

Improving Lives, Optimising Resources: How a strategy for rare diseases will benefit the people of England



RARE DISEASE | UK

The National Alliance for people with rare diseases & all who support them

In England, 3 million people are likely to be affected by a rare disease at some point in their lives (1 in 17 people). Collectively, rare diseases are not rare and they represent a significant health burden to the health services in England.

Rare Disease UK's (RDUK) report *Improving Lives, Optimising Resources: A Vision for the UK Rare Disease Strategy* describes how currently there are a number of obstacles preventing patients and families being able to access the high quality services, treatment, care and support that would ensure they benefit from optimum health outcomes. The report sets out recommendations developed in collaboration with a broad range of stakeholders in England and across the UK to address these issues. These recommendations should form the basis of an effective strategy for rare diseases.

A strategy for rare diseases would ensure that patients receive the highest quality healthcare whilst ensuring the most efficient and effective use of NHS resources.

The Department of Health's (DH) White Paper *Equity and Excellence: Liberating the NHS¹* makes a number of commitments to patients in England. RDUK supports those commitments and the ultimate aim of delivering the highest quality healthcare to the people of England. RDUK looks forward to working in partnership with the DH and key stakeholders to develop an effective strategy to ensure that the aims laid out in the White Paper and supporting documents are realised for people with rare diseases.

How RDUK's recommendations for a strategy for rare diseases would enable the Government to meet its aims:

Aim: for an NHS that is 'genuinely centred on patients and carers' and to develop a system that will 'focus on personalised care that reflects individuals' health and care needs'

RDUK's recommendations encourage this by, for example, the production of individual care plans, the designation of Care Coordinators, and the development of personalised information prescriptions for patients to provide them with ongoing, reliable information on their condition, relevant services and treatments. This would also help to empower patients with knowledge to facilitate shared decision making, promoting the idea of 'no decision about me without me'.

Aim: to ensure that 'everyone, whatever their need or background, benefits from these [NHS] arrangements' and to 'reduce inequalities in care'

RDUK's recommendations facilitate this through methods to ensure equitable, timely access to effective diagnostic tests, drugs and services for all patients that need them, regardless of their location or the rarity of their condition. Recommendations to aid timely diagnosis would avoid unnecessary or harmful interventions and ensure early access to effective interventions and better management of the condition.

Aim: for an NHS that 'is less insular and fragmented, and works much better across boundaries'

Adopting RDUK's recommendations would encourage effective collaboration and communication between everyone involved in a patient's care; it would improve continuity of care at transition periods; it would help to ensure clear communication between patients and professionals, including information on their condition, management and treatments.

1. 'Equity and excellence: Liberating the NHS', Department of Health, July 2010

Aim: to 'bring about an NHS information revolution'

A lack of information on rare diseases affects the ability of the NHS to deliver good quality healthcare to patients, the ability of patients and families to effectively manage their condition and is distressing for patients and families who struggle to access information on their condition and sources of support. RDUK's recommendations including information prescriptions, an online information portal and methods to facilitate the exchange of information between professionals would ensure that this 'information revolution' is realised for patients with rare diseases.

Aim: research 'is essential if we are to increase the quality and productivity of the NHS'

RDUK strongly supports this statement. Several recommendations are made to facilitate and improve research into rare conditions to help ensure that quality and productivity in this area can be improved. These include methods to reduce the bureaucracy involved in medical research, improve collaboration between researchers and to promote effective data collection initiatives.

Aim: to ensure that 'funding is used as efficiently as possible'

Patients with rare diseases already make heavy demands on the resources of both health and social services but these resources are often used inefficiently due to delays in diagnosis, misdiagnosis, fragmented care, a lack of information, few guidelines on the effective management of conditions and limited effective treatment options. Taking on board RDUK's recommendations will ensure the most effective use of resources whilst promoting the best possible health care for patients with rare diseases.

In June 2009, the Government adopted the Council of the European Union's Recommendation on an action in the field of rare diseases. This calls on Member States to develop plans or strategies for rare diseases. The Government has committed to working collaboratively with the devolved nations to develop a strategy for rare diseases. RDUK calls on the Government to take on board the recommendations outlined in *Improving Lives, Optimising Resources: A Vision for the UK Rare Disease Strategy* and implement an effective strategy for rare diseases.

Some excellent examples of good practice already exist in England and these are highlighted throughout the report. It is vital that these are developed or used as models on which to base new services. Where expertise already exists, it should be shared and utilised to benefit others.

About Rare Disease UK

RDUK is the national alliance for people with rare diseases and all who support them. Our membership is open to all and includes patient organisations, clinicians, health professionals, researchers, academics, industry and individuals with an interest in rare diseases.

RDUK was established by Genetic Alliance UK, the national charity of over 130 patient organisations supporting all those affected by genetic conditions, in conjunction with other key stakeholders in November 2008 following the European Commission's *Communication on Rare Diseases: Europe's Challenges*.

RDUK aims to work with government and policy makers to develop and aid the implementation of an effective strategy for rare diseases in the UK.



RARE DISEASE | UK



Genetic Alliance UK
Supporting. Campaigning. Uniting.

www.raredisease.org.uk | info@raredisease.org.uk | 020 7704 3141