



Rare Disease UK's response to the National Quality Board engagement exercise on the library of NICE Quality Standard NHS healthcare topics

Are there any significant gaps or omissions in the proposed library? If so, what are they?

Rare Disease UK (RDUK) is concerned that a system of Quality Standards which is largely geared towards prevalence of conditions, hospital activity, and the existence of NICE clinical guidance will not effectively drive quality care for patients with rare diseases. Whilst some of the proposed Quality Standards explicitly relate to rare diseases (e.g. motor neurone disease, cystic fibrosis) and some of the cross cutting quality standards could relate to rare diseases (e.g. long term conditions, managing symptoms with and uncertain cause, end of life care for infants, children and young people), it is unlikely that these will adequately capture the over 6,000 known rare diseases which will affect approximately 3.5 million people in the UK at some point in their lives. As a result, it is important to address how standards can be developed which benefit this considerable patient population.

The importance of Quality Standards in improving outcomes, driving up the quality of care and as "the backbone of the commissioning system" is reinforced repeatedly in this consultation document and in *Developing the NHS Commissioning Board*. RDUK is eager to know what will be driving this improvement for patients with rare diseases which the proposed Quality Standards do not adequately capture. This is especially so as, patients affected by rare diseases may access specialised services for elements of treatment and care, but the majority of day-to-day care will be delivered locally. In the absence of Quality Standards, what will be used by clinical commissioning groups and the NHS Commissioning Board to drive quality for these patients?

Are there any other cross-cutting topics that address health improvement and/or patient experience issues for which a Quality Standard would be of benefit?

Despite the fact that there are over 6,000 rare diseases, each with different symptoms and prognoses, the issues faced by patients in accessing quality care are often the same no matter what the condition. RDUK recognises that Quality Standards are largely drawn from current evidence available in the forms of NICE guidance and other sources accredited by NHS Evidence and there is likely to be little evidence of this sort about rare diseases. RDUK also accepts that it would be impossible to produce Quality Standards for each rare disease, the majority of which little will be known about. As a result, RDUK recommends that the National Quality Board investigate the possibility of producing generic Quality Standards for rare diseases. These could be broken down into paediatric/adult/elderly/end of life care, or Standards could be framed in terms of pathology; the proposed Quality Standard "Neurological problems (relatively uncommon neurological problems e.g. muscular dystrophy)" alludes to this, however, issues may arise as many rare diseases are multi-system and would span more than one Quality Standard.

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

The production of the library of Quality Standards should not be the end point. The process of developing the library has the potential to highlight disease areas where information and evidence is lacking and pro-active moves should be made by NICE to address these gaps.

October 2011

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