SHARED LEARNING ROUNDTABLE ON MANAGED ACCESS AGREEMENTS

June 2023

Introduction

The diagnosis, management and treatment of rare conditions present particular challenges for healthcare systems due to the high levels of uncertainty associated with the evidence base. Rare conditions tend to affect a small number of people in each country, leading to uncertainties about how many people have the condition (prevalence), how the condition affects them (natural history), as well as how it can best be managed and treated to help them live their lives to the fullest.

The uncertainties associated with rare conditions make it particularly important that people living with the condition and their family carers contribute their views to support informed decision-making by healthcare systems. Every condition needs patient and carer input when understanding the risks and benefits of a new approach to treatment and care, but rare conditions also need input from patients and carers to understand what the condition is and how it affects their day-to-day lives.

The charities and support groups helping those living with rare conditions tend to be small and specialist, due to the low prevalence and relative complexity of the conditions themselves. Many charities are either volunteer-led by the parents of someone living with a rare or ultra-rare condition (juggling their voluntary support for the group alongside their caring and employment responsibilities), or have one or two members of staff focused on fundraising and providing hands-on information and support for affected families. Patient organisations and support groups find themselves in the challenging situation of knowing that their input is vital to support informed-decision making by healthcare systems, but struggling to ensure they have the skills and capacity needed to provide the insight and support that they desperately want to contribute. Larger patient organisations also face challenges with staff taking on additional workload to support technology appraisals, especially if they are involved in multiple at a single time.

Informed decision-making in health technology appraisals

The combination of uncertainties associated with the evidence base and the cost of treatments for rare conditions have a significant impact when clinical-effectiveness and cost-effectiveness is being assessed through a National Institute for Health and Care Excellence (NICE) health technology appraisal (HTA).

If a decision cannot be made at the end of an appraisal because there are too many uncertainties in the evidence base, treatments may go into a Managed Access Agreement (MAA). An MAA allows the treatment to be provided for eligible patients for up to five years while further data is collected. At the end of the MAA period the treatment will be re-evaluated taking into account this new data.

Shared learning roundtable on MAAs

In November 2022, in response to a request for support from a small charity being asked to contribute to its first MAA, Genetic Alliance UK called together a group of its members with experience of MAAs for a shared learning roundtable. The roundtable highlighted that many of these charities and support groups had similar challenges and experiences, the themes of which are outlined below.

These themes have fed into a series of recommendations which have been agreed upon by the group to improve the process of MAAs with respect to engaging the patient community and supporting patient organisations that may embark on these programmes in the future.

Our aim is to provide a constructive contribution to help make it as easy as possible for our small and specialist charities and support groups to provide their vital input and support informed decision-making by NICE and its committees in the future.

Attendees and their experiences with MAAs

Amanda Mortensen **Batten Disease Family Support Association**	Treatment in a MAA since 2019, due to be reevaluated in autumn 2024. Families want to feel in control of the process and reassurance for continuing treatment post review.
Helen Morris, Jonathan Gibson **Metabolic Support UK**	Treatment in a MAA since 2017 for hypophosphatasia, received an extension due to Covid. Interim decision was surprising, only approved for a particular age group. Final committee meeting in December 2022 led to a positive decision and Metabolic Support UK have updated the community on this information.
Liz Ryburn **Spinal Muscular Atrophy UK**	Total of three treatments in MAAs, in collaboration with MDUK. Nusinersen has been in an MAA since July 2019, Risdiplam since February 2021. Zolgensma was reevaluated in February 2023 and received a positive decision. One of the MAAs has been extended to collect further data. Also involved with Karen Facey relating to the iMPACT HTA project, an international research project looking into improving HTA methodology.
Robert Burley, Fay Yorath **Muscular Dystrophy UK**	Currently involved in four MAAs, and on the oversight groups for three of those. The first MAA started in 2018 (Translarna) which has recently received an interim negative decision. Final committee meeting in December 2022 resulted in a positive decision. Other three MAAs are related to SMA therefore working closely with SMA UK.
Sophie Thomas **MPS Society**	Completed a MAA for elosulfase alfa, treating MPSIVA, which received interim negative decisions but was then approved.
Ellie Davies	Currently have four treatments in an interim access agreement, only

Cystic Fibrosis Trust	one of those has been through a NICE appraisal in 2016/17 resulting in a negative recommendation by NICE. After campaigning, NHSE reached an agreement with the company in 2019 for an interim access agreement, based on the uncertainties NICE identified. Recently received NICE scoping consultation documents as these four treatments will be appraised via a multiple technology appraisal (MTA).
Louise Fish (Chair), Nick Meade, Sophie Peet, Rachel Clayton **Genetic Alliance UK**	

Theme 1: Communication with the community

MAAs and technology appraisals both take place over several years and patient communities want to be kept informed of ongoing progress. All members of the group agreed that patient organisations often take on the responsibility of liaising between NICE and the patient community. There is an understanding that patient organisations do have a part to play in this role however, the patient organisations that are involved in MAAs vary greatly in size, experience and capacity. For smaller organisations contributing to the MAA and managing communications with the community can divert limited capacity away from their usual work of supporting families and raising funds to ensure their future sustainability.

Recommendation for NICE: Establish with the patient organisation what skills and capacity they have, and what level of support they may need throughout the MAA.

Some members shared that they have felt frustration over the little information they are able to share with their communities. Particularly in situations where the community is becoming distressed or conflicts emerge from within the community. There is an understanding that some information is necessary to be confidential but a lot of the challenges or tensions within the community come from the lack of information patient organisations are allowed to share. Some members expressed the importance for different patient organisations that are engaged in the same MAA to align messaging and deal with disagreements privately rather than publicly to prevent tensions within the community.

Some members of the group shared experiences where communications from NICE have been sent without context, causing confusion and putting patient organisations under increased pressure. For example, a notification email was sent to all stakeholders but it was expected that there would be a meeting to discuss the results before an all stakeholder email notification.

Similarly, some members gave examples where NICE were late or did not notify them that a national news story, related to the MAA they were involved with, was to be published. These news items often are painted as a positive headline but don't contain a lot of specific information therefore creating a period of confusion for the community as they are unsure as to who exactly may be eligible. Communities therefore reach out to patient organisations for clarification but organisations often have to wait for confirmation from NICE about

what information they are allowed to share. This unexpected influx of queries increases the pressure on patient organisations and can cause a great deal of stress for affected families.

Recommendation for NICE: Ensure clear and timely notification for patient organisations involved in the MAA about when media releases are due to be published, and share the media release under embargo one week prior to publication so the organisation can prepare for its release.

Patient organisations often end up creating materials such as FAQ documents or patient information leaflets to explain what an MAA is, how the process works and what being on a MAA means for patients but organisations do not receive any funding or supporting materials from NICE. Some members shared examples where they were compiling information documents for their community that were reviewed by NICE and 'diluted' to such an extent that there was little valuable information in those documents.

Recommendation for the group and NICE: Compile communications materials that have been used by members of the group during their MAAs to generate a toolkit/template/resource for all patient organisations to use, and explore with NICE PIP programme whether to produce these resources independently or to co-produce them with NICE PIP's support.

Members of the group praised the Public Involvement Programme (PIP) team for their communication and support throughout the MAA process, especially when NICE staff can communicate directly to the community through attending webinars or discussion panels. There was wide acknowledgement that the PIP team are always willing to listen and learn, and some members shared their positive experience of previous recommendations they have made starting to be implemented. Another member shared that the PIP team allowed trustees of their organisation to attend training events designed for patient experts which was very helpful in allowing the organisation to better understand the process and what is expected from patient experts, therefore enabling them to better support patient experts from their community.

Recommendation for NICE: Invite patient organisations representatives to training events held for patient experts so that they can be better prepared to support patient experts from their community through a MAA.

Recommendation for NICE: Produce a step-by-step guide to the MAA process for patient organisations, clearly outlining a plan of what is expected from patient organisations from the beginning. Some of this information could be provided in the format of a recorded webinar available on the NICE website so it can be continually referred to as a resource. The type of information that would be useful for patient organisations to know from the outset include:

- The name of a project manager / main contact from start
- Single point of contact for all communications (senior level)
- Clear roles and responsibilities of all stakeholders
- What information is expected to be gathered for the re-evaluation.
    - The Managed Access Oversight Committee (MAOC) could be more involved in the re-evaluation discussions. For example, pre committee review meetings between committee membership and the MAOC could take place to understand what the expectations of the

committee are, what the issues with previous data are, what model is used and if there are any areas of uncertainty that may need clarification from a clinical and/or patient or carer perspective.
    - A scoping workshop to discuss original scope, review and focus of MAA data, clinical and patient organisations views, committees focus, expectations, exclusions etc. Guidance from this should be provided to the company and stakeholders so there is a clear understanding of what data is to be presented
- A clear MAA exit process.
    - This should be started at least 18 months in advance, with clear timelines, expectations, evidence plan and include earlier discussions between NHSE, NICE and companies.
- A co-ordinated communication plan
    - For example if an MAA needs to be extended, NSHE sometimes need to lead and direct these communications, this needs to be planned and communicated in advance to prevent any disruptions or delays to patients receiving treatment.

Theme 2: Impact of an interim 'no'

Throughout a MAA, interim decisions are published by NICE. At the first meeting of this group, all members had received an interim negative decision and only one member of the group had completed a MAA, where a positive recommendation was granted at the end of the MAA period following an interim no.

Members discussed how distressing an interim negative decision can be for the patient community and their families, despite the emphasis on the fact that interim decisions are not final. There was some discussion that an interim negative decision feels like a negotiation tactic to encourage the company to lower the price of the treatment and the impact this has on affected families is not considered. Some members have experienced frustration from their patient community for 'not doing enough' despite the organisation's ongoing engagement.

When a community receives an interim negative decision, there is concern over whether there will be continued access for those already receiving the treatment. Members reported instances where reassurance is sometimes verbally given by NICE during webinars or panel discussions but there is an unwillingness to provide this reassurance in writing, therefore making it difficult for patient organisations to feed this back to the community resulting in fear, confusion and frustration for families. This is further exacerbated by the uncertainty over who is responsible to pay for ongoing treatment, the NHS or the company, in case of a negative final decision. Some individuals from the community have embarked on judicial reviews as a result of an interim negative decision through fear of not being able to access the treatment.

Recommendation for NICE: At the beginning of a MAA, provide a standard statement that addresses continued access for patients already receiving treatment.

Some members shared that patients who have been a part of the MAA process have found it to be such a challenging experience that the patient organisation now struggles to recruit lay experts from their

community to appraisals of new medicines because the news has spread within the community of how mentally and emotionally difficult involvement with NICE's work can be.

Some members also reported instances where interim negative decisions are misunderstood to be final decisions by clinicians and therefore they do not offer the treatment to patients, or they inform patients that the treatment is no longer available. This news can spread through the community and patient organisations are tasked with managing this misinformation.

Recommendation for NICE: Develop clear and consistent generic information for patient organisations and clinicians about what the impact of an interim or final 'no' will be on people who are currently receiving treatment, and set out a clear process and timeline for what will happen next.

Recommendation for NICE: Work with the relevant patient organisations to give them embargoed advance notice of an interim or final 'no' decision, and undertake a tailored impact assessment of the community to understand how this decision will impact on them and determine what support NICE should provide to the patient organisation, patients, carers and clinicians when the announcement is made.

Theme 3: Burden on charities and data collection issues

All members of the group mentioned how labour intensive being involved in a MAA is for patient organisations. Liaising communications between NICE and the community plays a big part in that but also patient organisations spend a lot of time supporting the enrollment of patients to MAAs and collecting data during a MAA. Some members expressed challenges with not knowing what type of data and information they should be collecting to address the specific concerns of the committee and uncertainties as to what was expected from patient organisations during the data collection processes.

Some members also felt that they had to proactively seek out what the next steps were, particularly for those organisations who were unfamiliar with the process. The PIP team were very helpful in responding to these queries but at each stage of the process, the patient organisation had to ask 'what does this mean', 'what do we have to do', 'how does this impact patients' etc.

Patient groups spend a lot of time and resource collecting quality of life data and patient reported outcomes that aim to respond to the uncertainties that are highlighted during the treatment's appraisal. Overall, members of the group felt that the data that is collected during a MAA isn't used much in the final decision, emphasised by the fact that some members are told to re-submit the same evidence from previous evaluations throughout the MAA.

Members of the group had contrasting experiences on how their data was used in the re-evaluation. Some members expressed frustration over their experiences where the quality of life data they had collected couldn't be used in the final assessment because it didn't fit into the company's pre-existing evaluation models, therefore having a minimal impact on the decision. Other members also shared that there appeared

to be a disconnect between what the company thought they had to submit and what the committee were expecting to see in the final re-evaluation. On the other hand, some members shared positive experiences of their data being highly accepted in the absence of evidence from the company.

Some members of the group also reported that they have previously found themselves having to educate clinicians that patients can access treatment through a MAA as they were unaware of it. Some clinicians can act as a 'gatekeeper' to the treatment where they don't put patients forward for the MAA believing that they are not eligible for the treatment however it's not the clinicians that make the decision. Patient groups therefore spend a significant proportion of time tackling misinformation that is shared amongst the community from healthcare professionals.

Recommendation for NHSE: Produce a simple guide for clinicians to help them understand how patients can access treatments through an MAA and that interim negative decisions are not final.

Members shared feedback they have received from their community saying that a significant proportion of appointment times, in this example physiotherapy appointments, is spent on measuring clinical effectiveness, as per the MAA, but that doesn't leave enough time for physiotherapists to talk to patients about how to manage their condition. Some clinicians have fed back that the data collection takes them away from treating other conditions where there are no treatments available.

Recommendation for NICE: Consider organising similar roundtables with clinicians and pharmaceutical companies to listen to their experience of MAAs and see whether there is a similar opportunity for shared learning to improve the MAA process.

There is a general sense from those contributing to the roundtable that MAAs are burdensome for all involved, the clinicians, patient groups and the pharmaceutical companies. Members were clear that now that a significant number of MAAs have been carried out there is a real opportunity for NICE to learn from what is working well, what is working less well, and to take steps that will improve the process for patient organisations that take part in future.

We hope this report is a constructive contribution to help make it as easy as possible for small and specialist charities and support groups to provide their vital input and support informed decision-making by NICE and its committees.