

Cochrane Database of Systematic Reviews - - Cochrane Protocol (ongoing review)

Nucleic acid-based therapies for people with cystic fibrosis

Code: CD016370

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Author: Addy C

Study design (if review, criteria of inclusion for studies)

Randomised controlled trials (RCTs) of parallel design (published or unpublished). Data from the first treatment period of any cross-over studies. Excluded: quasi-RCTs (i.e. trials using inappropriate strategies of allocating interventions), cluster-RCTs.

Participants

Eligible participants are people with a confirmed diagnosis of CF, of any age, sex or disease severity, regardless of CF genetic variant. If any studies include both healthy volunteers and people with CF

Interventions

Nucleic acid-based therapies (NABTs) (including variant-specific antisense oligonucleotide therapies, variant-agnostic mRNA, and DNA-based gene therapies) in comparisons with either no treatment, a placebo or a different NABT. These therapies are currently predominantly inhaled, but authors will not exclude any other methods of administration, e.g. intravenous, which may be employed in the future. There will be no restrictions on dose, frequency, duration or setting of the intervention.

Outcome measures

Critical outcomes: Quality of life (QoL) (Total score, Respiratory domain). FEV1. AEs. Survival. Pulmonary exacerbations. Important outcomes: Hospitalisation. School or work attendance (i.e. number of days missed). Extra courses of antibiotics (measured as time to the next course of antibiotics and the total number of courses of antibiotics), Sweat chloride. FVC, Lung clearance index. Radiological measures of lung disease. Microbiology: Acquisition of CF-associated pathogens, Eradication of CF-associated pathogens. Nutrition and growth: Weight, Body mass index (BMI), Height

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See also

Addy C, Johl N, Prayle AP, Jahnke N, Smith S, Cochrane Cystic Fibrosis . Nucleic acid-based therapies for people with cystic fibrosis. Cochrane Database of Systematic Reviews 2026, Issue 7. Art. No.: CD016370. DOI: 10.1002/14651858.CD016370. Accessed 15 September 2026.

Keywords

Gene Transfer Techniques; non pharmacological intervention - genetic& reprod; pharmacological_intervention; Nucleic acid-based therapies (NABTs); oligonucleotide;