystic fibrosis reviews (continued)
Vitamin A supplementation for CF
Vitamin D supplementation for cystic fibrosis
Vitamin K supplementation for cystic fibrosis

Cystic fibrosis protocols
Antibiotic treatment for Burkholderia cepacia complex in people with cystic fibrosis experiencing a pulmonary exacerbation
Antibiotic treatment for *Stenotrophomonas maltophilia* in people with cystic fibrosis
Appetite stimulants for people with cystic fibrosis
Autogenic drainage for CF
Bronchoscopy-guided therapy for CF
Immunosuppressive drug therapy to prevent rejection following lung transplantation for cystic fibrosis
Inhaled antibiotics for pulmonary exacerbations in people with cystic fibrosis
Inhaled mannitol for cystic fibrosis
Interventions for the eradication of methicillin resistant *Staphyloccocus aureus* in people with CF
Interventions for promoting physical activity in people with cystic fibrosis
Intravenous antibiotics for pulmonary exacerbations in people with CF
Nebuliser devices for drug delivery in cystic fibrosis
Pancreatic enzyme replacement therapy for people with cystic fibrosis
Pneumococcal vaccines for cystic fibrosis
Recombinant growth hormone therapy for children and young adults with cystic fibrosis
Standard versus biofilm antimicrobial susceptibility testing for infection of *Pseudomonas aeruginosa* in cystic fibrosis
Vitamin E supplementation for cystic fibrosis

Haemoglobinopathy reviews
Antibiotics for treating acute chest syndrome in people with sickle cell disease
Antibiotics for treating community acquired pneumonia in people with sickle cell disease
Antibiotics for treating osteomyelitis in people with sickle cell disease
Blood transfusion for acute chest syndrome in people with sickle cell disease
Blood transfusion for preventing stroke in people with sickle cell disease
Deferasirox for iron chelation in people with transfusion-dependent sickle cell disease
Deferasirox for iron chelation in people with transfusion-dependent thalassaemia
Desferrioxamine mesylate for managing transfusional iron overload in people with transfusion-dependent thalassaemia
Drugs for preventing red blood cell dehydration in people with sickle cell disease
Fluid replacement therapy for acute episodes of pain in people with sickle cell disease
Gene therapy for sickle cell disease
Hematopoietic stem cell transplantation for children with sickle cell disease
Hydroxyurea for sickle cell disease
Inhaled bronchodilators for acute chest syndrome in people with sickle cell disease
Inhaled nitric oxide for treating acute chest syndrome in people with sickle cell disease
Neonatal screening for sickle cell disease
Oral deferiprone for iron chelation in people with thalassaemia
Phytomedicines (medicines derived from plants) for sickle cell disease
Piracetam for reducing the incidence of sickle cell disease crises
Pneumococcal vaccines for sickle cell disease
Preoperative blood transfusions for sickle cell disease
Prophylactic antibiotics for preventing pneumococcal infection in children with sickle cell disease
Psychological therapies to sickle cell disease and pain
Psychological therapies for thalassaemia
Regular long-term red blood cell transfusions for chronic chest complications in sickle cell disease
Splenectomy versus conservative management for acute sequestration crises in people with sickle cell disease
Stem cell transplantation for people with beta thalassaemia major
Treatment for avascular necrosis of bone in people with sickle cell disease
Treatments for priapism in boys and men with sickle cell disease
Vaccines for preventing invasive salmonella infections in people with sickle cell disease

Haemoglobinopathy protocols
Angiotensin-converting enzyme (ACE) inhibitors for proteinuria in people with sickle cell disease
Interventions for treating leg ulcers in people with sickle cell disease
Zinc supplementation for thalassaemia and sickle cell disease

Coagulopathy reviews
Clotting factor concentrates given to prevent bleeding and bleeding-related complications in people with hemophilia A or B
Recombinant Factor VIIa concentrate versus plasma derived concentrates for the acute treatment of Haemophilia A & inhibitors

Inborn errors of metabolism reviews
Bisphosphonate therapy for osteogenesis imperfecta
Carnitine supplementation for the treatment of inborn errors of metabolism
Dietary interventions for phenylketonuria
Dietary treatment for familial hypercholesterolaemia
Enzyme replacement therapy for Fabry disease
Enzyme replacement therapy with idursulfase for mucopolysaccharidosis type II (Hunter syndrome)
Hematopoietic stem cell transplantation for Gaucher disease
Newborn screening for homocystinuria
Protein substitute for children and adults with phenylketonuria
Recombinant growth hormone therapy for X-linked hypophosphatemia in children
Sapropterin dihydrochloride for phenylketonuria
Statins for familial hypercholesterolemia in children
Tyrosine supplementation in phenylketonuria

Inborn errors of metabolism protocols
Enzyme replacement therapy with laronidase (Aldurazyme®) for treating mucopolysaccharidosis type I

Orphan reviews
Dietary advice for illness-related malnutrition in adults
Embolisation therapy for pulmonary arteriovenous malformations
Intravenous alpha-1 antitrypsin augmentation therapy for treating patients with alpha-1 antitrypsin deficiency and lung disease
Oral protein calorie supplementation for children with chronic disease
Pycnogenol® for the treatment of chronic disorders

Orphan protocols
Surgical interventions for treating pectus excavatum