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We are an alliance of brain tumour support, advocacy and information groups around the world, including brain tumour patients and caregivers, researchers, scientists, clinicians and allied health professionals who work in the field.

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**Second Parliamentary Summit on Rarer Cancers -Thursday, 19<sup>th</sup> June 2008**

**Orphans for Adoption:  
NICE and the commissioning process for orphan cancer medicines**

By Kathy Oliver, International Brain Tumour Alliance (IBTA)

We are witnessing a time of unparalleled discovery in the struggle to find better treatments and ultimately a cure for cancer.

There is unprecedented progress in refining surgical approaches. Novel, targeted therapies and combination therapies showing promise are emerging.

The Cancer Genome Atlas is looking at what happens when the code of life is hijacked by aberrations and cancer occurs. Indeed, the first three cancers being studied in this endeavour include two of the less common ones: ovarian cancer and glioblastoma multiforme brain tumours.

And thanks to European legislation on orphan medicinal products which creates incentives for the development and placement of orphan drugs onto the market, pharmaceutical companies at long last are showing interest in developing treatments for small population cancers.

We stand eagerly and hopefully on the threshold of a very exciting time in cancer treatment.

As we heard so eloquently and movingly this morning from patients' evidence, those who have a rare cancer, love and care for someone who does or those of us who represent a rare cancer charity know all too painfully that what actually happens at the point of need is often in stark contrast to the excitement of taking a new therapy from bench to bedside.

Ensuring access to these new treatments is critical. Nowhere is it more controversial than in the role which Health Technology Assessments play in approving or rejecting health system reimbursement for orphan therapies to treat rare cancers.

A recent European Observatory report on Health Technology Assessment said these appraisals play a valuable role in healthcare decision-making, but the process must be transparent, timely, relevant, in-depth and usable. It must also honour the basic human rights of equity, access and choice. It should also facilitate patient representative input.

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Our own National Institute for Health and Clinical Excellence is often seen to be at the heart of denying patients access to life-extending treatments.

NICE has a tough job. But at the same time as it's being criticised, it's often held up as an example for other countries to emulate. Indeed, patient groups welcome NICE's initiatives on "Improving Outcomes Guidance", the Citizens' Council and examining philosophical issues like the Rule of Rescue and the Social Values Judgement. Why NICE rejected the Rule of Rescue in its second draft of the Social Values Judgement puzzles us. However, that's another topic for another time.

But with the emergence of expensive new orphan drugs to treat patients with rare cancer, very serious concerns and criticisms have arisen from a system which increasingly appears to be creating an atmosphere of growing despair, nihilism and hopelessness.

We can't blame NICE for **all** the problems we have in accessing orphan drugs. NICE doesn't operate in a vacuum and there are other influencing issues.

But patients with a rare cancer have no sympathy with the oft-repeated mantra: "We only have a limited pot of money and so cannot help everyone." When patients read of a twelve billion pound NHS computer system that is six years in the making and still doesn't work, they get angry. When patients read of a two billion pound "underspend" by the NHS this year and they have to battle to receive cancer drugs, they are filled with dismay.

And as of 48 hours ago I was going to also say that when patients do scrape together the money to pay for a non-NICE-approved therapy, and they are then denied the remainder of their care on the NHS, people begin to question what kind of warped value society places on their humanity when it denies them the right to try to survive, even if that survival may only be for a matter of months.

But as we all now probably know, a fundamental re-think on the issue of "co-payments" or "top-ups" was ordered on Tuesday by Health Secretary Alan Johnson who has asked Mike Richards to look at this issue and report back to him in October.

Therapies for rare cancers don't come cheap. They don't easily fit into the regulatory system. We think it's time for NICE to stop making macro decisions based on an inappropriate and blunt instrument like the QALY. We need a new, separate process whereby criteria for orphan drug reimbursement approval on the NHS takes into account the severity of the rare cancer in question; the therapy's clinical efficacy; the issue of unmet need where there is no satisfactory alternative; the **realistic** total cost to the NHS and vitally, the true value to society of a human life - even though that life may be tragically cut short.

As Dr Viktor Frankl wrote: "We cannot judge a biography by the number of pages in it; we must judge by the richness of the contents."

The length of time it takes NICE to issue appraisals is another concern. We appreciate that the new single technology appraisal process addresses that to some degree. But the time frame is still not optimal.

Perhaps the freedom to be more innovative is needed here? For example, Thomas Lonngren, executive director of the European Medicines Agency, recently said the emergence of health technology institutes like NICE posed a challenge for drug manufacturers because these bodies often required additional research. He even suggested there might be scope for closer cooperation with the EMEA in designing drug approval programmes in the future.

Perhaps QALYs could be weighted so as a disease becomes less common a higher cost per QALY is used. Risk-sharing programmes and ring-fenced funding are other possible options.

The QALY also doesn't take into account the financial burden to the state which is removed when a caregiver is involved.

Sometimes patients with a rare cancer who have access to cutting-edge therapies are able to return to work and have an excellent quality of life. One of these examples is the chemotherapy temozolomide for newly diagnosed high grade glioma brain tumours. We know of patients who have malignant brain tumours and who have accessed this therapy. They have been able - for a time - to return to work and to be fully participating and contributing members of society.

Of course, despite these types of treatments being available, they are sadly not a cure. But even a survival benefit of a few months of normality - working, contributing to the human race and giving back rather than taking - has to be worthy of reimbursement on the NHS.

From our perspective there are other big issues.

How will new, expensive combination therapies be dealt with by NICE?

In addition to the Department of Health Good Practice Guidance on the introduction of new healthcare interventions appraised by NICE, how can it be made even clearer to PCTs that in the absence of NICE guidance, or while an appraisal is being undertaken PCTs should rely on clinical evidence to inform their decision regarding access? If a therapy is not yet "NICED" patients should not be denied treatment for that reason.

With the current focus of governments and major cancer control organisations on improving cancer outcomes through prevention, screening and lifestyle changes - which incidentally have no relevance at all for many rare cancers - how can NICE help ensure that an equal focus of attention is put on access to therapies? This is frequently the only hope for patients with a rare cancer.

You cannot truly understand the devastation of a rare cancer until it directly touches your life. I had no idea of what this journey was like until my then 24-year-old son was diagnosed with a brain tumour. But I hope that the last five minutes have enabled a greater intimacy with our fears as a rare cancer community. This ludicrous inequity for accessing cutting edge therapies for rare cancers must be consigned to history.

Orphans need to be happily adopted - by pharmaceutical companies, by licensing and regulatory authorities and importantly, by health technology assessors like NICE.

Please, give orphans a home and give those suffering from the devastation of a rare cancer at least a chance for extended survival.

Thank you.