

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



RARE DISEASE | UK

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

In Northern Ireland, 100,000 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 health and social services in Northern Ireland.

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 Northern Ireland 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 Health and Social Care resources.

*Quality 2020, A 10-year quality strategy for health and social care in Northern Ireland – A consultation document*¹ and *Priorities for Action 2010/11*² produced by the Department of Health, Social Services and Public Safety (DHSSPS) make a number of commitments to patients in Northern Ireland. RDUK supports those commitments and the ultimate aim to deliver the highest quality health and social care to the people of Northern Ireland. RDUK looks forward to working in partnership with the DHSSPS and key stakeholders to develop an effective strategy to ensure that the aims laid out in these documents are realised for people with rare diseases.

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

Aim: to ensure services are organised 'around the needs of the individual patient, meeting their clinical needs, working in partnership and treating them with dignity and respect'²

RDUK's recommendations encourage this by, for example, the production of individual care plans, the designation of Care Coordinators and the development of information prescriptions for patients. Partnership with patients is promoted through the use of patient held records and enabling patients access to their records online.

Aim: there should be 'consistency and fairness in service delivery'¹ and 'no inappropriate variation in the care and treatment people are receiving'²

RDUK's recommendations facilitate this through methods to ensure equitable, timely access to effective diagnostic tests, drugs and therapies 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: to work 'collaboratively across all disciplines, sectors and specialisms in health and social care to ensure an integrated team-based approach'¹ and to ensure 'continuity of care across the system'¹

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.

Aim: 'to ensure that all of the resources available to the Northern Ireland health and social care service are used appropriately and effectively to improve the health and wellbeing of the Northern Ireland population and to provide better treatment and care.'²

Implementing RDUK's recommendations will ensure the most effective use of resources whilst promoting the best possible outcomes for patients with rare diseases.

1. 'Quality 2020, A 10-year quality strategy for health and social care in Northern Ireland. A consultation document', Department of Health, Social Services and Public Safety, January 2011

2. 'Priorities for Action 2010/11', Department of Health, Social Services and Public Safety, May 2010

In June 2009, the UK 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 DHSSPS has committed to working collaboratively with the other home nations to develop a strategy for rare diseases. RDUK calls on the DHSSPS 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.

It is vital to ensure that patients are able to access specialised services based elsewhere in the UK and that diagnostic tests available in other parts of the UK are available in Northern Ireland. However, there are excellent examples of good practice in Northern Ireland. 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.

Cross border and cross sector collaboration: an example of good practice in Ireland

The Irish Platform for Patient Organisations, Science and Industry (IPPOSI) covers the island of Ireland and is a partnership between the three key groups to provide a structured way of facilitating interaction on policy, legislation and regulation around the development of new treatments and interventions for unmet medical needs in Ireland. The aim is to ensure that state-of-the-art healthcare innovations are available at the earliest stages to patients in Ireland and that there is a smooth progression from basic science in laboratories to delivery of treatments to patients that need them. IPPOSI now submits to consultations, holds annual conferences, produces monthly newsletters and updates, produces reports and briefing papers, provides advice and facilitates interaction and information sharing between various bodies at national and international levels.

European Surveillance Project: an example of good practice based in Northern Ireland

The European Surveillance of Congenital Anomalies (EUROCAT) is based at the University of Ulster and is a European network of population-based registries for the epidemiologic surveillance of congenital anomalies. This network is made up of 43 registries in 20 countries, which together survey more than 1.5 million births per year in Europe. This network provides an excellent infrastructure for research into the causes and prevention of congenital anomalies and the treatment of affected children. It is able to provide epidemiological information on congenital anomalies, evaluate the effectiveness of primary interventions, act as an information and resource centre, facilitate the identification of teratogenic exposures and facilitate the planning and evaluation of health services.

It is an excellent example of international collaboration which is essential to gain a better understanding of rare diseases.

Patient support workers and rare disease clinics: an example of good practice

The Society for Mucopolysaccharide Diseases (MPS Society) and the Primary Immunodeficiency Association, in partnership with the Belfast Trust, have appointed an **All-Ireland Advocacy Support Worker** to provide support to patients and families affected by these conditions across Ireland. The role is to provide expert information and advice to patients, families and carers on the non-clinical issues associated with these conditions, including access to appropriate care packages and equipment, information on benefits and education, and ensuring that all health and social care professionals that they deal with are aware of the complexities of their condition. They also act as a listening service for patients and families and help them to make informed decisions.

The MPS Society have played a key role in meeting the needs of individuals diagnosed with MPS and related diseases in Northern Ireland for many years. Their involvement at the **bi-annual specialist MPS clinic** is an example of this. The clinic provides an opportunity for patients to be reviewed by consultants (from across the UK) who specialise in MPS and related diseases; without having to leave Northern Ireland or attend multiple appointments. The MPS Society plays a key role in funding, organising and coordinating these clinics; as well as providing patients with a point of access to the support and advocacy service provided by the Society. The MPS Society also provides follow-up support to patients and families. Importantly, these clinics also provide patients and families with the opportunity to meet other families with similar experiences of living with MPS.

This service is now also being provided at three monthly joint cardiac and genetics Fabry clinics.

Produced by Rare Disease UK

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