

Cystic Fibrosis Trust – Statement of Purpose

What is the Cystic Fibrosis Trust?

The Cystic Fibrosis Trust is the UK's only national charity dealing with all aspects of Cystic Fibrosis (CF). It funds research to treat and cure CF and aims to ensure appropriate clinical care and support for people with Cystic Fibrosis.

The Cystic Fibrosis Trust was founded in 1964. Its objectives are:

- To fund medical and scientific **research** to develop a cure and provide effective treatments for Cystic Fibrosis
- To ensure appropriate **clinical care** for those with Cystic Fibrosis
- To provide **information, advice, support** and, where appropriate, financial assistance to anyone affected by Cystic Fibrosis.

Research

The aim of our research is to understand, treat and cure Cystic Fibrosis. The Cystic Fibrosis Trust is the major funder of medical and scientific CF research in the UK. Our research falls into two main categories:

1. Gene therapy - We currently invest around £4 ½ million each year in a programme of research into gene therapy to make it a clinical reality in the foreseeable future. Gene therapy aims to add a healthy copy of the faulty CF gene to the lung. The Cystic Fibrosis Trust has brought together 80 of the UK's leading scientists and clinicians to drive this research forward through the UK Cystic Fibrosis Gene Therapy Consortium. The scientists have developed a product, which they plan to put into a one year multi-dose clinical trial. It is estimated the product for the trial will cost at least £6 ½ million. A more precise figure will be known following a single dose pilot study, which began in 2009.
2. Medical and Scientific research – We support research aimed at curing and treating the symptoms of CF and improving the lives of those living with the condition. We currently spend around £800,000 a year on non-gene therapy research that will have important and/or imminent clinical benefit to those with Cystic Fibrosis.

Clinical Care

Cystic Fibrosis is a complex disease requiring considerable specialist treatment. In the last 15 years, the Trust has helped to set up and staff over 45 specialist CF treatment centres throughout the UK. Since 1997, the Cystic Fibrosis Trust has invested over £10 million in the NHS to improve clinical care for the 8,500 people in the UK with CF, helping fund doctors, nurses and multidisciplinary teams. We

continue to fund the training of the CF specialists of tomorrow and we make occasional small development grants to improve the NHS care of those with CF in conjunction with Hospital Trusts and Specialist Commissioners.

We set national standards on clinical care, provide and fund a UK CF Clinical Database and through peer review of CF centres and clinics, measure levels of service provision. Our Expert Patient Advisers (who all have CF) work with health providers and government to influence and improve the care of those with CF across the UK.

Information, advice and support

The Cystic Fibrosis Trust provides a confidential helpline service, 9-5 Monday to Friday for advice and support on any aspect of Cystic Fibrosis. We also offer information and advice to those affected, their families and friends, schools and employers and anyone interested in Cystic Fibrosis. We advise families on government benefits that may be available and, where appropriate, provide financial assistance and welfare grants.

Key Messages

The Cystic Fibrosis Trust is the UK's only national charity dealing with all aspects of Cystic Fibrosis (CF). It funds research to treat and cure CF and aims to ensure appropriate clinical care and support for people with Cystic Fibrosis.

Cystic Fibrosis (CF) is one of the UK's most common life-threatening inherited diseases. Cystic Fibrosis is caused by a single defective gene. As a result, the internal organs, especially the lungs and digestive system, become clogged with thick sticky mucus resulting in chronic infections and inflammation in the lungs and difficulty digesting food.

There are over 8,500 people in the UK with Cystic Fibrosis.

Each week five babies are born with Cystic Fibrosis and three young people die – 90% from lung damage.

Around half of the CF population can expect to live over 38 years, although improvements in treatments mean a baby born today can expect to live for even longer.

1 person in 25 carries the faulty CF gene – over two million people in the UK. If two carriers have a baby, the child has a 1 in 4 chance of having Cystic Fibrosis.

The Cystic Fibrosis Trust funds gene therapy research to add a healthy copy of the faulty CF gene to the lung. This cutting-edge research costs around £4 million a year.

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Our top researchers have developed a gene therapy product. A single dose pilot study began in 2009 and a major clinical trial will follow the results of this costing at least a further £6 ½ million.

Since 1997, the CF Trust has invested over £10 million in the NHS to improve the clinical care of those with Cystic Fibrosis by funding doctors, nurses and multidisciplinary teams.

Further information can be found on our website www.cftrust.org.uk. Help and advice for those affected by Cystic Fibrosis is available through the Cystic Fibrosis Helpline on 0845 859 1000.

Press Release: Notes to Editors:

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Each week five babies are born with Cystic Fibrosis and three young people die – 90% from lung damage. Median predicted survival is now 38, although improvements in treatments mean a baby born today is expected to live longer.

Further information can be found on our website www.cftrust.org.uk. Help and advice for those affected by Cystic Fibrosis is available through our Helpline on 0845 859 1000. For further information, media should contact....