NICE and NHS England changes to technology appraisals and highly specialised technologies

Summary of All Party Parliamentary Group for Muscular Dystrophy meeting

“Everyone knows that money in the NHS is tight, but delaying access to life-extending drugs only hurts boys like Charlie without actually addressing the cost of drugs. Another muscular dystrophy drug, Translarna, was made available to patients in a special five-year test arrangement. We need an agreement like that for Exondys 51, not bureaucratic hurdles that will cost us valuable time with our son Charlie

Claire Liggett’s son, Charlie, has Duchenne muscular dystrophy
Background to the consultation

Between 13 October 2016 and 13 January 2017, NICE and NHS England conducted a joint consultation on changes to technology appraisals and highly specialised technologies. This featured proposals to introduce budget impact thresholds to delay implementation of medicines which would exceed £20 million of costs to the NHS. Patient groups, including Muscular Dystrophy UK, raised serious concerns that these and other proposals in the consultation were likely to disadvantage patients needing access to high-cost treatments for rare diseases.

Recent years have seen a positive step change taking place in the therapeutic approaches being adopted to treat rare diseases. It is to be welcomed that the prospect of therapies has become a reality for many hitherto untreatable conditions, such as the following drugs for muscular dystrophy and related neuromuscular conditions:

- Translarna to treat some patients with Duchenne muscular dystrophy caused by a nonsense mutation was approved by NICE in July 2016.
- Exondys 51 and Raxone (both to treat groups of patients with Duchenne muscular dystrophy) are being reviewed by the European Medicines Agency (EMA).
- Nusinersen, to treat spinal muscular atrophy (SMA), is also being reviewed by the EMA and is currently the subject of an Expanded Access Programme for eligible infants with SMA Type 1.

Whilst extremely welcome, this does present challenges to the NHS and to NICE. Drugs can often emerge in quick succession, adding pressure to review capacity and to short term budget cycles.

The consultation therefore took place in the context of a number of treatments being deemed cost-effective by NICE, which placed some additional pressure on NHS budgets. In some cases, NHS England requested a longer implementation period and was not able to make the drug available to a broad number of patients. In the case of Translarna, the drug is now available to patients but NHS England did require more time for discussions with the drug company, PTC Therapeutics, following initial NICE guidance.

What have NHS England and NICE now announced?

Following the close of the consultation, NICE and NHS England announced on 15 March that they plan to move ahead with a series of changes to technology appraisals and highly specialised technologies. Many of the original proposals from the consultation were retained, and will come into force from 1 April. These include:

- Inviting NHS England to agree special funding arrangements with drug companies where a NICE approved drug would exceed £20 million of costs for the NHS in any one of its first three years
- Giving additional time to introduce a new drug which exceeds the £20 million threshold and where the funding of other services could be disrupted
- Introducing a maximum threshold of between £100,000-£300,000 per quality adjusted life year (QALY). This will be determined by the life-long impact of the drug. In light of consultation responses, NICE and NHS England did not go ahead with proposals to pass drugs going over this threshold to NHS England’s own prioritisation process.
It is important to recognise the budgetary pressures in which NHS England is operating and the need to secure best value on new treatments.

However, these proposals could work against fast access to treatments for rare diseases, such as muscular dystrophy.

- The £20million threshold is too low– and would have impacted upon 20% of NICE approved drugs last year. There is also lack of clear rationale on the £20million figure

- The £100,000-£300,000 per QALY figure appears arbitrary and may adversely affect patient access to treatments for rare diseases with high unmet medical needs

- Negotiations with companies should take place at the earliest stage possible so they don’t delay patient access to treatment

- Implementation of the plans on 1 April has not left enough time to explore all alternative options – for example, developing innovative new approval models which can enable patient access to new drugs, gather more evidence and achieve better value for the NHS. The Managed Access Agreement for Translarna provides a good model that could be developed further

- NHS England needs increased support and capacity to negotiate special funding mechanisms with drug companies
Summary of APPG meeting for Muscular Dystrophy meeting on Wednesday 29 March

Attendees:
Mary Glindon MP, Member of Parliament for North Tyneside, Chair of the APPG for Muscular Dystrophy
Baroness Thomas of Winchester, Vice Chair of the APPG for Muscular Dystrophy
Jim Shannon MP, Member of Parliament for Strangford
Vernon Coaker MP, Member of Parliament for Gedling
Justin Madders MP, Shadow Health Minister, Member of Parliament for Ellesmere Port and Neston
Steve McCabe MP, Member of Parliament for Birmingham Selly Oak
Charlie Hawken, Parliamentary Researcher, Office of the Rt Hon Cheryl Gillan MP
Robert Meadowcroft, Chief Executive, Muscular Dystrophy UK
Peter Sutton, Campaigns and Engagement Manager, Muscular Dystrophy UK
Tanvi Vyas Brady, who has spinal muscular atrophy
Claire Liggett, whose son, Charlie, has Duchenne muscular dystrophy
Gary and Louisa Hill, whose son, Archie, has Duchenne muscular dystrophy
Manoj Thakrar, whose son, Shiv, has Duchenne muscular dystrophy
Emily Crossley, co-founder, Duchenne UK
Diana Ribeiro, Chief Executive, Action Duchenne
Doug Henderson, Managing Director, SMA Support UK
Louise Coleman, Policy Officer, Genetic Alliance UK

- Mary Glindon MP opened the meeting, and welcomed Sheela Upadhyaya from NICE. NHS England were unable to provide a representative for have agreed to meet at a later date. A meeting with specialised commissioners is currently being finalised.

- Peter Sutton from Muscular Dystrophy UK gave an overview of the changes NICE and NHS England have announced to drug approvals. He outlined the potential impact on drugs for rare diseases, which often have a higher cost per patient and may struggle to meet the £100,000-£300,000 per QALY threshold announced by NICE. The Managed Access Agreement for Translarna provides a good model to develop – and it is important that NICE and NHS England work with companies to develop risk sharing arrangements, or pricing based on a drug’s performance in clinical practice. Delays in access to treatments can be devastating for patients with muscle-wasting conditions.

- Justin Madders MP questioned whether negotiations with drug companies will be effective in lowering the price of drugs. Sheela Upadhyaya (SU) said there are clear examples of when the NHS and drug companies have negotiated prices down.

- Gary Hill highlighted delays in access to Translarna and said that it had been difficult for the drug company to find a clear point of contact at NHS England. SU said the current process is too long and has a lack of clarity. The process is subjective and there is a challenging financial situation in the NHS. The new process will enable companies to engage early with NHS England if the drugs exceeds the budget impact trigger of £20m in the first three years of introduction to the NHS.
SU said due to this the consultation was held – and that the new process does include a fast track of drugs at £10,000 per QALY (although this is not necessarily relevant to rare disease drugs). She stressed that the £20 million is a trigger for companies to be able to have early discussions with NHS England. £20m is therefore not the maximum amount that the NHS will ever pay for a new drug. There can and will be circumstances where it is appropriate for the NHS to pay more in order to make new treatments available for patients. The drugs will still be evaluated by the NICE HST process, but an agreement between the company and the NHS would need to be struck, if the above situation applies. The budget impact trigger would not have affected 80% of approved technologies in the last year (although it would have impacted on 20%).

Manoj Thakrar told the group that the trigger point was the wrong way round – the focus should be on early discussions prior to approval, not delaying access post approval. SU acknowledged this and confirmed that this is why the trigger has been put in place at the beginning of the process.

Baroness Thomas questioned how long negotiations would take – and stressed patients cannot afford delays. SU said negotiations would need to be conducted and concluded within a maximum 12 week pause period.

SU said the changes announced are about providing clarity in the system. It was felt that the HST thresholds may prompt a more attractive offer from pharma. NICE and NHS England had listened to feedback in the consultation and withdrawn the proposal that drugs exceeding the £100k QALY threshold would be passed to NHS England’s clinical prioritisation process.

Steve McCabe MP questioned whether secret deals with pharma were desirable – a lack of public scrutiny could mean a 'bad' secret deal for NHS England.

Diana Ribeiro from Action Duchenne stressed that current Duchenne treatments may struggle to meet the new QALY thresholds – the higher QALY thresholds would apply to drugs at the curative end of the spectrum.

Emily Crossley from Duchenne UK questioned how this new paradigm will be managed. SU emphasised that the impact of the changes is not yet fully known – and that this will be reviewed and monitored. Many rare disease treatments do not go through NICE and instead go through NHS England,. NICE continues to focus on the value, not cost, of a drug. Patient testimony and experiences will still form a key part of the HST evaluation.

Vernon Coaker MP asked for an update on Exondys 51, on behalf of his constituents, the Roe family. SU informed him the drug had been through the HST scoping process and a decision on the European Medicines Agency licence is awaited.

SU emphasised that risk sharing arrangements and other innovative methods of approval are not off the table. Pharma needs to work with the NHS and patients and keep costs at a manageable level.