Orphan Medicines

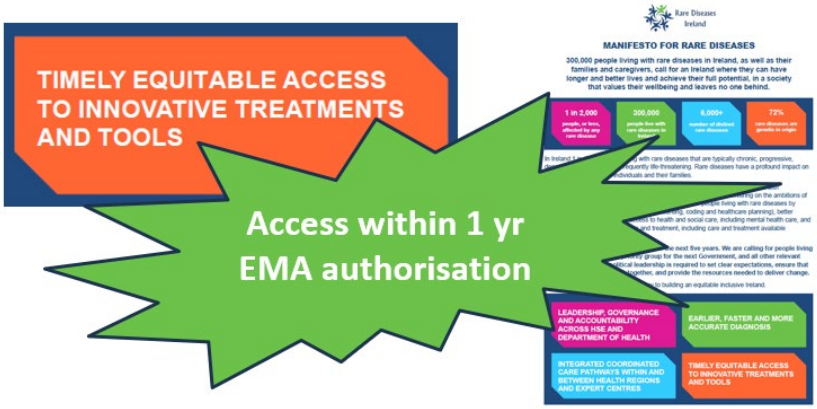
Saving lives, Reducing disability

Rare Diseases Ireland

What are Orphan Medicines?

- They are medicines that **prevent or treat rare diseases**
- **171 orphan medicines** have received market authorisation from EMA
- **Less than 5% rare diseases** can be treated with an orphan medicine
- Orphan medicines are **produced by 87 different companies**, the vast majority **(~80) with one/two orphan medicines in portfolio**

What do we want?



Orphan medicines EMA authorised 2019-2023 (5 years)*

68 EMA authorised in 5yr period

- **28% (19) reimbursement approved** by HSE
- **32% (22) pending** HSE decision
- **40% (27) NO submission** for reimbursement made

Time delays

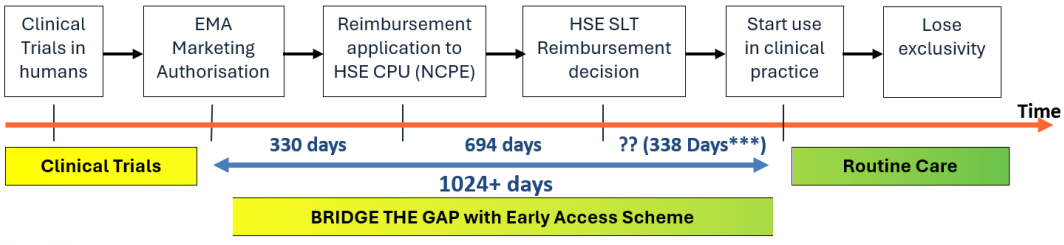
- **330 days** EMA authorisation to reimbursement application (where made)
- **1,024 days** EMA authorisation to HSE reimbursement approval (EU average 611 days**)

Access to orphan medicines in similar sized countries**

COUNTRY	#Orphan meds available
Ireland	13
Scotland	29
Finland	25
Denmark	34

Opportunities for reform (as outlined in Programme for Government)

- **Revise & update reimbursement system to accommodate orphan medicines**
- **Introduce Early Access Scheme for all orphan medicines**



*Data extracted from European Medicines Agency & National Centre for Pharmacoeconomics websites 9/10/2025
** EFPIA WAIT Report – 2024
*** IPHA Feb 2025 report – The Case for Fairer & Faster Access to Medicines