

SPECIALISED HEALTHCARE ALLIANCE BRIEFING
ADJOURNMENT DEBATE ON DEVELOPING A UK STRATEGY FOR RARE DISEASES
30TH APRIL 2012

The Specialised Healthcare Alliance is a coalition of 76 patient-related organisations, supported by 11 corporate members, which campaigns on behalf of people with rare and complex conditions. Although high quality specialised services matter to the population as a whole, they are particularly important to people with rare diseases. While the Alliance welcomed the opening of a consultation on a UK Plan for Rare Diseases by the Department of Health on 29th February 2012, we are concerned that the proposals presently lack ambition and fall short of the required vision. A strong UK Plan for Rare Diseases would have the potential to provide an effective means of improving care for the large number of people with less-common conditions whose interests can often be overlooked in the context of a locally-focused NHS. The current rare disease plan consultation will need significant improvements if it is to meet this standard.

Key principles for a rare diseases strategy

A rare diseases strategy should establish core principles for the treatment of people with rare conditions across the UK. The UK's shared vision could start from the premise that the NHS should leave no-one behind because their condition is rare and therefore relatively more expensive to treat. We would like to see prominent recognition for three key principles in the plan to be elaborated in each of the home countries. First, it is vitally important that there is **clear accountability** for specialised services at all levels of the NHS. In particular, patients and their families should be able to see who has lead responsibility for their service without being pushed from pillar to post. Secondly, **access to services** should be equitable wherever you live and, given the size of the UK and scarcity of clinical expertise and resources, this will often have to be planned across borders or, indeed, at European level. Thirdly, there needs to be a relentless **focus on outcomes** achieved by services as part of a feedback loop supporting continuous improvement.

Proposals

In a number of specific areas we would urge more concrete action from a UK Plan for Rare Diseases, going beyond that which is currently included in the consultation document. In 2011, the Specialised Healthcare Alliance produced a report entitled *Leaving No-One Behind*, which is cited in the consultation document, and which sets out a number of recommendations to improve the co-ordination and delivery of specialised services in all parts of the UK. The Alliance's report built on the sensible proposals on rare diseases put forward in the Chief Medical Officer for England's report for 2009, and the earlier Carter Report on Specialised Commissioning in 2006. It is important that the UK Plan for Rare Diseases sets out concrete proposals for implementation.

In particular, we would urge:

- The appointment of a **national clinical director** for specialised services, including rare diseases, in each of the home nations. This individual should have explicit responsibility for implementing the plan and for leading in areas like professional training and the development of networks.
- The **creation of a strategic clinical network** or clinical senate for specialised services. This would address a longstanding problem, where the requirements of specialised services and people with rare diseases go unheard in the context of more general forums.

- The **retention of the Advisory Group for National Specialised Services**, which advises Ministers on new highly specialised services, products and technologies using its well-regarded ethical decision-making framework. People with very rare diseases in all four nations benefit from these services, which often have wider strategic importance.
- **Patient registries** for specialised services, to ensure that good data is held in the right way, benefitting the planning and co-ordination of specialised care and empowering patients.
- **HealthWatch England should be responsive to people with rare diseases**, with a clear duty to gather the views of users of specialised services and to represent them to the NHS Commissioning Board.

Implementation and ambition

The Specialised Healthcare Alliance acknowledges the difficulty of producing an effective Plan for Rare Diseases given the divergent approaches to the NHS across the four UK nations. Each nation should therefore commit to producing its own implementation plan after the final publication of the UK Plan for Rare Diseases, to ensure that measurable objectives are set out to aid effective execution of the plan at national level.

A shared vision embedded in a Rare Diseases Plan, with proposals as outlined above and subject to national implementation, could set a strong framework to help drive up standards of care for people with rare conditions across the country, and ensure no-one is left behind.