Rare Diseases Ireland

Oireachtas All Party Group

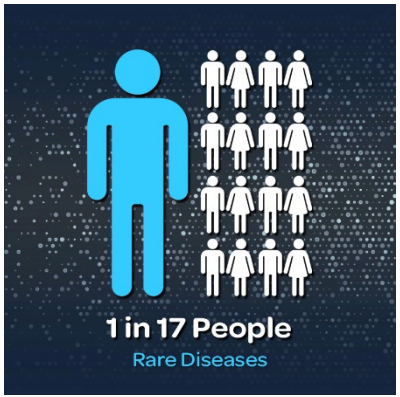
ACCESS TO ORPHAN MEDICINES

SAVING LIVES, REDUCING DISABILITY

July 8th 2026

What are Rare Diseases?

- Conditions that affect less than 1 in 2,000 people
- Estimated 6,000 - 8,000 different rare diseases







What are Orphan Medicines?

- Medicines that prevent or treat rare diseases
- Only **4.7%** of rare diseases benefit from an **orphan medicine** in Europe

"Rare diseases in numbers," Orphanet Report Series, February 2026

Huge unmet need in rare diseases driving orphan medicine investment…

Orphan medicine investment…

- Rare diseases account for **44% global clinical trials**
- Rare disease **pipeline grew 55%** over past decade
- Dominated by **small emerging biopharma**
  (running 61% of rare disease trials)

"From Orphan to Opportunity: Mastering Rare Disease Launch Excellence," IQVIA, April 2024

Investment is following unmet need;
but access is not keeping pace…

What do we want?

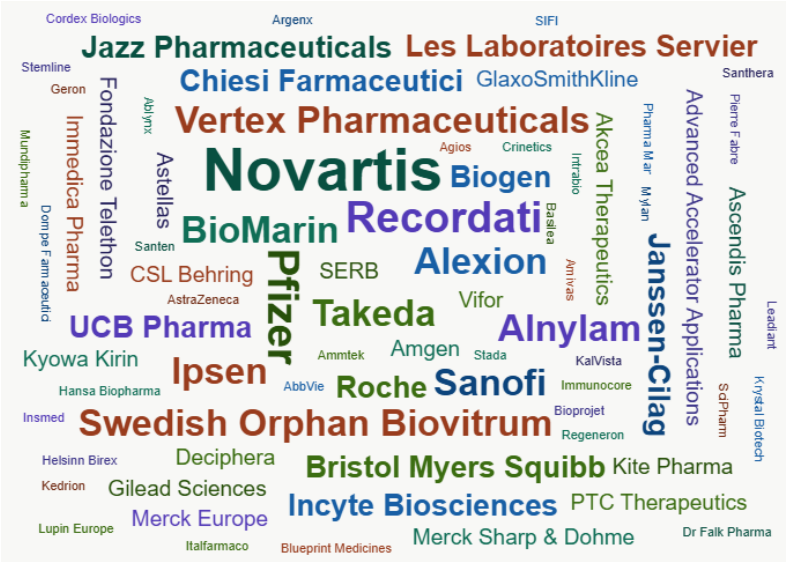


Appoint a cross-se h group to develop and implement a strategy that ensures innovative medicines are available to people living with rare diseases within one year of receiving European Medicines Agency (EMA) marketing authorisation, with appropriate safe delivery and outcome evaluation, including outcome registries.

EU authorised orphan medicines

- 163 orphan meds now authorised in EU

https://www.ema.europa.eu/en/documents/report/medicines-output-medicines-report_en.xlsx Jun 25, 2026



from **86 companies**, **66 of whom have one or two orphan meds in their portfolio**

Orphan medicine access in Ireland

- 163 orphan meds now authorised in EU

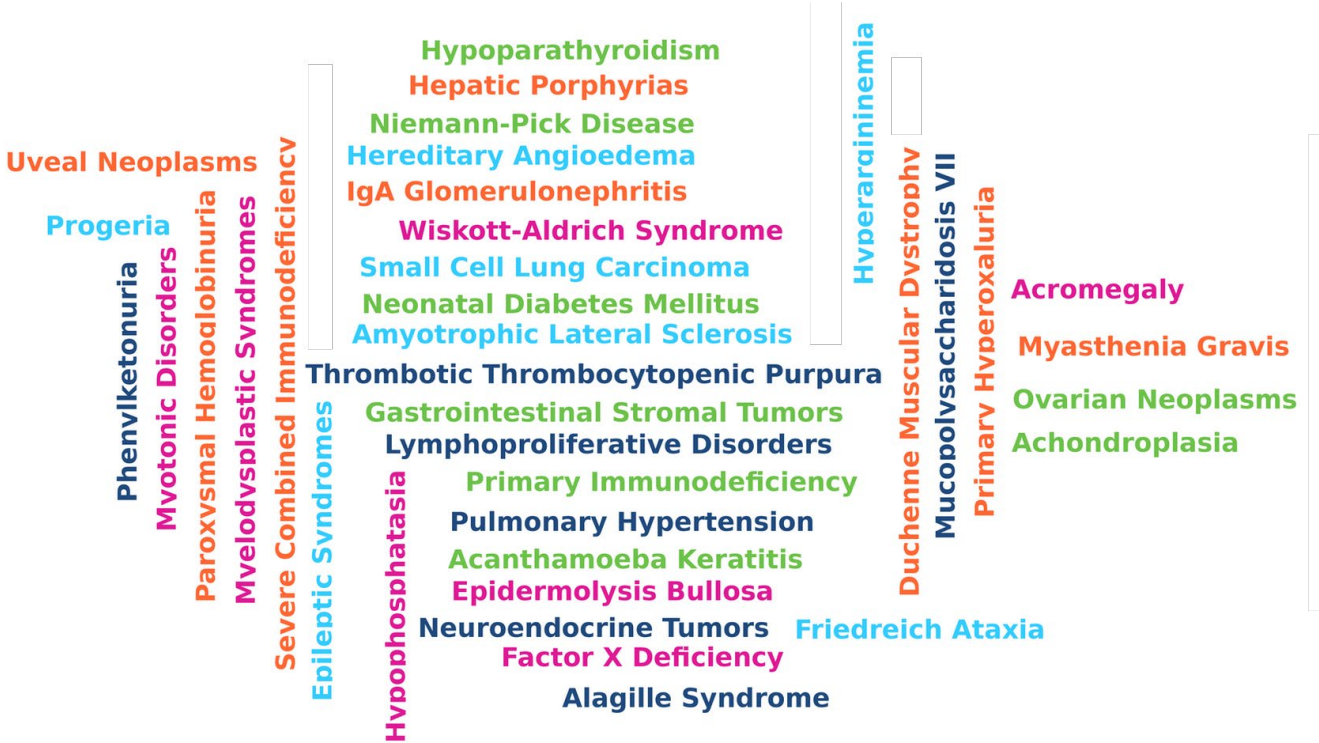
https://www.ema.europa.eu/en/documents/report/medicines-output-medicines-report_en.xlsx Jun 25, 2026

  - **37% (61) reimbursed** by HSE
  - 20% (32) in the 'reimbursement system'
  - **43% (70) no submission** for reimbursement

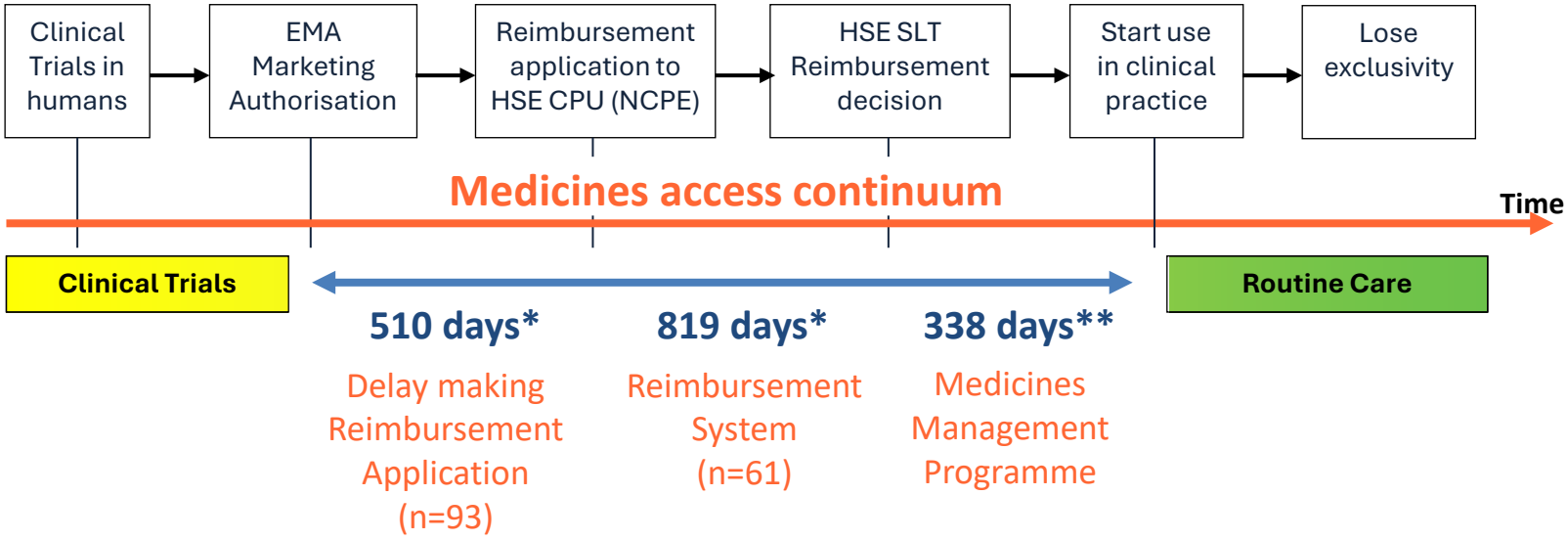
From NCPE website & HSE Online Tracker Jun 25-29, 2026



Some of the missed opportunities for improved quality of life



Access delays – all authorised orphan meds

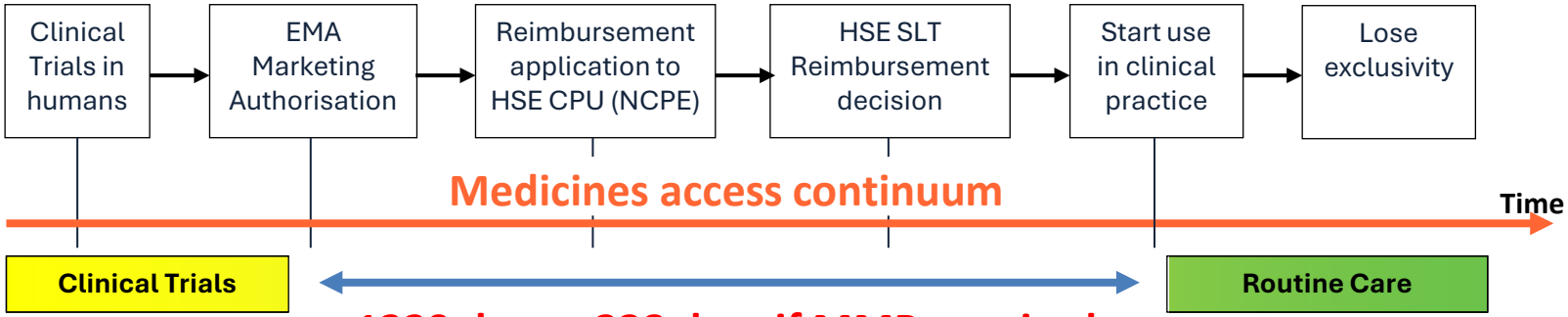


* From NCPE website & HSE Online Tracker Jun 25-29, 2026

* https://www.ema.europa.eu/en/documents/report/medicines-output-medicines-report_en.xlsx Jun 25, 2026

**IPHA Feb 2025 report

From EMA authorisation to use in clinical practice



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

Delays in orphan medicine access in Ireland

- 163 orphan meds now authorised in EU
  - **37% (61)** reimbursed – **1329 (+338 MMP) days**
  - 20% (32) in the ‘system’ – **1362 days**
  - **43% (70)** no submission – **no hope!**

Please ask the Health Minister to…

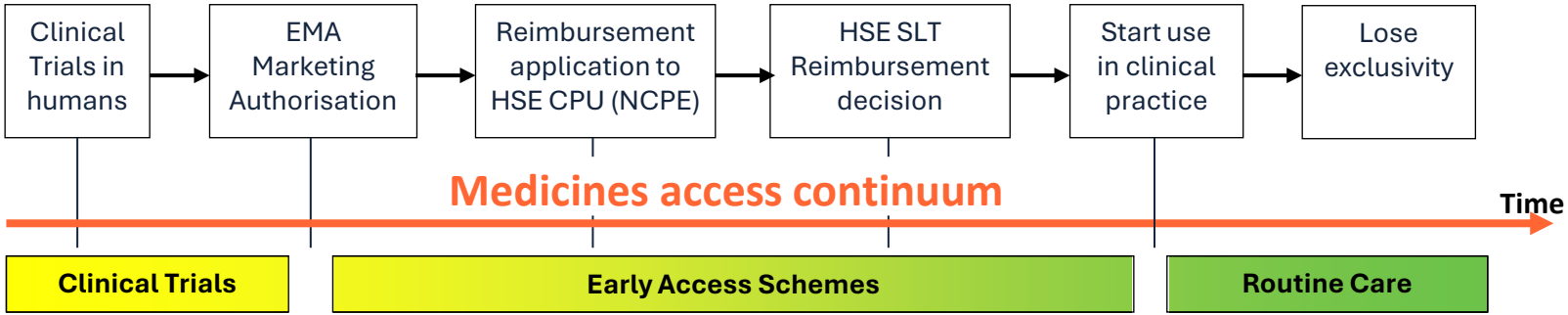
direct the Department of Health to establish a time bound multistakeholder group in January 2026 that will review and make recommendations on

- revising the drugs reimbursement process, and
- implementing an early access schemes 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.

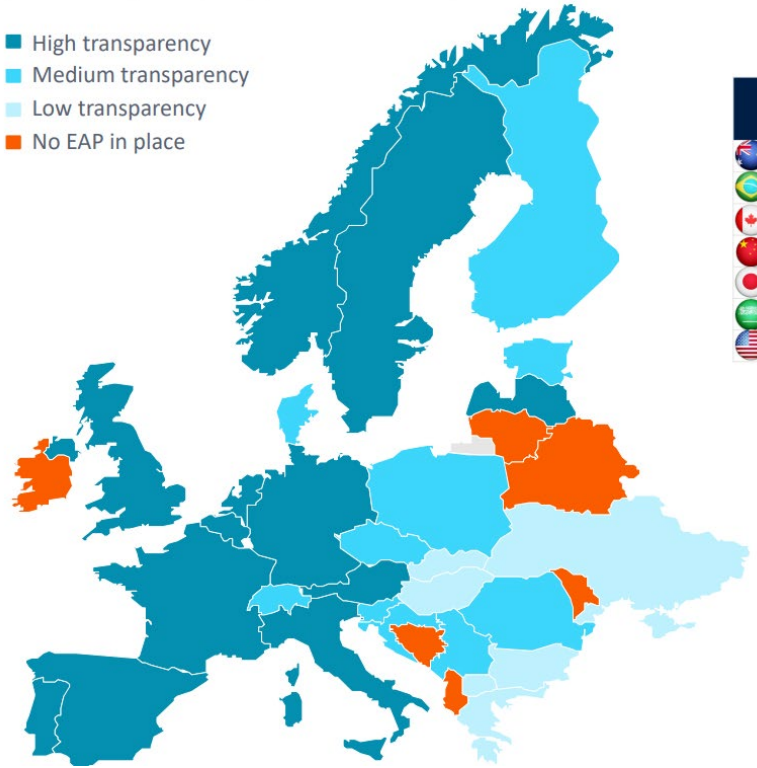
Our ask DEC 2025

Early access scheme to bridge delays



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

Early access schemes….



Non-European countries	EAP transparency
Australia	High
Brazil	Low
Canada	High
China	Low
Japan	Medium
Saudi Arabia	Medium
US	High

Ireland is an outlier with NO early access scheme

https://www.ispor.org/docs/default-source/euro2024/isporeurope24prueferthpr204poster146303-pdf.pdf?sfvrsn=53b4bbd3_0

Early access scheme noted in

- ✓ Programme for Government - Jan 2025
- ✓ National Rare Disease Strategy - Aug 2025
- ✓ Framework Agreement - Mar 2026

Where is the URGENCY?

We don't have the luxury of time…

Please ask the Health Minister to…

direct the Department of Health to

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.