January 2022

NICE Systems Review

Innovative Medicines Fund

Spinal Muscular Atrophy UK (SMA UK)

Principle 1: The Innovative Medicines Fund should operate as a managed access fund for non-cancer medicines so that any patient, regardless of their condition, has equal potential opportunity to benefit from promising but uncertain medicines.

The SMA community has significantly benefitted from innovative drugs that have been made accessible through Expanded Access Programmes (EAP) and Managed Access Agreements (MAA). We acknowledge the lifesaving impact that these schemes, however, our experiences have been ones of long timelines to achieve these and of partial access to some groups. We therefore welcome an organised pathway that will potentially allow more patient cohorts to benefit from faster access to new life saving treatments. Will the IMF route replace the specialised and highly specialised technology evaluations?

We would like assurance that the IMF budget will not be overwhelmed by medications for conditions that have a higher prevalence in the population. It could be argued that such medications are better value for money than some of those for the rare disease communities. For example, Zolgensma™ gene therapy, with a list price of £1.79 million per dose, has repeatedly been reported in the media as 'the most expensive drug in the world'. But for the intrathecal drug Spinraza™, over 5 years, which is the maximum managed access timeframe, "the treatment costs per person is £1.35 million pounds" (NICE;2019;2) [1]. The clinical benefits of these drugs have been lifesaving. 340 million per year for the IMF sounds like a generous fund, but will it be enough to ensure that expensive health technologies for rare diseases get this "equal potential opportunity". Should the budget be more flexible and linked to ILAP horizon scanning?

Principle 2: Clear and robust criteria should ensure that the Innovative Medicines Fund targets the most promising medicines for which there is significant remaining uncertainty around the level of clinical benefit.

We are encouraged by the inclusion of this principle and the potential for it to benefit the rare disease communities. Given the small populations there is less data. Even from worldwide clinical trials, data collection is more bespoke, and outcomes take longer to assess. If the IMF can target medicines like this which have huge prospective clinical efficacy, then it will change the landscape for rare disease technologies and the speed of their impact on the 1 in 17 of the population that are affected by them.

[1] NICE final appraisal document 'Nusinersen for treating spinal muscular atrophy' 2019

Principle 3: Innovate Medicines Fund recommendations should be reserved for medicines that (a) demonstrate plausible potential to be cost-effective; and (b) are priced responsibly during the period of managed access, reflecting their uncertain clinical benefit

Establishing value for money is never straightforward for rare disease health technologies. We would like assurance that when evaluating cost-effectiveness for the IMF, NICE will continue to evaluate with different criteria, ensuring patient consultation and will continue to work to strike landmark deals with pharmaceutical companies as they did for Zolgensma™. "The vulnerability of very small patient groups with limited treatment options, the nature and extent of the evidence, and the challenge for manufacturers in making a reasonable return on their investment because of the very small populations treated. In evaluating these drugs, NICE takes into account a greater range of criteria about the benefits and costs of highly specialised technologies than is the case with its appraisals of mainstream drugs and treatments." (Tom Powell, Michael O'Donnell;2019;5) [2].

We would also like clarity on how administrative costs are included in the cost-effectiveness assessment. Drugs which require specialised techniques of administration such as intrathecal procedures will come with heavier administrative costs that may not be covered by the 2% of the IMF that is being put aside.

If companies are paying a 'proportional' cost of data collection, what is this proportion and how is it calculated? Data collection for rare disease technologies can involve costly and time-consuming outcome measuring and recordings, involving specialist healthcare monitoring. We need assurance that NICE will still work with the company to facilitate the development of an appropriate framework for data collection even in these specialised cases that require specific clinical skills and knowledge and therefore come at higher costs.

Principle 4: Managed access should be for the shortest time necessary to collect the data required to resolve any uncertainties identified by NICE.

Given the specialized nature of rare disease medical research we feel the timescales should be more flexible and take a more individual medicine-centred approach. Given smaller patient numbers, once a trial has collected sufficient data to access the IMF, it may take significantly longer to collect enough data to reliably assess for clinical and cost efficacy. Many orphan technologies are targeted at babies or very young children where the benefits can take many years to come into fruition. Outcome measures can be inconsistent due to the nature of children and best fit should be reviewed over extended periods to ensure reliable data.

[2] House of Commons Library, Debate Pack, Number CPD-2019-0022, 12TH March 2019, NICE appraisals of rare diseases

Principle 5: **The entire eligible patient population, as determined by NICE, should have the opportunity to access medicines recommended for the Innovative Medicines Fund in the managed access period**

We would like to see medicines deemed eligible for an entire population when the treatment addresses the core cause of the condition across all subgroups subject to clinical safety considerations. It has been disheartening to have 5q SMA broken down into ‘types’ when considering eligibility for medicine. There is a continuous spectrum of phenotypes in SMA, so it is not a fair process, and many have missed out on life changing treatments.

Principle 6: **All medicines that enter the Innovative Medicines Fund must be re-evaluated by NICE, who will make final recommendations on whether the treatment should be routinely available on the NHS.**

We fully support this process with the assurance that it will follow the normal NICE appraisal process with engagement from the patient community and their representing bodies.

Principle 7: **Any patient who starts treatment with an Innovative Medicines Fund recommended medicine during the period of managed access should have the option of continuing treatment in the event that NICE is unable to recommend its routine use in the NHS at the point of re-evaluation.**

This is a very reassuring principle, and we are pleased that it has been included. We hope that it does not discourage pharmaceutical companies from agreeing to the ECM, but with smaller patient numbers it should work in favour of the rare disease population.

Principle 8: **The Innovative Medicines Fund should never have to close to potential new entrants**

We welcome this assurance and hope that it will see patient groups in their entirety accessing treatment. We look forward to examining how equity to access across the UK will be assured.