Feidhmeannacht na Seirbhíse Sláinte
Health Service Executive

Rannan na nOspideil Ghearmhíochaine
Aonad 4A – Áras Dargan
An Ceantar Theas
An Bothar Mileata
Cill Mhaighneann
BÁC 8

Acute Operations
Health Service Executive
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Heuston South Quarter
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Dublin 8.

Date: 11th October 2022

Deputy Colm Burke
Dáil Éireann
Leinster House
Dublin 2.

PQ 47133/22 **** To ask the Minister for Health the specific drugs that have been considered by the rare diseases technology review committee of the HSE to date in tabular form; the outcomes of these decisions; the specific drugs to be considered by the committee; the number of meetings that have taken place and if he will make a statement on the matter .****

Dear Deputy Burke,

The Health Service Executive has been requested to reply directly to you in the context of the above Parliamentary Question, which you submitted for response. I have examined the matter and the following outlines the position.

Response:

The Rare Diseases Technology Review Committee for (RDTRC) (as recommended by the National Rare Disease Plan for Ireland) was appointed in 2017 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.

The RDTRC meets as required and has met on four occasions since Sept 2020.

The table below summarises the drugs that have ben considered to date;

Company	Product	Active Ingredient	Treatment
BioMarin	Kuvan	Sapropterin	PKU
Shire	Revestive	Teduglutide	SBS
Biogen	Spinraza	Nusinersen	SMA
Kyowa	Crysvita	Burosumab	X-linked hypophosphataemia
Alnylam Pharma	Onpattro	Patisiran	Amyloidosis
Novartis	Luxturna	Voretigene Neparvovec	Inheritated Retinal Dystrophies
Takeda	Alofisel	Darvadstrocel	Crohn's disease

The RDTRC does not have decision making capacity in relation to new medicine approvals. The applications listed above have been approved by the HSE EMT, with the exception of Darvadstrocel which remains under assessment, having been referred by the Drugs Group in September 2022, for input by the RDTRC.

Voretigene Neparvovec has been approved subject to the implementation of a HSE Managed Access Programme, and this is currently being developed.

I trust that this answers your question to your satisfaction.

Yours sincerely,

Brian Dunne
General Manager
Acute Operations