Life Saving Drug Program Review Submission

1. Review the clinical effectiveness and safety of medicines currently subsidised through the LSDP.

Medicines currently subsidised through the Life Saving Drug Program (LSDP), for some of our MPS patients, are the following forms of Enzyme Replacement Therapy (ERT): Laronidase (Aldurazyme) for MPSI; Idursulfase (Elaprase) for MPSII and; Galsulfase (Naglazyme) for MPSVI. The application for Elosulfase alpha (Vimizim) for MPSIVA is currently being considered by the PBAC.

Any review of the clinical effectiveness of the medicines must take into consideration the wide heterogeneity within the MPS diseases. It must be remembered however that all patients, even those with attenuated forms of the disease, face serious, progressive, life threatening and life-limiting health complications. Any improvement or stabilisation in health or function from these medicines must be compared not just to baseline results but to the progressive, devastating decline that is the known prognosis for these diseases without treatment.

2. Review emerging clinical treatments and diseases, including those that identify subgroups by molecular target, which could potentially seek subsidisation through the LSDP in the future.

All people with MPS or a related disease need timely access to subsidised treatments, as soon as they are developed for their particular condition. For example, research is fast progressing for MPSIII. It is vital that when an effective medicine is developed, Australian patients can access it as soon as possible to prevent any further deterioration and ensure the best possible outcomes. To gain earlier access to new treatments, we also need a framework that actively enables and encourages Australian patients' participation in clinical trials. The Society calls for the removal of the current guideline that effectively excludes LSDP patients from clinical trials, and thus their early access to new treatments.

While we know these medicines (ERTs) are effective for MPS, they are currently unable, or only partially able, to reach all parts of the body, e.g. the brain, due to the body's blood-brain barrier and other parts, such as bones and heart valves. Globally, research is progressing in regards to treatment overcoming the blood-brain barrier but also for treatment that targets those other areas that have been traditionally hard to reach. It is likely that subsidy could be sought for specially formulated or modified treatments that will complement and enhance the benefit of an existing treatment, as it enables treatment to reach and treat more parts of the body and dramatically improve patient outcomes.

Globally, clinical practice has increasingly featured the short-term/ targeted use of ERT for a subset of MPS patients: those who are undergoing transplant. These patients are currently excluded from accessing ERT (Aldurazyme and Elaprase) subsidised through the LSDP. As a result, in Australia, a clinician's ability to follow international recommended practice for this group of patients is greatly impacted. Uncertainty about access to treatment options further impacts on the patients' families who are already dealing with a recent diagnosis and preparing for the extremely difficult and long process of transplant.

There is also promising research into an oral treatment that can increase the effect of ERT, potentially reducing the frequency of weekly intrusive IV treatments. Importantly, this shows great potential to reduce the burden of treatment for both patients and carers in the future.

From these examples, clearly it is vital that the LSDP is able to stay abreast of research developments in the MPS space, to ensure that the needs of patients are met now and in the future. Unfortunately by disbanding the Advisory Committees, the Health Department has also arguably lost its connection with those key clinicians who are best positioned to advise the Department on research and clinical developments in Australia and overseas. We need a structure that allows and encourages this needed discourse between the Department and key clinicians and researchers. We need a framework that actively enables and encourages Australian patients to participate in clinical trials. As has been shown, research is continually developing in the treatment of MPS. Treatments for more types of MPS are being developed, and international experts believe existing treatments will likely be combined with new targeted treatments to improve patient outcomes in the future.

3. Conduct an international comparison of subsidisation of drugs for rare diseases and the definitions for a rare/ultra-rare disease.

The broader Australian community expects Australia to compare favourably to the rest of the world when it comes to access to medicines. It is vital that Australian patients can access treatments that are available to patients from other parts of the world. Subsidisation of drugs literally changes lives; but also helps provide certainty, clarifies the path ahead, and provides much needed hope.

In an increasing number of countries, subsidisation of drugs is a key part of a broader formalised response to those living with Rare Disease. Unlike many countries, Australia does not yet have a National Rare Disease Plan. While a crucial mechanism to provide access to specific treatment, the LSDP is no substitute for a much needed broader rare disease policy and framework. This is an area that urgently needs to be developed and resourced in Australia to keep up with best practice internationally.

4. Compare the subsidisation and equity principles of the Pharmaceutical Benefits Scheme and the LSDP.

It is vital that the specific features and challenges of rare disease are acknowledged. The purpose/ aims of the LSDP and its inherent differences to the PBS must remain central to any program review. The PBS subsidises a broad range of medicines for consumers of all ages for a range of diseases and conditions. Quality of Life is a considering factor. For a medicine to be funded through the PBS, it must demonstrate clinical and cost effectiveness. Treatments for MPS and other rare diseases will never meet cost effectiveness criteria.

The LSDP aims to provide subsidised access for eligible patients to expensive and life-saving drugs for serious and rare medical conditions. It is clearly vital for the MPS and broader rare disease community to access much needed treatment. An LSDP criterion requires evidence to predict that a patient's lifespan will be substantially extended as a direct consequence of the use of drug. This is highly problematic. There is, and always will be limited data in the Rare Disease population. It is necessary to set a data threshold however we would argue that there has been insufficient recognition of the inherent realities of rare disease, i.e. a greatly limited data set. As a result, the LSDP runs the risk of not being able to actually achieve the aims of the Program, for the specific patient group for which it was designed.

In this context, the name 'lifesaving drug' is also problematic. We would suggest that it is more meaningful and accurate to use terms and require data that relate instead to a change or stabilisation of the predicted course of the disease. We would also recommend that Quality of Life is considered, as long as it is considered in relation to Quality of Life without treatment. It is also important when considering the clinical effectiveness of a drug, not to do so in isolation but to recognise that often it is the only treatment option available. The Society strongly calls for all MPS patients to be able to access the most effective treatment available at any current time.

5. Assess the value for money of the medicines subsidised on the LSDP by evaluating the benefit of each drug's treatment outcomes, including in terms of quality of life achieved through the programme, and their cost.

The true impact of clinical changes from the ERTs can arguably only be fully understood through the patients' perspective. In our experience even the most positive data cannot always fully reflect the full benefit or real-life impact for MPS patients. Increases in walking distances represent increased endurance, but in real life the significance of this is less reliance on a wheelchair, increased mobility and social participation. Similarly, reduced liver and spleen size plus increased growth results in a more 'normal' body shape and appearance. In reality, this translates to increased self-esteem and self-confidence for patients. Patients/Parents report that as their tongue reduces, patients breathe better and start talking more. Increased joint flexibility translates to increased ability to care for oneself, and reduces the need for a carer. As one of our MPS mums states, "ERT is allowing him to just be one of the boys and not the boy with the disability." Before ERT, one 16 year old girl with MPSVI was told she would never walk again. After starting ERT later that year, she was walking independently and remains so 13 years later. There are countless more stories like this. It is vital that patient voices are central to any focus on effectiveness, value for money or quality of life.

As stated we believe that all patients should have access to the best possible treatment available at the time. Treatments will develop, over time, that are more effective, more targeted, and less intrusive. This needs to be strongly encouraged. In the search for value for money, Government needs to ensure that continual innovation is not discouraged, or that subsets of patients are not excluded from treatment. Alternate pricing arrangements or models must be negotiated between Government and pharma. Ways must be found to manage the price of these drugs, while still providing pharma adequate profit and incentive to keep investing in new and innovative treatments A way forward has to be found. It is not fair to discriminate against those who are unlucky in having an illness where science has not found a cost-effective intervention.

6. Review the administration of the LSDP, including the Guidelines with which the programme is administered for each condition, and assess alternative administration systems.

The current two stage process of a rare disease drug having to be first assessed by PBAC as not cost effective before it can even be assessed by the LSDP is unnecessarily cumbersome and time consuming. As patients have progressive and deteriorating conditions, the impact of this delay should never be understated.

The removal of the Advisory Committees has streamlined the new patient application process. Arguably it also means clinicians can more freely advocate on behalf of their patients. However, it is vital that there is some formal communication structure between the LSDP and expert clinicians to ensure that the LSDP remains informed about developments in best practice and research for MPS from around the world.

We recommend that the LSDP look to increasingly involve the relevant patient group representatives and other key stakeholders in formal discussions regarding a proposed or current drug. We believe this this would help progress discussions, ensure patients' needs remain central to the process, and certainly increase transparency and accountability.

A requirement of continued subsidisation is periodic assessments of patients on treatment. Wherever possible this needs to be done in a manner that is least burdensome for MPS patients (often young children) and their carers, who are already contending with numerous hospital visits and procedures/ interventions.

7. Establish a framework for data collection on rare diseases in Australia and assess how this could function internationally.

As limited data is unfortunately a feature of rare disease, it is of utmost importance to collect as much data as possible that will help guide best practice and future research. It is our experience that all key stakeholders (patients, clinicians, researcher) support this. Current challenges and obstacles that need to be overcome are funding and resources, who owns the data and who can access the data; how is the data kept and for how long. Often valuable data is only collected for short-term specific purposes. Despite these challenges it is important that an affective framework is developed, and crucial to address the needs of the MPS and broader rare disease community now and in the future.