Irish Pharmaceutical Healthcare Association

IPHA Submission to National Rare Disease Strategy for Ireland Consultation Paper.

What do you think are the greatest challenges faced by people living with rare diseases?

- Access to drugs and innovative technologies
- Access to specialist medical care and treatment
- Ability to take part in research opportunities, including clinical trials and studies.

Are there any other challenges missing from the previous question, that you would include in your top three challenges

1. Enhance patient involvement in approval of rare diseases medicines for reimbursement.

The NCPE have stated that the biggest issue with patient involvement is that most medicines evaluations do not have a patient submission dossier to accompany it. In 2021 there were ten submissions of evidence supplied by Patient Organisations to support the evaluation of medicines. This represents just 37% of all evaluations made by the NCPE that year. This issue is compounded when it comes to rare diseases where there may not be a patient group who can submit an evidence template on behalf of patients. The following measures could be considered:

- **Develop a stakeholder consensus on how to improve patient involvement.** Looking at best international practice, a consultation should be held with relevant stakeholders to reach a consensus on the best way to support patient advocacy groups in elevating the patient and rare diseases community's voice in the medicines reimbursement process.
- **Clarify the role and weighting attached to patient input in the decision-making process.** While there is consensus that patient evidence is a valuable data set when evaluating a new medicine, it is less clear how this data is evaluated and its impact on the decision-making process. The input of patients and clinicians should complement the scientific and economic evidence. Putting a measure on the patient experience will ensure it is an integral part of the decision-making process and can be documented.

2. Improve access to clinical trials in Ireland.

Clinical trials are critical in developing new treatments, but they also give patients access to sometimes life-saving medicines. However, in Ireland we need to do better in terms of attracting clinical trials as currently we are lagging some European countries with similar populations and economic performances. Denmark for example has nearly three times as many clinical trials when compared to Ireland.

3. Provide earlier screening and boost investment in genetic testing.

For instance, in the PAH therapy area, the following measures should be considered:

- A strategy to improve resource management: In the centre of excellence only 20% of staff time is allocated to PAH; if there were posts with 100% capacity, this would improve access and diagnosis. Roles should be provided on a full-time contract basis rather than short term.

- A strategy to improve education around PAH (for GPs/clinicians) could allow for reduced time to diagnosis and faster access to treatment. For instance, patients referred to Cardiologists are diagnosed quicker than when referred to Pulmonologists, as Pulmonologists have to request echo etc., and this causes longer waits and delays in diagnosis. Hospitalisation costs increase with delayed diagnosis hence a strategy towards early diagnosis could save money in the long term.

What can be done to address these challenges?

Rare diseases are among the hardest medical conditions to treat yet they will affect one in seventeen people in Ireland. As noted by the HSE's own model of care for rare diseases, Ireland has less access to innovative orphan therapeutics than other similar European countries. The most recent EFPIA Patient WAIT indicator stated that only 14% of recently EMA licenced orphan medicines were available on the Irish reimbursement list as of January 2024. This is well below countries with similar populations such as Austria, Denmark, Scotland, Slovenia, Greece and Switzerland.

IPHA feel a specific research stream to understand the reasons behind this would be warranted and would support the Department were it to commission this.

The problem is threefold as IPHA see it:

1. The Irish health system is not attracting applications from international pharmaceutical companies, particularly for orphan medicines, and this cannot exclusively be put down to Ireland's population.
2. Even when applications are forthcoming, they take on average nearly 6 months longer than non-orphan treatments from the time of application to patient availability.
3. There is constrained capacity throughout the reimbursement process in Ireland.

All of the above results in unequal access to medicines between patients in the general population and those with rare diseases when it comes to accessing the most recent developments in medicines. It also results in unequal access between Irish patients and similar health systems across Europe.

Ireland is the only European health system with no formal mechanism in place to have access to medicines in advance of the completion of assessment and a commercial contract. Given the timelines it is currently taking to achieve this, this is leaving many rare disease patients behind.

IPHA have previously given an undertaking to maintain patient supply of treatments even if the reimbursement application is not successful. This offer remains open and orphan treatment areas are the optimal starting point for such pathfinder schemes. Such a scheme would facilitate the needs of patients while assessment is ongoing allowing for the management of risks and uncertainties.

Secondly, international research indicates that health systems that have specific assessment and reimbursement pathways for orphan medicines and/or modified pathways for orphan treatments tend to have better levels of access to innovation.

Scotland and France are examples of countries with practical approaches to HTA whereby access is granted to patients pending final assessment and price agreement so that the final outcome can be linked to real world clinical experience.

We therefore believe the following could address the challenges in the current system:

1. Engage in structured dialogue with pharmaceutical companies to understand the reasons behind non-applications and/or to identify where unmet health needs arise in Ireland.
2. Establish an Orphan specific equivalent of the HSE Drugs Group. The Rare Disease Technology Review Group could fill this role.
3. Establish pathfinders for early or immediate access on EMA licensing via a new programme.
4. Review the methodology being applied to the assessment of orphan treatments. Unmet need, human value, solidarity and societal factors are not formally considered when assessing medicines in Ireland.

EFPIA Patients W.A.I.T Indicator - Orphan medicines				
Survey Year	**2020**	**2021**	**2022**	**2023**
Years examined	(2016-2019)	(2017-2020)	(2018-2021)	(2019-2022)
Overall ranking TTA	23/32	29/33	31/36	22/34
Western Europe ranking TTA	2nd last	Last	2nd last	4th last
Ireland TTA (days)	756	870	877	597
Average European TTA (days)	653	636	625	542

TTA=Time to Availability

IPHA Orphan medicines	**n=**	**Days from commencement of application to reimbursement**
2020	4	799
2021	11	955
2022	8	611
2023	4	759
2024	3*	737
2020 – 2024*	30	794

* Reimbursed as of 1st July