Editorial

Increased number of reviews in Inborn Errors of Metabolism and Coagulopathies

Tracey Remmington
Managing Editor

As we reported in the last issue of our newsletter, we have made a commitment to increase the number of reviews in ‘Inborn Errors of Metabolism’ (IEM) and Coagulopathies. Our plan is to publish a minimum of ten new reviews in each of these areas over the next five years. We are very pleased to report progress in the production of reviews in IEM. Since Dr John Walter has joined the editorial team we have rapidly increased output in this area and are attracting new review authors to begin undertaking new reviews.

This year alone (by Issue 7 of The Cochrane Library) we will have published three IEM reviews in familial hypercholesterolaemia, phenylketonuria and in Anderson-Fabry’s disease.

We are also hoping to report the same effect soon for our coagulopathy reviews. Our new Coagulopathies Editor, Dr Alfonso Iorio, has been working hard to update our two existing coagulopathy reviews and recruit new authors for a number of new reviews.

We are very keen to continue this momentum and further expand into these areas of our scope and look to welcome many more new authors, peer reviewers and consumers to the Group, while still producing good quality reviews in the other areas of our scope, cystic fibrosis and haemoglobinopathies.

- Current Titles Registered -

• Inhaled mannitol for cystic fibrosis
• Surgical interventions for treating pectus excavatum
• Newborn screening for homocystinuria
• Stem cell transplantation for people with beta thalassaemia major
• Vitamin E supplementation for cystic fibrosis
• Recombinant growth hormone therapy for children and young adults with cystic fibrosis
• Pneumococcal vaccines for cystic fibrosis
• Timing of hypertonic saline inhalation in cystic fibrosis
• Enzyme replacement therapy with laronidase for treating mucopolysaccharidosis type I
• Angiotensin-converting enzyme (ACE) inhibitors for proteinuria in people with sickle cell disease

Output of the CFGD Group

89 reviews & 21 protocols will be published on Issue 6, 2010 of The Cochrane Library

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50th CF review published!

Nikki Jahnke
Asst Managing Editor

Issue 5 2010 of The Cochrane Library saw the publication of our 50th systematic review on interventions for cystic fibrosis (CF).

Our CF reviews cover a wide range of topics and interventions. We have reviews on drug interventions covering antibiotic use, steroids and mucolytic drugs. There are a number of reviews on physiotherapy and exercise and several more on nutrition. We cover interventions affecting many aspects of life in dealing with CF including the lungs, the digestive system and psychological issues. We have reviews looking at traditional therapies alongside those looking at new and emerging therapies. And we still have a list of review titles that need undertaking to establish the evidence base for treatment of people with CF. This list will continue to grow as new therapies are developed.

Congratulations to all our CF authors and peer reviewers for their hard work in achieving this milestone!

Usage of The Cochrane Library in 2009

Nikki Jahnke
Asst Managing Editor

Wiley have now announced information about usage of The Cochrane Library for 2009. In 2009, full text downloads from The Cochrane Library on Wiley InterScience grew by 19% compared to 2008. When considering usage across all web platforms (including Wiley InterScience, Cochrane.org, La Bibliotheca Cochrane Plus, Bireme, OVID and EBSCO) Wiley estimate that there was a search on The Cochrane Library every 1 second, an abstract viewed every 2 seconds and a full text review downloaded every 3 seconds in 2009.

Our top CF review was ‘Conventional chest physiotherapy compared to other airway clearance techniques for cystic fibrosis’ and had over 1000 hits worldwide. The top review of interventions for sickle cell disease was ‘Hematopoietic stem cell transplantation for children with sickle cell disease’ with almost 500 hits worldwide.

Further usage details can be accessed at: http://www.thecochranelibrary.com/view/0/WebsiteUpdates.html#Usage_data

An interesting article…….

The theme of this year’s colloquium is ‘Bringing evidence-based decision-making to new heights’ – highly appropriate given the setting!

The annual Colloquium in 2010 is the first joint Colloquium between the Cochrane and Campbell Collaborations. While you are all familiar with The Cochrane Collaboration, some of you may not have heard of The Campbell Collaboration. It is an international research network that produces and publishes systematic reviews of the effects of social interventions in three areas - education, crime and justice and social welfare. The Campbell Collaboration aims to promote positive social change by contributing to better-informed decisions and better-quality public and private services around the world. The Campbell Library provides free online access to our systematic reviews, titles, protocols and user abstracts. For more information visit: www.CampbellCollaboration.org.

Registration for the colloquium opened in February and early registration ends on 6th July. Registration will be capped at 1200 attendees and will close on 4 October 2010. Onsite registration, if available, will be accessible after online registration closes.

The 2010 Colloquium promises to be interesting and highly informative. While workshop details are not yet available, the programme is taking shape. Confirmed speakers include Patricia Schroeder, a noted US Congressional Representative for many decades, who served recently as the president of the Association of American Publishers. She also led a multi-year study for the Institute on Civil Society to identify and promote programs to encourage social cohesion and restore a sense of community for Americans. Also speaking will be Professor David Weisburd who is the 2010 recipient of the Stockholm Prize in Criminology. These two speakers will be joined by Bob Wachter, the author of ‘Internal Bleeding: The Truth Behind America’s Terrifying Epidemic of Medical Mistakes’ and a memoer of Google’s Health Advisory Council, and also Ida Sim, who is Director of the Center for Clinical and Translational Informatics at the University of California, San Francisco.

There is also a varied social programme on offer which includes the welcome reception featuring a live 13-piece funk band complete with horn section, a native American blessing at the opening session and a farewell party with an old fashion barn dance complete with Bar-B-Que, line dancing and more! You will also have a free afternoon giving you the chance to explore the nearby natural wonders of the American West: Rocky Mountain National Park, Aspen, Black Canyon of the Gunnison, Dinosaur National Monument, Sand Dunes National Park and Glenwood Springs.

For more details about the Colloquium, details of how to register and the cost, please visit the colloquium website:

Abstract

Background
Respiratory syncytial virus (RSV) infection causes acute lung infection in infants and young children worldwide, resulting in considerable morbidity and mortality. Children with cystic fibrosis (CF) are prone to recurrent lung inflammation, bacterial colonisation and subsequent chronic airway disease, putting them at risk for severe RSV infections requiring intensive care and respiratory support. No treatment currently exists, hence prevention is important. Palivizumab is effective in reducing RSV hospitalisation rates and is recommended for prophylaxis in high-risk children with other conditions. It is unclear if palivizumab can prevent RSV hospitalisations and intensive care unit admissions in children with CF.

Objectives
To determine the efficacy and safety of palivizumab (Synagis®) compared with placebo, no prophylaxis or other prophylaxis, in preventing hospitalisation and mortality from RSV infection in children with CF.

Search strategy
We searched the Cochrane CF and Genetic Disorders Group Trials Register and scanned references of the eligible study and related reviews.


Selection criteria
Randomised and quasi-randomised studies.

Data collection & analysis
The authors independently extracted data and assessed risk of bias.

Main results
One study (186 infants up to two years old) comparing five monthly doses of palivizumab (N=92) to placebo (N=94) over one RSV season was identified and met our inclusion criteria. At six months follow-up, one participant in each group was hospitalised due to RSV; there were no deaths in either group. In the palivizumab and placebo groups, 86 and 90 children experienced any adverse event, while 5 and 4 children had related adverse events respectively. Nineteen children receiving palivizumab and 16 receiving placebo suffered serious adverse events; one participant receiving palivizumab discontinued due to this. At 12 months follow-up, there were no significant differences between groups in number of Pseudomonas bacterial colonisations or change in weight-to-height ratio.

Authors' conclusions
We identified one randomised controlled trial comparing five monthly doses of palivizumab to placebo in infants up to two years old with CF. While the overall incidence of adverse events was similar in both groups, it is not possible to draw conclusions on the safety and tolerability of RSV prophylaxis with palivizumab in infants with CF because the trial did not specify how adverse events were classified. Six months after treatment, the authors reported no clinically meaningful differences in outcomes; however no data were provided. Additional randomised studies are needed to establish the safety and efficacy of palivizumab in children with CF.
Abstract
Background
Cystic fibrosis is a genetically inherited, life-threatening condition that affects major organs. The management of cystic fibrosis involves a multi-faceted daily treatment regimen that includes airway clearance physiotherapy, taking pancreatic enzymes and other medications. Previous studies identified that compliance with this intensive treatment especially among adolescents with cystic fibrosis is poor. Because of both the nature and consequences of the illness and the relentless demands of treatments, many individuals with cystic fibrosis are likely to have a poor quality of life. Anecdotal evidence suggests that singing may provide rigorous exercises for the whole respiratory system as well as a means for emotional expression, which may enhance quality of life.

Objectives
To evaluate the effects of a singing intervention in addition to usual therapy on the quality of life, morbidity, respiratory muscle strength and pulmonary function of children and adults with cystic fibrosis.

Search strategy
We searched the Group's Cystic Fibrosis Trials Register, the Cochrane Central Register of Controlled Trials, major allied complementary data bases, and clinical trial registers. Hand searching for relevant conference proceedings and journals was also carried out.

Date of search of Trials Register: 02 September 2009.

Date of additional searches: 17 September 2009.

Selection criteria
Randomised controlled trials in which singing (as an adjunctive intervention) is compared with either a sham intervention or no singing in people with cystic fibrosis.

Data collection & analysis
No trials were found that met the selection criteria.

Main results
No meta-analysis could be performed.

Authors' conclusions
As no studies that met the criteria were found, this review is unable to support or refute the benefits of singing as a therapy for people with cystic fibrosis. Future randomised controlled trials are required to evaluate singing therapy for people with cystic fibrosis.
Abstract

Background
Pulmonary arteriovenous malformations are abnormal direct connections between the pulmonary artery and pulmonary vein which result in a right-to-left shunt. They are associated with substantial morbidity and mortality mainly from the effects of paradoxical emboli. Potential complications include stroke, cerebral abscess, pulmonary haemorrhage and hypoxaemia. Embolisation therapy is a form of treatment based on the occlusion of the feeding arteries to a pulmonary arteriovenous malformation and can prevent many of these debilitating and life-threatening complications.

Objectives
To determine the efficacy and safety of embolisation therapy in people with pulmonary arteriovenous malformations including a comparison with surgical resection and different embolisation devices.

Search strategy
We searched the Cystic Fibrosis and Genetic Disorders Group's Trials Registers (last searched 07 September 2009). We also searched the following databases: the Australian New Zealand Clinical Trials Registry; ClinicalTrials.gov; International Standard Randomised Controlled Trial Number Register; International Clinical Trials Registry Platform Search Portal (last searched 22 November 2009). We checked cross-references and searched references from review articles. Finally, we contacted manufacturers and specialised centres for unpublished and ongoing trials.

Selection criteria
Trials in which individuals with pulmonary arteriovenous malformations were randomly allocated to embolisation therapy compared to no treatment, surgical resection or a different embolisation device. Studies identified for potential inclusion were independently assessed for eligibility by two authors, with excluded studies further checked by a third author.

Data collection & analysis
No trials were identified. As this was the case, no analysis was performed.

Main results
There were no randomised controlled trials identified.

Authors' conclusions
Currently there are no randomised controlled trials to support or refute embolisation therapy for treatment of pulmonary arteriovenous malformations. However, randomised controlled trials are not always feasible on ethical grounds. Observational studies suggest that embolisation therapy reduces mortality and morbidity compared to no treatment in patients. A standardised approach to reporting with long-term follow up through registry studies can help to strengthen the evidence base for embolisation therapy in the absence of randomised controlled trials. Future viable randomised controlled trials may compare different embolisation devices against each other.
Reviewers: El Dib RP, Pastores GM

Abstract

Background

Anderson-Fabry disease is an X-linked defect of glycosphingolipid metabolism. Progressive renal insufficiency is a major source of morbidity, additional complications result from cardio- and cerebro-vascular involvement. Survival is reduced among affected males and symptomatic female carriers.

Objectives

To evaluate the effectiveness and safety of enzyme replacement therapy compared to other interventions, placebo or no interventions, for treating Anderson-Fabry disease.

Search strategy

We searched 'Clinical Trials' on The Cochrane Library, MEDLINE, EMBASE, LILACS and the Cystic Fibrosis and Genetic Disorders Group's Inborn Errors of Metabolism Trials Register (date of the most recent search: 07 April 2010).

Selection criteria

Randomized controlled trials of agalsidase alfa or beta in participants diagnosed with Anderson-Fabry disease.

Data collection & analysis

Two authors selected relevant trials, assessed methodological quality and extracted data.

Main results

Five studies comparing either agalsidase alfa or beta in 187 participants fulfilled the selection criteria. Both trials comparing agalsidase alfa to placebo reported on globotriaosylceramide concentration in plasma and tissue; aggregate results were non-significant. One study reported pain scores, there was a statistically significant improvement for participants receiving treatment at up to three months, mean difference -2.10 (95% confidence interval (CI) -3.79 to -0.41); at up to five months, mean difference -1.90 (95% CI -3.65 to -0.15); and at up to six months, mean difference -2.00 (95% CI -3.66 to -0.34). There was a significant difference in pain-related quality of life at over five months and up to six months, mean difference -2.10 (95% CI -3.92 to -0.28) but not at other time-points. Neither study reported deaths. One of the three trials comparing agalsidase beta to placebo reported on globotriaosylceramide concentration in plasma and tissue and showed significant improvement: kidney, mean difference -1.70 (95% CI -2.09 to -1.31); heart, mean difference -0.90 (95% CI -1.18 to -0.62); and composite results (renal, cardiac, and cerebrovascular complications and death), mean difference -4.80 (95% CI -5.45 to -4.15). There was no significant difference between groups for death; no studies reported on pain.

Authors' conclusions

Five small, poor quality randomised controlled trials provide no robust evidence for use of either agalsidase alfa and beta to treat Anderson-Fabry disease.
## 2010 Timetable for Cochrane Workshops

### Australasian Cochrane Centre

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<tr>
<th>Date</th>
<th>Location</th>
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<tr>
<td>07 June</td>
<td>Melbourne</td>
<td>Developing a Protocol for a Review</td>
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<tr>
<td>08 June</td>
<td>Melbourne</td>
<td>Introduction to Analysis</td>
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<tr>
<td>21 June</td>
<td>Gold Coast</td>
<td>Developing a Protocol for a Review</td>
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<tr>
<td>22 June</td>
<td>Gold Coast</td>
<td>Introduction to Analysis</td>
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<tr>
<td>23-24 June</td>
<td>Brisbane</td>
<td>Cochrane Review Completion and Update Program</td>
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<tr>
<td>07 July</td>
<td>Sydney</td>
<td>Cochrane Review Completion and Update Program</td>
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### Brazilian Cochrane Centre

For more information see: [http://www.centrocochranedobrasil.org.br/](http://www.centrocochranedobrasil.org.br/)

### Canadian Cochrane Centre

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<tr>
<td>01-02 June</td>
<td>Ottawa</td>
<td>Train the Trainer Workshop for Cochrane Review Author Training</td>
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### Dutch Cochrane Centre

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<th>Date</th>
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<tr>
<td>12 October</td>
<td>Amsterdam</td>
<td>Protocol workshop</td>
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### German Cochrane Centre

For more information see: [http://www.cochrane.org/events/w-shops/w-shops-de](http://www.cochrane.org/events/w-shops/w-shops-de)

### Iberoamerican Cochrane Centre

For more information see: [http://www.cochrane.es/Agenda](http://www.cochrane.es/Agenda)

### Nordic Cochrane Centre

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<tr>
<td>12 October</td>
<td>Copenhagen</td>
<td>Protocol workshop</td>
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### South African Cochrane Centre

For more information see: [http://www.mrc.ac.za/cochrane/project.htm](http://www.mrc.ac.za/cochrane/project.htm)

### US Cochrane Centre

For more information see: [http://apps1.jhsph.edu/cochrane/](http://apps1.jhsph.edu/cochrane/)

### UK Cochrane Centre

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<tr>
<td>08 June</td>
<td>Glasgow</td>
<td>Developing a Protocol for a Review</td>
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<td>09 June</td>
<td>Glasgow</td>
<td>Introduction to Analysis</td>
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<tr>
<td>16 June</td>
<td>Oxford</td>
<td>Advanced Topics in the Analysis and Reporting of Systematic Reviews</td>
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<td>28 September</td>
<td>York</td>
<td>Developing a Protocol for a Review</td>
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<td>29 September</td>
<td>York</td>
<td>Introduction to Analysis</td>
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### Asia-Pacific Region Workshops

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<tr>
<td>31 May -04 June</td>
<td>Vellore</td>
<td>Systematic Review Protocol Development and Analysis Workshop</td>
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<tr>
<td>05 June</td>
<td>Vellore</td>
<td>Summary of Findings and Grading Recommendations in Systematic Reviews</td>
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</table>
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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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

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**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 postal mailings: Yes / No
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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Dr Alfonso Iorio (Italy)  
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Dr Peter Wark (Australia)

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Prof Sally Davies (UK)  
Dr Ian Hambleton (Barbados)  
Prof Felix Ratjen (Canada)  
Prof Ros Smyth (UK)  
Dr John Walter (UK)

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 1789 references to 1065 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 604 references to 320 trials, the coagulopathies register has 261 references to 189 trials, and there are also 142 references for phenylketonuria and 658 references for hyperlipoproteinemia (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
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
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
Nebulized 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
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
Physical training for cystic fibrosis
Prophylactic anti-staphylococcal antibiotics for cystic fibrosis
Psychological interventions for people with cystic fibrosis and their families
Singing for children and adults with cystic fibrosis
Sodium channel blockers for cystic fibrosis
Topical cystic fibrosis transmembrane conductance regulator gene replacement for cystic fibrosis-related lung disease
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
Vitamin A supplementation for CF
Vitamin D supplementation for cystic fibrosis
Cystic fibrosis protocols
- Active cycle of breathing technique for cystic fibrosis
- Antioxidant micronutrients for inflammation and oxidation in cystic fibrosis lung disease
- Appetite stimulants for people with cystic fibrosis
- Inhaled antibiotics for pulmonary exacerbations in people with cystic fibrosis
- Nebuliser devices for drug delivery in cystic fibrosis
- Non-antibiotic therapies for pulmonary infection in cystic fibrosis
- Pancreatic enzyme replacement therapy for people with cystic fibrosis
- Percutaneous long lines for administering intravenous antibiotics in people with cystic fibrosis
- Self-management education for cystic fibrosis
- Timing of dornase alfa inhalation for cystic fibrosis
- Topical nasal steroids for treating nasal polyposis in people with cystic fibrosis
- Vitamin K 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
- 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
- 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
- Splenectomy versus conservative management for acute sequestration crises in people with sickle cell disease
- 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
- Deferasirox for iron chelation in people with transfusion-dependent sickle cell disease
- Deferasirox for iron chelation in people with transfusion-dependent thalassaemia
- Gene therapy for sickle cell disease
- Interventions for treating leg ulcers in people with sickle cell disease
- Regular long-term red blood cell transfusions for chronic chest complications in 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
Hematopoietic stem cell transplantation for Gaucher disease
Protein substitute for children and adults with phenylketonuria
Recombinant growth hormone therapy for X-linked hypophosphatemia in children
Tyrosine supplementation in phenylketonuria

Inborn errors of metabolism protocols
Enzyme replacement therapy with idursulfase for mucopolysaccharidosis type II (Hunter syndrome)
Intravenous alpha-1 antitrypsin augmentation therapy for treating patients with alpha-1 antitrypsin deficiency and lung disease
Sapropterin dihydrochloride for phenylketonuria
Statins for familial hypercholesterolemia in children

Orphan reviews
Dietary advice for illness-related malnutrition in adults
Embolisation therapy for pulmonary arteriovenous malformations
Oral protein calorie supplementation for children with chronic disease

Orphan protocols
Proanthocyanidin supplements for the treatment of chronic disorders