Advice created by all the NHS LSD Specialist Centres in the UK

PAEDIATRIC ADVICE

Categories of patients: There will be only two categories, exactly according to government advice. However, within the high-risk group, centres may wish to start ceiling of care discussions with individual patients deemed to have a particularly poor prognosis, if ventilated. This might include, but not be limited to, Pompe patients with respiratory weakness and MPS patients with compromised airways. This is not only for individual patient quality of life, but also for the rational allocation of scarce life-saving resources.

When to trigger self-isolation: We will follow government advice and support patients who choose to self-isolate based on this advice. Units may contact some families to clarify whether their underlying condition constitutes a high risk group that would benefit from self-isolation but this will be highly individual, patient-specific advice.

If a patient is self-isolating because of COVID-19 symptoms, nurse visits for ERT infusions are paused for the duration of self-isolation (currently 14d). If patients can become independent, that would be advantageous, but homecare resources are not available for large-scale, short-term training.

If a family chooses to strictly self-isolate because of underlying vulnerability, LSD centres will have an individual discussion with the family about their ERT and the risk/benefit balance of taking a prolonged "drug holiday". We will reassure patients undergoing a "drug holiday" that this is a decision in their best interest, based on the greater risk to them of being infected with COVID-19, than any modest or moderate effect of missing treatment for a period. If patients can become independent, that would be advantageous to enable some of those patients to continue ERT, but homecare resources are not available for large-scale, short-term training.

If a family chooses to undertake "more stringent social distancing" because of underlying vulnerability, but short of full family self-isolation, it may be appropriate for some of those patients to continue receiving ERT as long as homecare companies can deliver this. This is felt to be essential for infantile LAL deficiency, infantile Pompe Disease Type III Gaucher and some MPS patients being prepared for stem cell transplantation. [CLN2 patients receiving intrathecal cerliponase in hospital will also continue to be prioritised for this treatment]. If homecare company staffing becomes critically low, then patients with other diseases may be required to reduce their dosing frequency.

Patients with indwelling venous access devices who are having a drug holiday should have these flushed at the longest interval compatible with keeping the specific device in operation. This may appear to contradict the statement in relation to infusions, but it is a balance of risk decision based on how critical to the patient's health the individual procedural visit is.

Critically ill lysosomal disease patients should not be transferred to LSD centres unless there are clear clinical reasons for this. They should as a rule continue to receive treatment at their local hospital as appropriate.

We will defer any discussion of catch-up infusions until the crisis is over.

We will keep in contact with self-isolated patients by regular telephone calls, the frequency to be determined by need and staff availability.

23 March 2020