



Rannán na nOspidéal Ghéarmhíochaine
Aonad 4A, Áras Dargan,
An Ceantar Theas,
An Bóthar Mileata
Cill Mhaighneann,
Baile Átha Cliath 8

Acute Operations
Health Service Executive
Unit 4A, The Dargan Building,
Heuston South Quarter,
Military Road,
Kilmainham,
Dublin 8

Deputy Colm Burke,
Dáil Eireann,
Leinster House,
Kildare Street,
Dublin 2

22nd September 2020

PQ 22873/20* To ask the Minister for Health the status of the work of the rare diseases technology review committee of the HSE; the meetings it has held to date in 2020, in tabular form; the meetings and therapies it has scheduled for the remainder of 2020; and if he will make a statement on the matter..

Response:

Dear Deputy Burke,

The Health Service Executive (HSE) has been requested to reply directly to you in the context of the above Parliamentary Questions, which you submitted to the Minister for response.

A Technology Review Committee for Rare Diseases (as recommended by the National Rare Disease Plan for Ireland) was appointed in late 2018 with responsibility for:

1. Reviewing proposals received from industry or expert groups in Ireland for funding of new products for rare diseases, or expanded indications for existing products for rare diseases and making recommendations as to the implementation of the relevant recommendations from the National Rare Diseases Plan 2011-2018; and
2. Providing contributions to the development of clinical guidelines for relevant Orphan Medicinal Products (OMPs) and supporting the implementation of guidelines in conjunction with the National Drugs Management Programme Office where applicable.

Since the Committee was established, there have been five meetings held following requests to review new or existing medicinal products for rare diseases. Between meetings, a significant volume of work is carried out by members including the production of prescribing guidelines, outlining how managed access programmes would work, identifying starting and stopping criteria for drugs etc. Due to COVID-19 pressures, the Committee has not met in

recent months, however meetings are scheduled for September and during Q4 2020 at which the terms of reference will be reviewed.

On the agenda for the upcoming meeting the following drugs are scheduled for review:

- Patisiran, for the treatment of hereditary transthyretin-mediated (hATTR) amyloidosis.
- Burosumab, for the treatment of X-linked hypophosphatemia and tumour-induced osteomalacia.

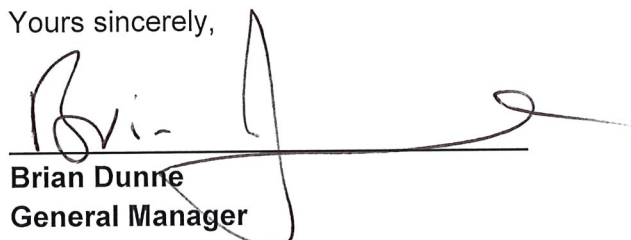
The meeting schedule for the remainder of the year will be largely determined by any future requests received.

Refresh of the membership is currently underway which includes the filling of the currently vacant Pharmacist and Administration posts funded to support the Committee.

I trust the above addresses your query but please do not hesitate to contact me if you require any further details.

I trust this answers your question to your satisfaction.

Yours sincerely,



Brian Dunne
General Manager
Acute Operations