Metabolic Dietary Disorders Association (MDDA) submission regarding management and communication of medicine shortages.

Background

The MDDA is the peak health advisory patient body for people impacted by inborn errors of protein metabolism (IEM – amino acid). As people with these conditions are unable to correctly metabolise protein most disorders in this group are treated with PBS subsidised supplements that provide a 'safe' form of protein as well as other nutrients that people unable to eat natural forms of protein need to remain healthy. In addition, some IEM amino acid disorders require medications one of which is on the Life Saving Drugs program (LSDP).

Comments on the Proposal

PBS Subsidised Supplements

The MDDA suggests that all PBAC listed treatments, including those not registered with the TGA, be included in the reporting and communication to ensure that patients are adequately informed about shortages and discontinuations. Although there are different choices for specialised supplements meaning that there should never be a critical shortage we believe shortages and discontinuation of specific supplements should be reported to patients either via their treating medical professional or via the MDDA. As these supplements form the bulk of dietary intake for people with these conditions and the condition cannot be managed without them it is important that patients are aware they may need to transition to alternative forms of their dietary supplement. These conditions are lifelong conditions diagnosed via newborn screening and poor compliance during childhood has severe health implications including neurological damage, seizures and liver damage. Supplement refusal can be a serious problem in children and sudden changes to familiar and tolerated supplements can make compliance extremely difficult, therefore shortages of specific supplements should be reported to allow families, parents and treating professionals time to transition patients to alternative supplements that they can tolerate.

We feel that the reporting periods recommended are suitable however for the purposes of this specific group of products we would like to see reporting for specific items listed on the PBS rather than medicines as a whole. This is due to the fact that the form and taste of different supplements that may have the same therapeutic benefit vary widely.

In terms of penalties for non-compliance with reporting requirements, infringement notices and civil penalties for deliberate non-compliance should be considered as many medicines for rare conditions such as IEMs may have a single supplier so naming them on website or in the public arena is unlikely to drive change in consumer or sponsor behaviour if the non-compliance is deliberate.

LSDP Medications

The MDDA would like to suggest that all LSDP medications be on the medicine watch list. These are highly specialised, usually orphan drugs that prolong life and for this reason should be automatically included on the Medicines Watch List. Nitisinone (Orfadin), a drug listed on the LSDP, is used to treat Tyrosinaemia Type 1, an extremely rare IEM- amino acid, any interruption of supply would have catastrophic impact on patients and for this reason should be included on the medicines watch list and treating professionals notified immediately.