In relation to access to medicines and technologies for people with rare diseases, recommendation 30 from the National Rare Disease Plan for Ireland 2014 – 2018 suggested the establishment of an assessment system similar to that for cancer therapies established under the National Cancer Control Programme i.e. the NCCP Technology Review Group. The HSE has implemented this recommendation setting up the Rare Diseases Technology Review Committee (RDTRC). The aim of the RDTRC is to facilitate the incorporation of the patient and clinician perspective into an agreed statement which will be presented to the HSE – Drugs Group to aid the Drugs Group in it’s reimbursement recommendations to the HSE Leadership team. The RDTRC is not a voting or decision making forum. Professor Michael Barry was asked by the HSE to help establish and to Chair the RDTRC.

Membership of the Committee includes the Chair, the Clinical Lead for the HSE Rare Diseases Programme, 5 Consultant Physicians with expertise in Rare Diseases, two Pharmacists, one Health Technology Assessor, one representative from the Health Information and Quality Authority and two patient representatives. The first meeting of the Committee was held on the 15th October 2018 and a second meeting was held on the 8th November 2018. Meetings were also held with patient representatives (30/11/2018, 8/11/2018) and the manufacturer of nusinersen on the 30th October 2018.

The Committee has focused on two medicines to date i.e. sapropterin (Kuvan) for Phenylketonuria and nusinersen (Spinraza) for the treatment of Spinal Muscular Atrophy (SMA). The Committee recommended to the HSE Drugs Group that access to Sapropterin be considered under a managed access programme with clear starting and stopping criteria. A RDTRC recommendation in relation to nusinersen (Spinraza) is expected shortly.

Michael Barry
Chair of RDTRC
19/11/2018