



Brendan Griffin, T.D.
Dáil Éireann,
Leinster House,
Kildare Street,
Dublin 2.

16th November 2021

PQ: 52331/21

To ask the Minister for Health further to Parliamentary Question Nos. 575 and 578 of 4 October 2021, the number of the 15 rare disease new medicines approvals, resulting from the €50 million for new medicines announced as part of Budget 2021 that were for the treatment of non-oncological rare diseases; and if he will make a statement on the matter.
-Brendan Griffin

Dear Deputy Griffin,

The Health Service Executive has been requested to reply directly to you in the context of the above Parliamentary Question (Reference 52331/21), which you submitted to the Minister for Health for response.

The Committee for Orphan Medicinal Products (COMP) is the European Medicines Agency's (EMA) committee responsible for recommending orphan designation of medicines for rare diseases. This designation is for medicines to be developed for the diagnosis, prevention or treatment of rare diseases that are life-threatening or very serious. In the European Union (EU), a disease is defined as rare if it affects fewer than 5 in 10,000 people across the EU. The European Commission decides whether to grant an orphan designation for the medicine based on the COMP's opinion.

The HSE CPU does not maintain a register of medicines for rare diseases and/or designated orphan medicines as the orphan status for a medicine is subject to change (i.e. may be removed from the Community Register of designated Orphan Medicinal Products). To check/clarify **the current orphan status of a medicine**, the appropriate repository for this information is the EMA website (<https://www.ema.europa.eu/en>).

Of the designated orphan medicine pricing and reimbursement applications for rare diseases approved for funding in 2021 (to date), eight are for non-oncology indications (uses).

Approvals of orphan medicines to date include medicines for the treatment of amyloidosis, cystic fibrosis and spinal muscular atrophy (SMA).

Yours sincerely,



Suzanne Doyle
Primary Care Eligibility & Reimbursement Service