

Lord Turnberg to ask Her Majesty's Government what action they are taking to improve access to treatments for patients with rare diseases – 3rd March 2015

Briefing by the Specialised Healthcare Alliance

The Specialised Healthcare Alliance is a coalition of 110 patient-related organisations supported by 15 corporate members, which campaigns on behalf of people with rare and complex medical conditions.

The subject of access to treatments for patients with rare diseases has many dimensions, including the MHRA's early access to medicines scheme, NICE's approach to appraisal of orphan and evaluation of ultra-orphan medicines through its technology appraisals and highly specialised technologies programmes respectively, and the Cancer Drugs Fund.

The SHCA, however, considers that the most urgent problems relate to NHS England's serially delayed prioritisation of commissioning policies for treatments outside these other processes, which therefore provides the focus of this briefing.

Because of this process delay and the consequences of NHS England's recent mistakes, NHS England has not been able to provide access to new treatments and services for patients with rare diseases for some months, with the delays set to continue until well into the summer unless action is taken.

Background

NHS England is the sole commissioner of specialised services prescribed under the Health and Social Care Act 2012. As such it is in a position to provide uniform access to treatments for people with rare diseases but needs to have robust and transparent processes for doing so based on ethical principles.

In September 2011, an initial draft framework and policies were discussed but rejected, in part because of the need for greater engagement with stakeholders. Despite serial efforts by the Alliance, such engagement failed to occur and essentially the same policies were presented NHS England's Clinical Priorities Advisory Group in March 2013. Given the need to provide a legal basis for NHS England's decision-making from 1st April 2013, the policies were reluctantly agreed on an interim basis on the strict understanding that a proper review would be completed by October 2013. Again, this failed to occur, with the Chief Executive of NHS England apologising for the delay in a letter to the Chair of the Specialised Services Patient and Public Engagement Steering Group in August of the same year.

During the course of 2014, a series of workshops were belatedly held to consider the principles that might inform a scorecard to support decisions about which treatments should or should not be funded by NHS England. The expectation was that a revised interim framework would be published in September as the basis for prioritisation in 2015/16 and for formal consultation during the course of this year.

Use of the interim framework and scorecard was, however, legally challenged on behalf of a 10 year old boy with Morquio A syndrome, causing NHS England to halt the prioritisation process for all patients in December.

Current position

The Board of NHS England commenced a 90 day consultation on revised principles to inform funding decisions in late January. The scorecard has been dropped pending further notice. The consultation will close in late April, making it unlikely that NHS England will decide on patient access to a large number of treatments until July.

Immediate need

As a consequence of this inexcusable situation, there are cohorts of patients who have been left in clinical limbo for approaching two years and who now face the prospect of substantial further delay.

NHS England typically cites its interim policy on Individual Funding Requests as a potential solution for such patients and their treating clinicians. This is misleading, as the IFR policy is specifically designed to identify cohorts of more than 20 patients across England and, having done so, effectively denies such patients access to treatment until a policy has been agreed – the self-same policies affected by the current delay.

Accordingly, the Alliance is urgently calling upon NHS England to make treatment available to patients in urgent clinical need outside established policy. NHS England has established precedent for this approach based on the principle that if a patient is presenting as clinically urgent the patient should be treated and any funding decisions take second place to the clinical needs of the patient. In circumstances of its own making, this seems the least that NHS England should do with immediate effect.

Permanent solution

Having addressed the immediate need, NHS England must avoid a repetition of these problems by developing **a clear and expeditious process for future policy development, alongside principles that recognise the particular circumstances of rare diseases**, drawing on the framework previously drawn up by the Advisory Group on National Specialised Services (AGNSS).

SHCA
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