July 2026 update

Orphan Medicines

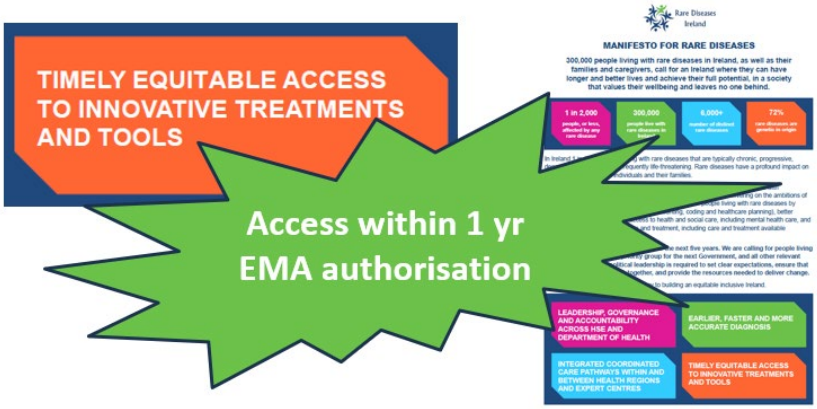
Saving lives, Reducing disability

Rare Diseases Ireland

What are Orphan Medicines?

- They are medicines that **prevent or treat rare diseases**
- **163 orphan medicines** currently have EU market authorisation (June 25, 2026)
- **Less than 5% rare diseases** can be treated with an orphan medicine
- Orphan medicines are **produced by 86 different companies**, the vast majority **(~66) with one/two orphan medicines in portfolio**

What do we want?



Orphan medicines currently EU authorised*

163 EU authorised orphan medicines

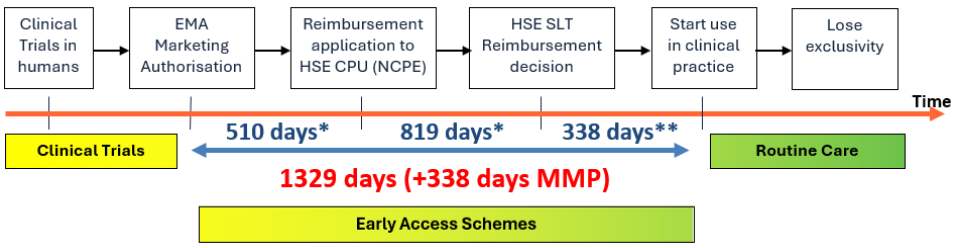
- **37% (61) reimbursed** by HSE
- **20% (32) pending** HSE decision
- **43% (70) NO submission** for reimbursement

Time delays with access in Ireland

- **510 days** (n=93) EMA authorisation to reimbursement application (where made)
- **819 days** (n=61) NCPE reimbursement application to reimbursement approval
- **338 days** Medicines Management Programme established, when needed.**

Programme for Government, National RD Strategy, Framework Agreement

- **Introduce Early Access Scheme for all orphan medicines**



URGENT request to Minister for Health
immediately establish a time-bound multistakeholder group that will review and make recommendations on
****implementing an early access scheme for rare diseases**** with a view to enabling access to orphan medicines within 1 year of EMA authorisation for people living with rare diseases in Ireland.



* From NCPE website & HSE Online Tracker Jun 25-29, 2026
* https://www.ema.europa.eu/en/documents/report/medicines-output-medicines-report_en.xlsx Jun 25, 2026
** IPHA Feb 2025 report – The Case for Fairer & Faster Access to Medicines