



Chair: Allyson Kaye MBE | CEO: Jane Lyons

Board: David Ryner, Helen Jameson, Clara Mackay, Helen Morement, Kathy Oliver, Rebecca Porta, John Solly, John Symons

The UK Strategy for Rare Disease: making sure rare cancers are not written out of the script

A Cancer52 Position Statement

February 2014

Background

Rare diseases affect some [3.5 million people](#) across the UK.¹ And those diagnosed with less common cancers are part of those affected: in 2011 over 150,000 people were diagnosed with a less common cancer.²

Rare diseases can have a dramatic impact on those diagnosed and their carers: their quality of life; their hopes for the future; and rare disease can sadly result in early death. Many people with rare diseases face problems: it's hard to get a diagnosis, a misdiagnosis is more likely, it can take a long time, and it can be difficult to get access to the right specialists and treatments. It's difficult too for the NHS across the UK to meet patients' and carers' needs, especially when in some cases a General Practitioner (GP) might only see one case of a particular rare disease in their career. Even clinicians need support in order to deliver care, and conduct research to bring about better treatments.

First Ever UK Strategy for Rare Disease

For the first time the four devolved nations of the UK have come together to set out a [UK Strategy for Rare Diseases](#). This is part of meeting [European Commission requirements](#), specifically "to ensure that patients have access to high quality care, including diagnostics, treatments, habilitation for those living with the disease and, if possible, effective orphan drugs". Whilst the Strategy is part of meeting the EC requirements, it shouldn't be seen as a box ticking exercise. It's been a huge effort to bring together the four nations and to develop the Strategy.

The Strategy includes 51 commitments across 5 main themes: empowering those affected by rare diseases, identifying and preventing rare diseases, diagnosis and early intervention, coordination of care, and the role of research. Cancer52 believes that cutting across the whole Strategy is the fundamental issue of research. Our members [directly fund](#) over £7million on cancer related research, close to 90% of this is specifically related to less common cancers. 21 commitments in the Strategy relate to research and our Members know just how important it is for us to work together, for patients to help design and take part in research, and to strip out bureaucracy.

Cancer52 believes that some of the commitments in the Strategy are particularly **important for those with less common cancers**:

¹ There are different definitions for 'rarity' and that can also be a challenge when planning and delivering services.

² Less common cancers are [defined](#) as those cancers that are not breast, colon and rectal, lung or prostate cancer. [46% of the 331,487 new cases of cancer](#) were for these cancers across the UK in 2011.

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the common voice
for less common cancers

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UK Rare Disease Strategy Commitment	Why is it particularly important for less common cancers?
Commitment 1: “strengthen the mechanisms and opportunities for meaningful and sustained patient involvement in rare disease service provision and research, recognizing patient groups as key partners – including in the development of the four country plans to implement the Strategy”	Some organizations that represent those with rare cancers are really small: they often struggle to get their voice heard. If the Government delivers on this commitment this should change
Commitment 10: “initiate action to ensure carrier testing approved by the appropriate commissioning bodies.....is accessible for at risk relatives”	Some inherited gene mutations can increase the chance of getting a less common cancer. With that knowledge, those affected can take risk reducing decisions for them and their families
Commitment 11: “work to achieve reduced times for diagnosis of rare diseases”	Late diagnosis, often in an emergency setting, is more likely to be the experience of a patient eventually diagnosed with a rare rather than a common cancer. We support efforts to improve assessment at entry (GP) level with decision aids and the like, just as we support swift onward referral, preferably to a specialist oncology centre, to ensure timely commencement of treatment. We are concerned that the number of patients waiting longer than the waiting time limit for treatment to begin following an urgent referral has grown rather than diminished since the publication of the government’s key cancer strategy document and suspect a disproportionate number of them will, as with diagnosis in an emergency setting, be those diagnosed with a rare cancer.

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UK Rare Disease Strategy Commitment	Why is it particularly important for less common cancers?
Commitment 13: “ensure that there are appropriate procedures for evaluating the costs and benefits of treatments for patients”	It can be hard to access some medicines for less common cancers because it’s hard to show their benefits in a way that really reflects patients' and carers' experiences, and there is scope to improve on how these decisions are made
Commitment 23: “ The generic care pathway will include: an appropriate care plan for all patients with a rare disease...”	The National Cancer Patient Experience Survey 2011/12 data analysis concluded that only 24% of patients said they had been offered a written assessment and care plan. We would want to see a care plan provision commitment in the Plans of all four nations.
Commitment 38: “work with the research community, regulators, providers of NHS services and research funders to develop risk-proportional permission systems”	Some less common cancers have treatments in development and some patients who have little other hope want to take part in trials but can’t because many agencies are too risk averse

From a UK Strategy to National Implementation Plans

The next stage is for the devolved nations to set out their detailed national implementation plans, due to be published on Rare Disease Day (28th February 2014). It might be better if we were to call them action plans, just to help maintain the focus not just on planning, but *doing* what it takes to make things better for those with rare diseases. And in the spirit of the Strategy, these should ensure alignment across the UK of key phases of implementation, otherwise synergies will be lost. This might be hard to do, but it’s what is right for patients with rare diseases.

We also believe that there are rare diseases that are so rare that there should be a single UK approach: a single UK centre of excellence to diagnose, monitor and deliver treatment. That might not fit with devolution, but again it is what is right for those patients. We want to work with the 4 countries to identify the prevalence rate that should trigger this single UK approach, and pool funding for those services and treatment. This would also be in the spirit of the UK National Screening Committee (cited in the Strategy on p13), a British Paediatric Surveillance Unit (cited in the Strategy on p26) and the UK Genetic Testing Network (cited in the Strategy on p18). So we know a single approach can be done across the UK.

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It's also a chance to improve on some of the commitments in the Strategy. Cancer52 believes that some commitments are too weak. For example: Commitment 5 is to '*Consider how to give all patients with a rare disease clear and timely information about: their condition and its development; treatment and therapy options; practical support*'. We would be disappointed if this was only considered and that change didn't happen.

Cancer52 is asking for:

- The Government to focus on action, not words and make swift progress on implementing the Strategy. **Progress reports should be published every year** and the **Health Select Committee** (and respective Committees across the UK) **should run annual inquiries** to hold each country to account
- **Maintain the focus on Rare Diseases** despite all the pressures that the NHS is under: it might be hard, and it might take time, but the benefits will be for millions of people and their carers across the UK. Commitments must not be abandoned as we've seen with the Specialised Commissioning Innovation Fund. Other initiatives need to bear fruit, such as the currently unfunded and opaque Commissioning through Evaluation initiative.
- **Less common cancers to be clearly identified in all work relating to the Strategy.** No mention was made of rare cancers, or indeed cancer, in the consultation on the UK Strategy. Now there is a single reference to cancer across the 35 pages of the Strategy, but only in reference to the NHS England 100,000 Genomes Project. Less common cancers lead to more than half of all cancer deaths – getting it right for those with less common cancers must not be overlooked.
- **Not lose sight of the importance of access to treatments.** This was one of the key elements of the European Commission's requirements and yet little more than half a page in the Strategy covers these issues.
- To work with patient and carer organizations to help implement, evaluate and improve; research, shared data collection, diagnosis, access to services and treatments for those with rare diseases. The existing Department of Health Stakeholder Forum Group will move into a monitoring role now that the Strategy has been worked up. But, with responsibility for implementation now resting with the four nations, we want each department of health across the 4 nations to set up **stakeholder groups and publish their Terms of References** and, as part of annual progress reports, publish the views of stakeholders. In England, we want NHS England to host the stakeholder group.

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