Opening Statement of Muscular Dystrophy Ireland to the Joint Committee for Health 12/07/17

I would like to thank the Committee for taking the time to hear our representations today.

I speak on behalf of Muscular Dystrophy Ireland and the 5 children currently known to us who could benefit from the drug Translarna.

I especially speak on behalf of the two children aged 5 and 7 who are currently losing valuable treatment time.

Duchenne Muscular Dystrophy is a debilitating condition resulting in the progressive weakening and wasting of the muscles. A child with DMD can expect to lose the ability to walk by the age of 10, to develop cardiac and respiratory problems in their teens and will have an average life expectancy of 27 years.

Translarna is the first ever treatment for DMD to receive EMA approval.

European Specialists indicate that it has the potential to change the course of the disease and improve the overall life expectancy of patients.

Since 2014, over 400 children in 22 European countries have been receiving this treatment. That means that 80% of eligible children are now receiving the drug and this leaves Ireland as one of the last remaining countries to facilitate treatment.

MDI have made several observations during the assessment of Translarna including an excessively long process, little consideration for the progressive nature of the condition, no meaningful engagement with patient groups and no evidence of expert clinical input.

There has been no sense of urgency to review Translarna, and delays to date have included

1. The decision not to include the compelling evidence from the phase 3 ActDMD study in the consideration.
2. 9 months from the NCPE's recommendation not to reimburse, to the HSE's communication of a decision not to reimburse.
3. 4 months from the HSE leadership decision to the communication of this decision to the company.
4. Failure to discuss Translarna at the most recent drugs group meeting, despite it being an Agenda item.

It is now almost a year since the HSE Leadership team stated that they were 'eager' to review Translarna again.

These considerable delays demonstrate clearly that there has been little understanding of the progressive nature of the condition, the clear unmet medical need and the small window of opportunity that exists for these children.

One requirement of treatment is the ability to walk at least 10 steps unaided. Delays in access will directly result in some of these children missing the opportunity for treatment as their condition deteriorates and they lose ambulation.

It would appear that the HSE/NCPE's decision not to reimburse for Translarna is at odds with regulators, health authorities and experts across Europe, implying that the European Medicines Agency and the 22 other European Countries have all gotten it wrong.

This decision also jeopardises the child's "explicit right to achieve their developmental potential and to sustain the highest possible standard of health", Under the UN Convention on the Rights of the Child.

For the first time ever, we are seeing treatment s in development for Duchenne, SMA, Alpha 1, Cystic Fibrosis, Cystinosis and a number of other rare diseases. Ireland does not appear to be ready for this and we have a situation whereby Irish patients are falling behind their European Counterparts.

In the case of access to Translarna, Ireland is now 3 years behind France and Germany and a full year behind the UK and Northern Ireland.

Our current system of assessment is not fit for purpose for rare diseases and we are concerned about the lack of development of strategies for the provision of high technology and orphan therapies. As it stands, No orphan drugs will get through this process and no people with a rare disease will be treated until a solution is put in place.

One such solution is the implementation of the recommendations of the national rare disease plan, approved by government in 2014. The HSE has committed to the development of a working group to bring forward appropriate decision criteria for the reimbursement of orphan medicines and technologies. The promised Technical Review Committee for Orphan Drugs, while in progress, is currently without a chair and un-resourced.

We also need to consider other avenues for accessing orphan drugs, including Fast track, Managed Access and Managed Risk Programs.

Our request as a patient organisation is for a timely conclusion of the review of Translarna and a fair and transparent process that will not leave rare diseases behind and for our children with Muscular Dystrophy to attain the same opportunities for health as their European Counterparts, especially those in adjoining jurisdictions.

Thank You