Rare Diseases Ireland

Oireachtas All Party Group

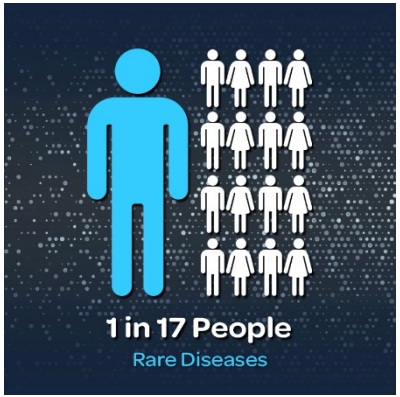
ORPHAN MEDICINES

SAVING LIVES, REDUCING DISABILITY

December 3rd 2025

What are Orphan Medicines?

- Medicines that prevent or treat rare diseases
  - Rare diseases affect less than 1 in 2,000 people
  - Estimated 6,000 - 8,000 rare diseases



Orphan Medicines*

- 151 orphan medicines with EMA authorisation
  - 7 companies selling 4 or more orphan meds
  - 80 companies selling 114 orphan medicines

- <5% rare diseases benefit from orphan medicine
  - 301 RDs as of March 2025

RDs represent huge unmet need & are target for innovation

* Orphanet's Medicinal Products for Rare Diseases in Europe report Apr 2025
https://www.orpha.net/pdfs/orphacom/cahiers/docs/GB/Medicinal_products_for_rare_diseases_in_Europe.pdf

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.

Orphan meds EMA authorised* (2019-23)

- 68 EMA authorised in 5yr period 2019-2023
  - 28% (19) reimbursement approved by HSE
  - 32% (22) pending HSE decision
  - 40% (27) **NO** reimbursement submission made

- 330 days (avg) - EMA authorisation to reimbursement submission
- 1,024 days (avg) - EMA authorisation to reimbursement approval (EU average 611 days**)

*Data extracted from European Medicines Agency & National Centre for Pharmacoeconomics websites 9/10/2025
** EFPIA WAIT Report – 2024

Orphan medicines access (2019-2023)*

Year EMA Authorisation	Number orphan medicines authorised	Number withdrawn from market	Number submitted to HSE (NCPE)	% eligible OMPs REIMBURSED by HSE	% eligible OMPs in HSE Reimbursement System	% eligible OMPs NOT submitted to HSE for Reimbursement
2019	5	1	3	25% (1 of 4)	50% (2 of 4)	25% (1 of 4)
2020	20	3	12	42% (7 of 17)	29% (5 of 17)	29% (5 of 17)
2021	17	2	10	40% (6 of 15)	27% (4 of 15)	33% (5 of 15)
2022	24	2	13	18% (4 of 22)	41% (9 of 22)	41% (9 of 22)
2023	10	0	3	10% (1 of 10)	20% (2 of 10)	70% (7 of 10)

*Data extracted from European for Pharmacoeconomics websites 9/10/2025

19 medicines
Avg 1,024 days
EMA to HSE approvals

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*Data extracted from European Medicines Agency & N [illegible] nics websites 9/10/2025

22 medicines
Avg 1,050 days since EMA approval

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*Data extracted from European Medicines Agency & National Centr

27 medicines
No submission made to HSE for reimbursement

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www.rdi.ie
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Small country market (fallacy)

- Orphan medicine availability in Dec 2024 in countries with similar population to Ireland* (EMA authorised 2020-2023)

- Delays in filing in Western Europe largely due to (in order of importance)**
  1. value assessment process
  2. evidence requirements
  3. lack of geographical footprint
  4. size of treatable population

COUNTRY	# OMPs
Ireland	13
Scotland	29
Finland	25
Denmark	34

* EFPIA WAIT Report – 2024

** EFPIA European Access Hurdles Portal: Results from the third year of data collection May 2025 – Charles River Assoc

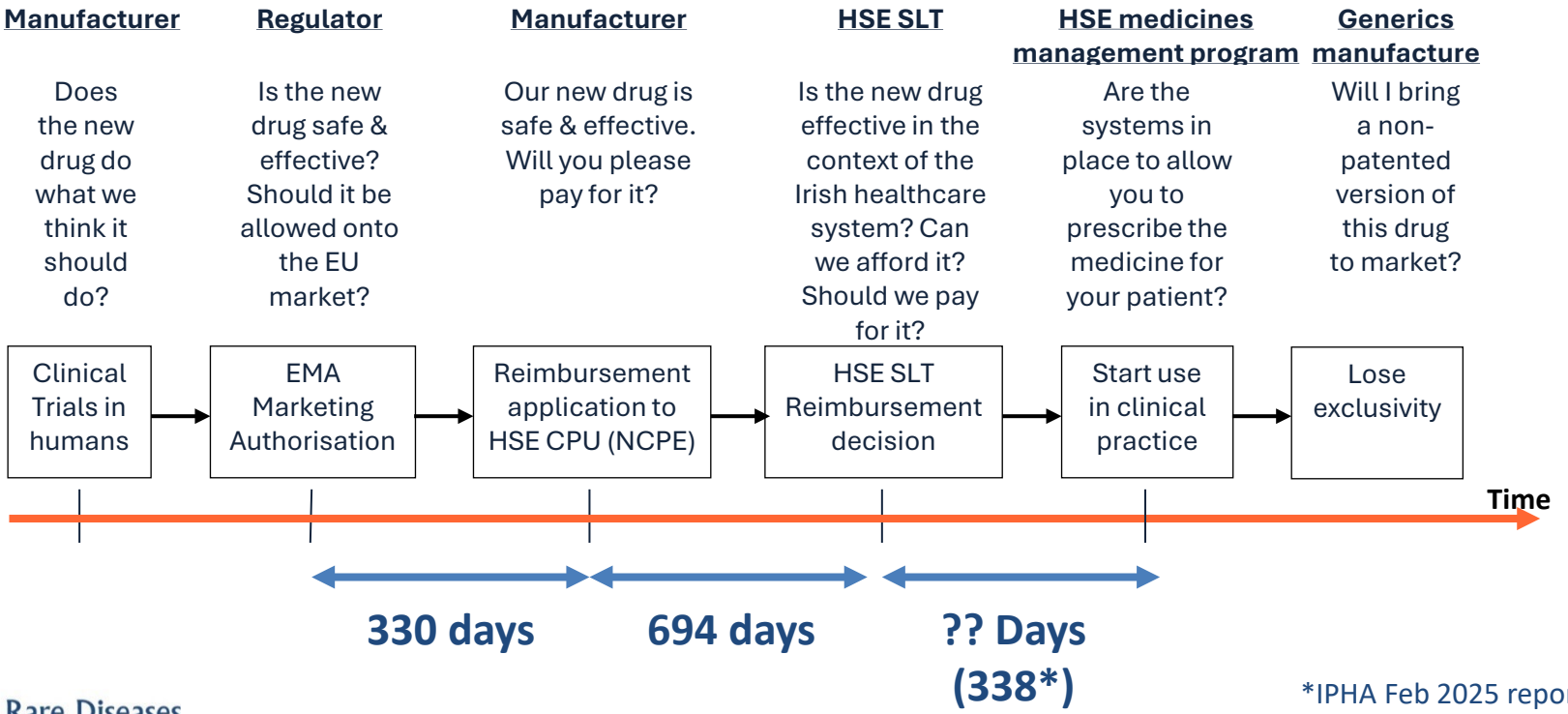
Take home message…

- Applications for reimbursement are not being made in a timely fashion (if at all)
- Time between EMA authorisation and HSE reimbursement is very long (~3 years at best)
- Market size is not the main reason that companies do not come to Ireland

A long way from our ask:

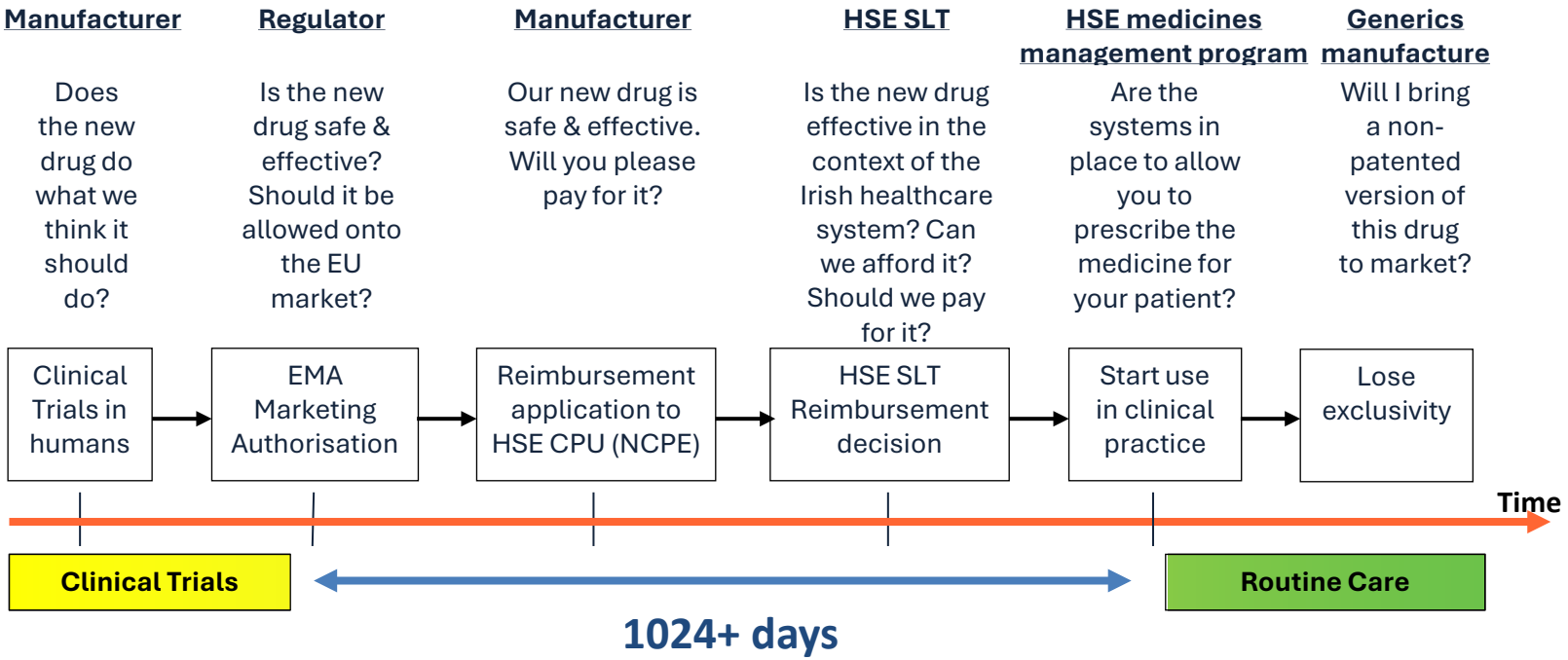
Access within 1 year of EMA authorisation

Key stages on medicines life cycle



*IPHA Feb 2025 report

Key stages on medicines life cycle



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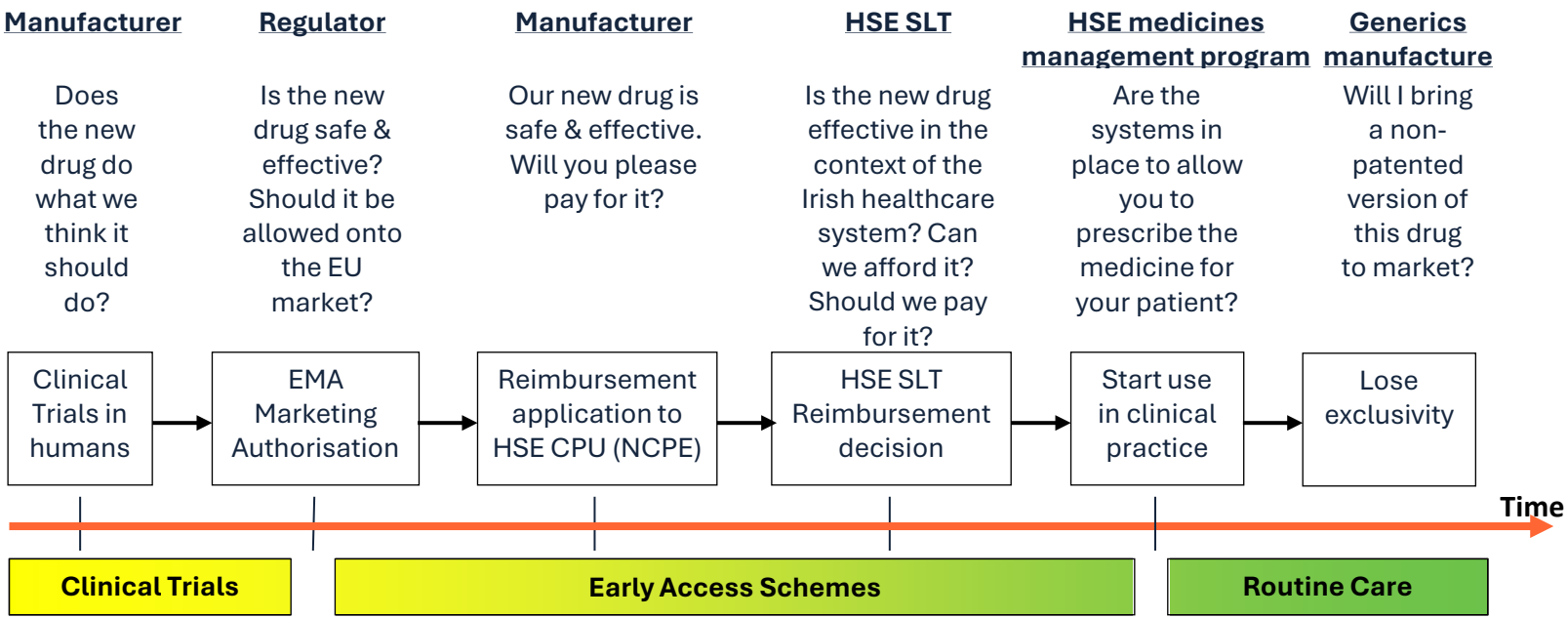
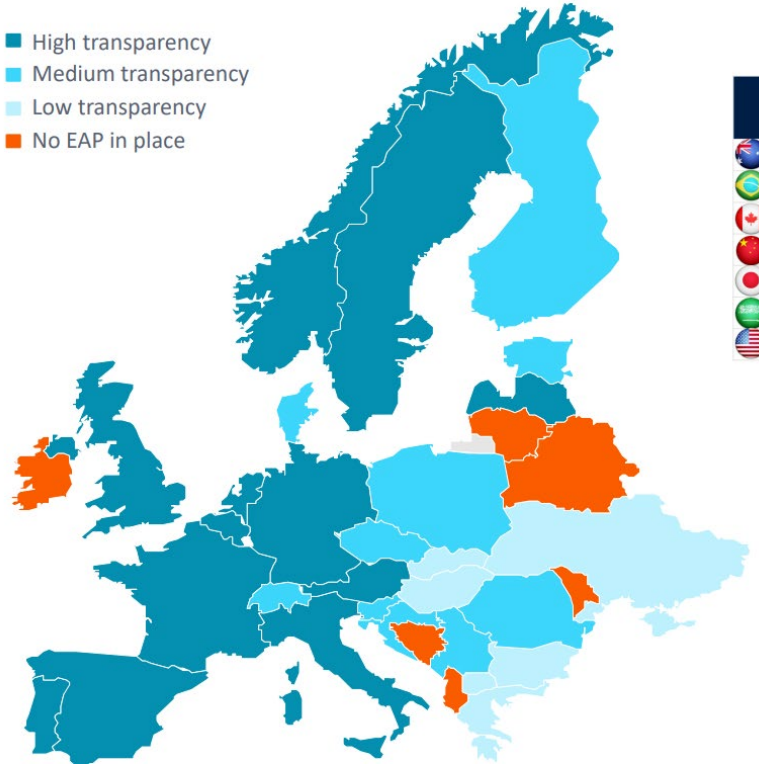


Early access schemes….



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

Programme for Government

- Publish and fund a new National Rare Disease Plan, improving access to orphan drugs

- This Government is committed to ensuring that patients have access to innovative new medicines and treatments as quickly as possible.
  - Commit to a review of the drugs reimbursement process
  - Investigate new methods for earlier reimbursement of certain treatments, including early access schemes for rare diseases

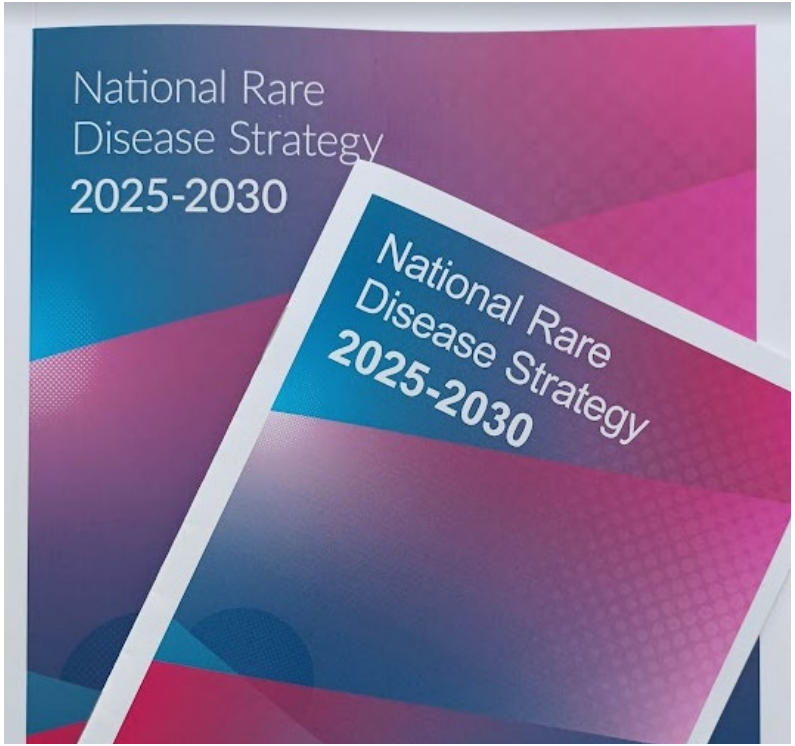
National Rare Disease Strategy

Orphan Medicines:

offer hope where, previously, little to none existed

aligns with the Programme for Government commitment to *review options for:*

- *earlier reimbursement of orphan medicinal products*
- *early access schemes for rare diseases*



National Rare Disease Strategy

Recommendation 8: Orphan Medicines	Suggested Lead Agency	Indicative Timeline
In relation to Orphan Medicines, the Steering Group recommends that ongoing efforts should be leveraged to maximise access and availability to treatments and technologies for people living with a rare disease and should be explored in full. This includes: a) Reviewing options for earlier reimbursement of certain treatments; b) Consideration of early access schemes for rare diseases, with a view to facilitating sustainable and consistent access to new medicines; c) Enhancing the State's engagement with international initiatives and Health Technology Assessment (HTA) collaboration aimed at improving people's access to medicines.	HSE/DoH	a-c) Subject to the progression of Programme for Government commitments

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.

Daily challenge of orphan medicines

Prof Susan O'Connell

- Setmelanotide (Imcivree®) –
- EMA authorised 16/7/21 –

……1,601 days later!

Daily challenge of orphan medicines

Prof Rachel Crowley

- Palopegteriparatide (Yorvipath®) –
- EMA authorised 17/11/23

……. 747 days later

- Burusomab (Crysvita ®)
- EMA authorised19/02/18

……. 2,844 days