FOI 23/554

Dear

Thank you for your email.

> *1. Breakdown of all orphan medicines which gained marketing authorisation approval in the calendar years **2020, 2021, 2022, 2023 YTD***
>
> *2. Breakdown of all ultra-orphan medicines which gained marketing authorisation approval in the calendar years **2020, 2021, 2022, 2023 YTD***
>
> *3. The number of Early Access to Medicines Scheme (EAMS) applications submitted and awarded, broken down by EAMS stage (Promising Innovative Medicine designation, and Scientific Opinion) as well as rarity of the indication (Orphan, ultra-orphan, non-orphan)*
>
> ***4. For all orphan and ultra-orphan medicines awarded a scientific opinion, could you please give the name of the drug and their indication***

Information on PIM designations and Scientific Opinions for EAMs can be found on the MHRA website:
https://www.gov.uk/government/statistics/early-access-to-medicines-scheme-applications-pending-refused-granted

Information on Orphan designation products, including the orphan register is available from MHRA via the following link:
https://www.gov.uk/guidance/orphan-medicinal-products-in-great-britain

Please find information on ultra-Orphan medicines on the Scottish Medicines Consortium:
https://www.scottishmedicines.org.uk/how-we-decide/ultra-orphan-medicines-for-extremely-rare-conditions/#:~:text=To%20be%20considered%20as%20an%20ultra-orphan%20medicine%20all,and%204%20the%20condition%20requires%20highly%20specialised%20management

Unfortunately, we do not collect information specifically in the format that you have requested and to do so would take longer than 24 working hours (so would be exempt under S12 of the FOIA). However, you have the list of EAMs medicines and Orphan Designation medicines from the MHRA website, and so can cross check these against the published SmPCs, PARs and our published lists of authorised products to get the remaining information.

If you disagree with how we have interpreted the Freedom of Information Act 2000 with regards to your request, you can ask for the decision to be reviewed. The review will be carried out by a senior member of the Agency who was not involved with the original decision.

If you were to remain dissatisfied with the outcome of the internal review, you would have the right to apply directly to the Information Commissioner for a decision. Please bear in mind that the Information Commissioner will not normally review our handling of your request unless you have first contacted us to conduct an internal review. The Information Commissioner can be contacted at:

Information Commissioner's Office
Wycliffe House
Water Lane
Wilmslow
Cheshire
SK9 5AF
Yours sincerely

MHRA Customer Experience Centre
Communications and engagement team
Medicines and Healthcare products Regulatory Agency
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