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Cancer52 publishes briefing paper on "Orphan" Medicines

Making reasonable decisions about access to "orphan" medicines in the UK More deliberation and less maths

Cancer52, an alliance of nearly 80 predominantly patient support charities working in the field of rare and less common cancers, is today (5 June 2014) publishing its [briefing paper](#) on access to orphan medicines in the UK.

Orphan medicines treat diseases that affect no more than 5 in 10,000¹ people in Europe. Rare cancers make up close to 40% of orphan medicines and more than 80 per cent of Cancer52's member organisations² work with rare cancers.

Generating access for patients to orphan medicines is therefore particularly challenging because of the small numbers involved. Difficult decisions need to be made about the benefit and risks of orphan medicines as the robust evidence that regulators and the NHS wants is not always available. Cancer52 supports the move to change the way that access is determined and says that decisions need to take into account the special circumstances faced in treating rare diseases, including cancers.

Cancer52 is asking for:

- **Full and open approach to evaluation of changes.** This should include how far proposals/changes in appraisal affect research priorities, including research for those with rare and less common cancers.
- **A debate on the merits of a single UK wide approach to appraising ultra orphan medicines**
- **Faster decisions** from agencies – patients can't always wait the time it takes for recommendations to be made

Says Clara Mackay, interim chair of Cancer52, "The current process for making orphan medicines available to patients is overly complicated, too financially driven and has too many agencies involved. It is patients who are currently paying the price for this and we want to shift the emphasis from doing the sums to deliberating more on the patient focus. As an alliance of nearly 80 charities we are willing and well placed to work with all stakeholders to make this happen. There is clearly an unmet need for orphan medicines as more than 80,000 people died of a rare or less common cancer in the UK in 2011.³"

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Further information

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¹ A rare disease is a life-threatening or chronically debilitating disease that affects 5 people or fewer in 10,000 and requires special, combined efforts to enable patients to be treated effectively.

² Source Cancer52 member survey February 2014

³ More than 84,300 people died of a rare or less common cancer in the UK 2011, accounting for 54% all of cancer deaths³. 46% of cancers diagnosed are rare and less common cancers³.