



REVIEW OF UK HEALTH RESEARCH

A response from the Cystic Fibrosis Trust

We have read with interest the recent report by Sir David Cooksey on the reorganisation of funding for research in Biomedical Sciences. We are broadly supportive of this approach and can see many advantages in a united strategy between MRC and the Research and Development funding from the NHS. We do, however, have two main areas of concern.

1. Maintaining a focus on translational research

We are concerned that the translational and clinical research that is currently the main focus of the NHS will remain a major focus under the new arrangement. Whilst we are very supportive of basic science, we strongly believe that there should be clear mechanisms for new discoveries made in model systems to be quickly translated into proof of principle studies in man thereby enabling new treatments to become available as soon as possible.

The current research assessment exercise in universities focuses many groups on basic science because the outputs from basic science are more quickly achieved and are often published in high impact factor journals more often than clinical research. Clinical trials, including proof of principle studies, take a considerable amount of planning and manpower support and are therefore often not priority areas of research for universities.

2. Prioritising orphan diseases

Our other concern is **that diseases** such as Cystic Fibrosis, which account for a relatively small number of people, may not be considered as priorities in the new arrangement. This would be unhelpful for a two main reasons.

Firstly, the burden of disease in people with cystic fibrosis is enormous and although the numbers are small the burden of illness and the cost to the NHS is very high.

Secondly, Cystic Fibrosis is, we believe, a sentinel disease in terms of seeing through to other therapies.

Currently the Cystic Fibrosis Trust is funding a long-term gene therapy program. This has involved bringing together three previously competing groups of scientists and clinicians to work together in a consortium to see the basic science developments in gene transfer agents and **plasmids** through to a proof of principle study in man. This program is approximately half way through and has attracted support from the Department of Health R&D Office. The proof of principle study should be completed by the end of 2008.

This approach has already accelerated the development of clinically useable product and has engaged academics, clinicians and industry in a very productive, collaborative approach. We strongly believe that if there is a unified budget it is important that this approach continues to be supported for Cystic Fibrosis and other orphan diseases.

We are grateful for the opportunity to provide some input. Although these do not address [all](#) of the specific review questions in the consultation document, we hope that our response will be helpful to your considerations.

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