A CHARTER
FOR PATIENT INVOLVEMENT

MEDICINES ASSESSMENT & REIMBURSEMENT IN IRELAND

IPPOSI

FOREWORD

The development of this Charter for Patient Involvement is an important milestone for IPPOSI. As a patient-led platform, we have led multi-stakeholder discussions and identified consensus within the medicines assessment and reimbursement space over the past decade. In keeping with the IPPOSI strategic priorities for 2016-2020, and as part of our 2018 Annual Theme on 'Access to Medicines', we are 'actively advocating' for fundamental change in the current process.

This Charter, developed with a cross-section of the IPPOSI patient membership as well as the support of the Medical Research Charities Group (MRCG) and Rare Disease Ireland (RDI), provides a collective and holistic response to address the many patient concerns expressed about levels of transparency, accountability, quality and fairness in the Irish Health Technology Assessment (HTA) and post-HTA process.

This Charter is not for the faint-hearted. We want patient voices to be heard, we want patients to have seats at the table, and we want patients to be informed and supported in these roles. To this end, the text of the Charter encourages the relevant national agencies to take a number of steps towards making this a reality, including but not limited to:

- The establishment of a Patient Advisory Panel to guide and improve the patient involvement work of the HSE in this area.
- The creation of a joint database between the NCPE and HSE to allow patients to track the real-time progress of a new medicine within the assessment and reimbursement process.
- The development of a national patient education programme to educate and train more patients on the process used in Ireland to assess and reimburse medicines.
- The nomination of patient representatives to HSE assessment committees and decision-making groups.
- The introduction of a 'patient involvement' payment to compensate patients who actively participate on assessment committees and decision-making groups.
- The sharing of relevant HSE meeting agendas, minutes and supporting documents with patient communities and interested members of the public.
- The preparation of "Recommendation or Decision Explained" documents to detail the evidence used to arrive at final HSE decisions.
- -The elaboration of a new appeals process to offer a mechanism for participating stakeholders to evaluate the robustness of HSE decisions.

Laura Kavanagh
Research & Advocacy Officer, IPPOSI

Derick Mitchell
Chief Executive Officer, IPPOSI

SIGNATORIES 35 PATIENT ORGANISATIONS

SIGNATORIES

As organisations representing patients with common, chronic and rare diseases in Ireland, we believe our members should receive the latest innovative medicines and technologies – once they have been shown to be effective and clinically appropriate. We know from experience that receiving the right treatment, at the right time, can be life-changing or even life-saving.

That said, we recognise that national resources are finite and we acknowledge that tough decisions need to be made. We are ready to work in close partnership with national health agencies (the Department of Health (DoH), the Health Service Executive (HSE), and the National Centre for Pharmacoeconomics (NCPE) to ensure that patient input, patient evidence, and patient experience are taken into consideration during the assessment and reimbursement of new medicines in Ireland.

Our goal is a person-centred process with timely, consistent and transparent access to medicines for Irish patients.

On behalf of our patient members, we pledge to ensure that patient involvement in the process delivers a perspective which is independent, holistic and constructive, representing the voice of patients as a collective. In submitting input & evidence to inform NCPE assessments and HSE decisions, we promise to share a genuine, measured and evidence-based account of the patient experience. We agree to comply with requests to sign declaration of interests or confidentiality agreements.

We want to work in partnership with national agencies. This Charter is our shared vision for moving forward, together. To join us or to learn more about the Charter's principles and provisions contact **research@ipposi.ie**

FRIENDS

As organisations working on the topic of access to medicines across Europe and internationally, we lend our support to the initiative taken by IPPOSI patient members to identify more opportunities for potential patient involvement in the medicines assessment and reimbursement process in Ireland.

EPF European Patients Forum

EURORDIS RARE DISEASES EUROPE

HTAi HEALTH TECHNOLOGY ASSESSMENT INTERNATIONAL

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ABOUT

This Charter represents a call to action from Irish patient organisations to the Department of Health (DoH), the Health Service Executive (HSE) and the National Centre for Pharmacoeconomics (NCPE). It is an open invitation to these national agencies to explore how patients are currently involved in the process of assessing and reimbursing new medicines in Ireland, and how this can be improved.

The Charter contains several 'asks' – some of which are immediately implementable and set the minimum standard for a 'patients involved' process. Other 'asks' are more ambitious and serve as targets for the longer term. The over-riding message is clear – patients no longer accept the status quo, patients expect to be involved in health decision-making, and patient 'asks' are likely to gather momentum in the years to come.

Development

The proposal to develop a Charter was initiated by IPPOSI – a patient-led platform between patients, science and industry – which has long explored the role of patients in the medicines assessment and reimbursement process in Ireland. In proposing a Charter, IPPOSI seeks to collate many of the proposals put forward over the last ten years by patient organisations into one comprehensive, coherent and compelling document.

In 2017 and 2018, IPPOSI – in partnership with the MRCG – invited its members to attend three workshops on the topic of access to medicines. Two 'Drug Iceberg' reports[1] detailed conclusions from the workshop discussions, and several of the report's recommendations have informed various provisions contained within the Charter. A Third report is due in Spring 2019.

In 2018, IPPOSI members agreed on an Annual Theme of 'Access to Medicines'. An 11-member IPPOSI Board Sub-Group from patient, science and industry members and a network of Research & Advocacy Officers from eight Irish patient organisations were established to feed into the Charter development process (See Annex 5 of this document for respective membership). In May 2018, IPPOSI consulted the patient representatives of the EUPATI National Platform in Ireland, and later in the same month, IPPOSI invited three health technology assessment agencies (Canada, Scotland, and Sweden) – identified as leading on the topic of patient involvement – to share their experience with members via a webinar. In July 2018, IPPOSI invited its patient members to provide their comment and feedback on a first public draft of the Charter and to express their interest in becoming signatories. In September 2018, two IPPOSI representatives traveled with the NCPE to meet the Scottish Medicines Consortium (SMC) Patient Involvement Team and to observe the consideration of evidence at the SMC Patient and Clinician Engagement (PACE) meeting. In October and November IPPOSI engaged (separately) members of the HSE Corporate Pharmaceutical Unit (CPU) team and the NCPE on the draft text. The IPPOSI Board approved the final version of the Charter in December 2018 for publication and dissemination.

Acknowledgements

Special thanks to the members of our IPPOSI patient-led groups for their active role in drafting the Charter. We extend our gratitude to representatives of the national agencies who shared their experience of patient involvement, namely the Canadian Agency for Drugs and Technologies in Health (CADTH, Canada), Scottish Medicines Consortium (SMC, Scotland) and the Swedish Agency for Health Technology Assessment and Assessment of Social Services (SBU, Sweden). The Charter was prepared by Laura Kavanagh and edited by Derick Mitchell (IPPOSI) with the support of MRCG and RDI.

[1] http://www.ipposi.ie/our-work/health-innovation/access-to-medicines/

RATIONALE & PURPOSE

"We believe that patients have perspectives and experiences that can uniquely contribute to the decision-making process. We also consider that patients should have the same rights to contribute to HTA as other stakeholders, and this requires processes to enable effective engagement."

National Centre for Pharmaco-economices (NCPE) Public Consultation 'Patient Involvement in HTA', April 2018

Rationale

The process of assessing and reimbursing new medicines in Ireland has come under major scrutiny in recent years. As more and more innovative medicines are developed, many with expensive price tags, the national agencies in Ireland that are responsible for this area (DoH, HSE, NCPE) have struggled to keep apace with their respective duties and responsibilities. Tasked with making difficult decisions, the HSE is often portrayed as lacking transparency – at times seeming to respond to the most vocal campaigners and political pressure. This is a situation which is far from ideal, as it can lead to inequitable decision-making and the marginalisation of groups which are not in a position to advocate for themselves or do not agree with pressurised lobbying tactics. Patients are concerned by the unfolding situation and firmly believe that their active involvement in the process can lead to more timely and fairer decisions as well as help to manage the expectations of patient communities. Elsewhere, many national agencies (Canada, England, Scotland, Sweden) have encouraged and supported this involvement and are now firmly wedded to progressing patient involvement.

In recent years, Irish patient communities and national agencies have taken a number of important steps in the journey towards effective patient involvement in Health Technology Assessment (HTA) and post-HTA (reimbursement) decision-making. Patients have indicated their willingness to get involved, with many applying to undertake specific training and education to improve their understanding of how medicines are developed, assessed and made available to patients. National agencies have started to review their policies and processes, with many opting to carve out new and improved spaces for the patient voice. In particular, the NCPE has made notable improvements; including the introduction in 2015 of a template for patient submissions to assessment committees, the employment of a Stakeholder Engagement Lead for Rare Diseases in 2018, the ongoing delivery of a patient education module on health technology assessment (in collaboration with IPPOSI), and a public consultation in late 2018 to fine-tune the patient submission process.[2] NCPE is also transparent with respect to the timelines and status of assessments, and efforts are underway to make information more readily available and to communicate more proactively with patients. The HSE has been less forthcoming and challenges exist in ensuring patient involvement in decision-making at this level, as patients remain excluded from (and unaware of) the work of the Corporate Pharmaceutical Unit, the Drugs Committee and the Leadership Team. Initial work to involve patients in the area of Rare Diseases has begun within the Rare Disease Technology Review Group. The challenge now is for all stakeholders to come together to build upon this initial work, to bring all partners into the fold, and to align and manage our different expectations for the future.

[2] http://www.ncpe.ie/for-patients/

RATIONALE & PURPOSE

"If you're not involving patients, you're not doing HTA! It's that simple. Patient involvement improves the quality, relevance and value of HTA. It is difficult to conceive of HTA being conducted in a meaningful way in the 21st century without the involvement of patients."

Brian O'Rourke, Chair of the International Network of the Agencies for Health Technology Assessment (2017)

Purpose

Our goal is a multi-stakeholder, person-centred medicines assessment and reimbursement process for Ireland. We hope the Charter can contribute positively to the achievement of this vision. Specifically, we hope that the Charter will:

- provide patients with a basic understanding of their rights and responsibilities
- summarise – in a coordinated way – the range of patient requests and demands
- assist national agencies to deliver on their responsibilities to meaningfully include patients
- serve as a basis for the development of national agencies' strategies to involve patients & the public
- be incorporated into the education programmes used to train public sector health officials

With the right political, institutional and community commitment, we believe many of the Charter's provisions are achievable within the next five years. We acknowledge that some provisions may be ambitious in light of current available resources, but rather than fall at the first hurdle, we ask national agencies and patient communities to work together to call for adequate resources to implement these provisions, and to find creative interim solutions. We believe that success lies in collectively acknowledging that the meaningful involvement of patients in the assessment and reimbursement of medicines in Ireland will improve the decision-making process. Patients, the public and agencies themselves stand to benefit from the resulting social gain.

Disclaimer

In drafting the provisions of the Charter we have sought to identify opportunities for patient involvement within the existing medicines assessment and reimbursement process in Ireland. It is widely acknowledged that this process is not fit-for-purpose, and while we continue to call for immediate reform, we recognise that genuine and meaningful process change will take time to come into effect. In this context, the implementation of the Charter can be viewed as an interim goal.

Although some of our proposals may seem overly-prescriptive or repetitive, this is largely to accommodate the current process and ensure that the patient voice is represented at all major junctures within the existing process.

'Conflicts of Interest'

In the interests of impartiality, transparency and accountability, for any of the opportunities outlined within this document, 'expert advisers representing the patient and carer voice' should adhere to relevant codes of conduct and practice for declarations of interest. This would include the need for any patient/carer representative to recuse themselves from any involvement on an application for reimbursement in their respective disease/condition area (i.e. patient with a heart condition should not be reviewing an application for a new heart medicine etc.)

EXECUTIVE SUMMARY

STRATEGIC COMMITMENT

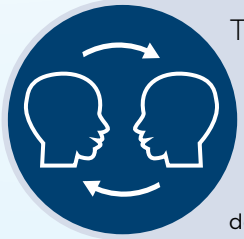
Patients call for a strategic commitment to advance their involvement

Patients are not an afterthought, patients are not another stakeholder, patients are not a box ticked. Patient involvement is an integral part of the work of national agencies engaged in the assessment and reimbursement of new medicines. Patients can be advisors, implementors, commentators. Whatever the role, we ask that our contribution is valued and that our added value is publicly recognised. We want the benefits of our involvement to be measured, and we hope that our partnership evolves as new opportunities arise.

EDUCATION & TRAINING

Patients need education, training and support to facilitate their involvement

Patients are not scientists, economists or clinical experts, but they do have a unique and lived experience to share. Patients need education and training to ensure that their voice is heard and represented. Patients need support to navigate the technical language and terminology, as well as to understand the methodology and measurements used. We ask that national agencies dedicate the resources needed to ensure that patients have the knowledge and the skills they need to interact with medicines assessment and reimbursement process.



TRANSPARENCY & COMMUNICATION

Patients demand access to information and regular communication

Patients need to be able to trust the decisions being made on their behalf. Patients denied access to a new medicine want to know that the assessors and decision-makers have arrived at their conclusions as a result of a transparent, accountable and evidence-based process. Patients want to have access to information; they want to know who makes decisions, how, and when. Patients want to be kept informed about developments and to be notified of the various opportunities to provide input and to become actively involved.

PATIENT & CLINICIAN EVIDENCE

Patients ask that patient + clinician evidence & input is systematically considered

Patients have an in-depth knowledge of the impact of their condition and their treatment on their daily lives. Patients know the outcomes which are important and which can offer a real improvement in their quality of life. Collecting and incorporating "patient-based evidence" into assessments results in better decision-making. Clinicians also have important information to share, including 'real-world' observations about how patients respond to a medicine. We ask that patient and clinician evidence & input complement scientific and economic evidence.

EARLY ENGAGEMENT

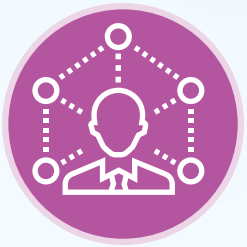
Patients want early and sustained engagement across the process

Patients in Ireland currently have limited patient involvement opportunities during the entire assessment and reimbursement process. Patients believe that there are missed opportunities and we call for the patient voice to be represented across the entire process to ensure that the methodology employed and the values used are truly patient-centric. Patients can help with early scientific advice, assessment scoping, evidence development, early access and much more. Patient perspectives should inform the methodology employed and the values used by assessors and decision-makers.

RIGHT TO APPEAL

Patients request the right to appeal

Patients want to contribute to recommendation-shaping and decision-making. Patients should be invited to formally comment on draft assessment reports and reimbursement decisions prior to publication – thereby completing the involvement circle from process start to finish. Patients should be able to challenge the process and we call for an appeal process to be developed to ensure an oversight framework is in place for final assessment and reimbursement decisions.



PATIENTS CALL FOR A STRATEGIC COMMITMENT TO ADVANCE THEIR INVOLVEMENT

1.1. Led by the Department of Health (DoH), a National Medicines Strategy should be developed with national agencies (NCPE, HSE) and patients participating as equal partners in setting out a vision for ensuring sustained and expanded access to new medicines in Ireland.

1.2. In this Strategy, a vision for developing patient involvement in the Health Technology Assessment (HTA) and post-HTA process should be outlined, which sets minimum standards and clear targets for national agencies to achieve.

1.3. Independent of a National Medicines Strategy, a process for the assessment and reimbursement of new medicines should be agreed between relevant national agencies (DoH, NCPE, HSE), with an emphasis on consistency, transparency and clear timelines. The agreed process should be published as a stand-alone process, separate to any document detailing the current DoH-HSE-IPHA agreement. Extensive consultation should be carried out with all key stakeholders, including patients, to identify existing process shortcomings and to propose genuine opportunities for patient involvement.

1.4. As the national framework takes shape, an in-house exercise should be completed by each national agency/centre/programme (HSE, NCCP, NCPE) to identify their own patient involvement objectives for the short, medium and long-term. The agreed objectives should be published, and ideally, passed through a public consultation.

1.5. Patient representatives should be invited onto existing governance and oversight bodies within national agencies (DoH, HSE) to provide ongoing advice and to monitor organisational strategy, policy, process and practice against patient priorities. In particular, the HSE should consider establishing a dedicated 'Patient Advisory Panel' to drive forward a strong patient involvement agenda as other agencies have done internationally. **To view what we believe a Patient Advisory Panel could achieve visit Annex 2 of this document.**

1.6. Funding to plan and undertake effective patient involvement should be allocated from the budgets of national agencies (HSE, NCPE). Resources must be sufficient to ensure the meaningful involvement of patients, as well as the timely and quality delivery of patient involvement objectives.

1.7. Training on how to involve patients (and the public) should be mandatory for relevant national agency staff (DoH, HSE, NCPE). Specific training should be provided to any relevant Chairs and Vice-chairs of Committees on which patient/carer representatives are members and seen as equal partners.

[3] http://www.ipposi.ie/wp-content/uploads/2017/09/Ice-Berg-Report-FINAL.pdf & http://www.ipposi.ie/wp-content/uploads/2018/02/IPPOSI_MRCG-Drug-Iceberg-2.0.pdf

PATIENTS NEED EDUCATION, TRAINING AND SUPPORT TO FACILITATE THEIR INVOLVEMENT

2.1. A nationally-recognised patient education and training programme on medicines assessment and reimbursement should be designed and supported by national agencies (NCPE, HSE) and education partners in order to grow a cohort of patients with a broad understanding of the process used to assess and reimburse new medicines in Ireland. There are individual education programmes to leverage from, including the IPPOSI patient education[4] module on HTA which was delivered with the support of the NCPE, TCD & HIQA and the EUPATI Training Course.[5]

2.2. A general, one-day, basic training on the HTA and post-HTA process in Ireland should be provided annually to patient organisations, jointly delivered by the NCPE, the HSE and HIQA. This training should include advice on how organisations can best represent the voice of patients across the entire process, as well as provide guidance on how to write good patient submissions. Peer-to-peer learning between patients should be encouraged.

2.3. The NCPE currently invite patient organisations to complete a database registration form[6] as part of an exercise to identify Irish patient organisations with an interest in medicines assessment. This contacts database should be further developed into a common HSE/NCPE database (see point 3.3. below) to generate mailing lists and be used to supply patient organisations with regular updates and information (including the details of upcoming trainings, the progress of new medicines through the assessment & reimbursement process).

2.4. The 'Patient Involvement Team' established within the NCPE should be resourced to provide additional administrative and logistical support to patients making written submissions or oral presentations during the assessment process. The Team should prepare plain-English guidance on the HTA process in general and on the specific role of the patient. A copy of previous patient submissions and/or presentations should be available for download to serve as a reference point for patients with little or no experience of the HTA process. The Team should complete a bi-annual review of the education and support provided to patients and should publicly communicate their learnings from 'exit surveys' conducted with patients who have engaged with the process.

2.5. The existing HSE policy on reimbursement of patient expenses should be revisited to provide for a 'patient involvement' payment. Compensating patients acknowledges the true value of their contribution, recognises the often-extensive personal time dedicated to the completion of involvement duties, and enables individuals who for financial reasons might otherwise be unable to participate. Patients should only be compensated for patient representation (e.g. participating on committees) and not for consultation activities (e.g. making written submissions).

[4] http://www.ipposi.ie/our-work/education/patient-education-programme
[5] https://www.eupati.eu/eupati-training-course
[6] http://www.ncpe.ie/for-patients/template-guidelines-and-tip-sheet

PATIENTS DEMAND ACCESS TO INFORMATION AND REGULAR COMMUNICATION

3.1. A proactive communication strategy should be publicly communicated by the NCPE Patient Involvement Team to reach out to patient communities with a likely interest in a new medicine being assessed (identified as part of Charter provision 2.3). As appropriate, the strategy should be continued by the HSE during the post-HTA process to avoid 'an information gap'.

3.2. A list of medicines due for assessment and/or reimbursement in the coming six-month period should be jointly published by the NCPE and HSE. This list should be disseminated to all patient organisations registered on the NCPE-HSE database of contacts every quarter. It should include a list of medicines pending NCPE assessment and a patient-friendly summary about each new medicine. It should also detail a calendar of HSE decision-making meeting dates (HSE Drugs Committee and Senior Leadership Team), as well as the draft agenda of new medicines to be discussed at each meeting.

3.3. A database of medicines undergoing (or completing) the assessment and reimbursement process should be jointly maintained by the NCPE and the HSE to allow patient organisations and other stakeholders to track the real-time progress of a medicine both while it is under consideration and after a final decision has been made. This database should be publicly accessible. Final assessment reports (NCPE) and final reimbursement decisions (HSE) should be available for download from the database. The database of medicines should have the capacity to perform statistical analysis (including, but not limited to the number of medicines recommended, the number of medicines reimbursed, the length of time taken to complete an assessment, the length of time taken to reach a decision). Alerts about new content on the database of medicines should be in place to notify the NCPE-HSE database of contacts (identified as part of Charter provision 2.3).

3.4. A model for facilitating patients (and the public) to observe and participate in HSE Drugs Committee should be developed, with the possibility of hosting deliberative or sensitive aspects of the meeting in a private session. In the meantime, a copy of the minutes of the HSE Drug Committee meetings should be available for public review as soon as possible. Redaction should be responsibly employed by the HSE and should not be used to restrict access to information which belongs in the public domain. A list of meeting members should be included in the minutes and their interests should be clearly declared.

3.5. (Distinct from Charter provision 6.2) The NCPE currently shares a summary of its assessment reports 48 hours in advance of publication with patient organisations who have made a corresponding submission. This practice should be replicated for all HSE reimbursement decisions which should be shared with relevant patient communities at least 48 hours before public publication. To reach a broader audience, final reports and decisions should be later disseminated using social media.

3.6. The NCPE and the HSE should improve the accessibility and functionality of their websites to allow the public to find schedules of upcoming meetings, minutes of past meetings, and to search a database of assessment reports and reimbursement decisions (Charter provisions 3.2, 3.3, 3.4)

PATIENTS ASK THAT PATIENT AND CLINICIAN EVIDENCE AND INPUT IS SYSTEMATICALLY CONSIDERED

4.1. A commitment to improve the collection, evaluation and appropriate use of 'patient-based evidence' (see Annex 3) in assessment and reimbursement decision-making should be made by the HSE.

4.2. For each medicine considered, the HSE should provide a plain-English "Recommendation Explained" or "Decision Explained" document for patients and the public. The document should summarise what evidence was made available (including any patient or clinician evidence and any patient submissions or presentations) and how it was used to inform or influence the final assessment report and/or reimbursement decision. This exercise should aim to give patients a meaningful understanding of the value and weight being given to their evidence.

4.3. For each assessment, to determine the degree of unmet medical need, the HSE should require evidence to be obtained from at least two independent clinicians with expertise in the specific disease area (rather than in the broad speciality area). Patients should be involved in the identification of clinical experts and international expertise should be sought in cases where specialists are lacking at the national level.

4.4. Complementing Charter provisions 2.3., 3.1 and 3.2, the HSE should establish a network of clinical experts (in addition to the clinical representatives sitting on assessment and decision-making committees). Clinical experts should be invited to comment on submissions undergoing assessment which are in their speciality area. The HSE should provide bespoke training and support for clinicians to improve understanding of the assessment process and the impact of their input on reimbursement decisions.

4.5. A new procedure should be established to permit HSE assessors and decision-makers to call upon patients with specific, relevant expertise to appear before them and to present evidence which reflects the patient perspective.

[7] For additional reading on any of the concepts discussed – K. Facey et al. (eds.), "Patient Involvement in Health Technology Assessment", 2018, Chapter 4 "Patient-Based Evidence in HTA"

PATIENTS WANT EARLY AND SUSTAINED ENGAGEMENT ACROSS THE PROCESS

5.1. A number of seats on the HSE Drugs Committee should be reserved for appropriately trained and impartial patient representatives. Patient representatives should be treated as equal partners, with full access to information granted and compensation received for time and expenses incurred (Charter provision 2.5). Patient representatives should be asked to sign confidentiality agreements and to recuse themselves in the event of any conflicts of interest.

5.2. Where appropriate, a transparent recruitment process, with the inclusion of patients on the recruitment panel, should be carried out to appoint patients to national agency roles and responsibilities. In certain circumstances, national agencies may approach patient organisations to assist with the identification and appointment of patients, in which circumstance a recruitment or election process may be conducted by the patient organisations.

5.3. A new procedure should be established to allow patients to request to make an oral presentation during a HSE Drugs Committee meeting. Decisions on whether to positively or negatively receive a request should be taken based on a clear criteria and details should be provided in the final HSE reimbursement decision.

5.4. Opportunities for patient involvement should be created at each stage of the HTA and post-HTA process. For example, the current horizon scanning template for new medicines submitted by companies to the HSE, NCCP, NCPE as part of the IPHA agreement, is completely removed from patients. Efforts should be made to increase the accessibility of information on emerging and new medicines for patients and carers. In this regard, Ireland should join the joint-horizon scanning initiative as part of the BeneluxA initiative.

PATIENTS REQUEST THE RIGHT TO REVIEW AND APPEAL

6.1. An appeal process should be developed to ensure an oversight framework is in place for HSE decisions. The appeal process should be designed with input from all stakeholders, including patients. Any stakeholder participating in the assessment and reimbursement process should be able to initiate an appeal within an agreed timeframe and scope. The appeal process should include the opportunity for patients to provide written submissions as well as participate in oral hearings. Patients should be nominated to appeal panels, appeal panel meetings should be held in public, and associated documents should be publicly available in advance of the relevant appeals meeting. The appeal process should adhere to strict (realistic) timeframes – for instance, appeals might aim to be concluded within a period of three months, and would need to address the release of commercially sensitive information into the public sphere.

6.2. Draft assessment reports and draft reimbursement decisions (HSE) should be shared with relevant patient communities two weeks prior to their publication. Patients should be invited to provide written feedback on the drafts. All patient feedback received should be appended, in full, to the final assessment report and the final reimbursement decisions sent for publication.

GLOSSARY

CADTH Canadian Agency for Drugs and Technologies in Health

CPU Corporate Pharmaceutical Unit (at the HSE)

DoH Department of Health

EUPATI European Patients' Academy for Therapeutic Innovation

HIQA Health Information and Quality Authority

HrQoL Health-related Quality of Life

HSE Health Service Executive

HTA Health Technology Assessment

IPHA Irish Pharmaceutical Healthcare Association

IPPOSI Irish Platform for Patient Organisations, Science and Industry

MRCG Medical Research Charities Group

NCCP National Cancer Control Programme

NCPE National Centre for Pharmacoeconomics

NICE National Institute for Health and Care Excellence

PPI Public and Patient Involvement

RDI Rare Disease Ireland

SMC Scottish Medicines Consortium

SBU Swedish Agency for Health Technology Assessment& Assessment of Social Services

TCD Trinity College Dublin

USEFUL RESOURCES

IPPOSI & MRCG Drug Iceberg Report 1.0;
http://www.ipposi.ie/wp-content/uploads/2017/09/Ice-Berg-Report-FINAL.pdf

IPPOSI & MRCG Drug Iceberg Report 2.0;
http://www.ipposi.ie/wp-content/uploads/2018/02/IPPOSI_MRCG-Drug-Iceberg-2.0.pdf

European Network for Health Technology Assessment;
https://www.eunethta.eu/

Health Technology Assessment International, Patient and Citizen Involvement Group;
https://htai.org/interest-groups/pcig/

Health Technology Assessment International, Values and Quality Standards for Patient Involvement in HTA, 2014;
https://htai.org/wp-content/uploads/2018/02/PCISG-Info-PosterValuesandStandards-30-Jun14.pdf

The International Network of Agencies for Health Technology Assessment;
http://www.inahta.org/

EUPATI Guidance for patient involvement in HTA;
https://www.eupati.eu/health-technology-assessment/guidance-for-patient-involvement-in-hta

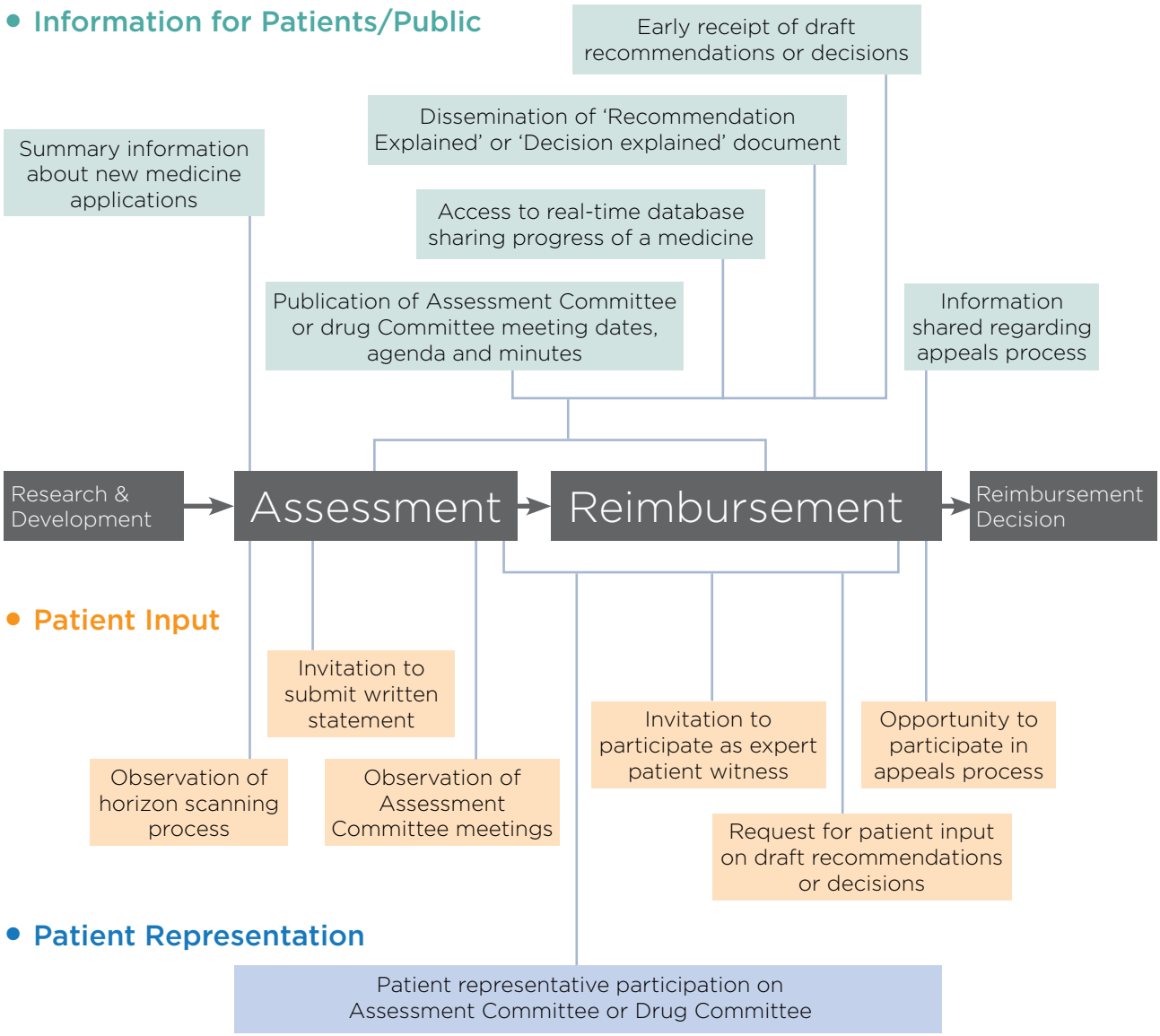
ISPOR Health Technology Assessment Central: A repository of resources;
https://www.htacentral.org/

ISPOR Patient Representative Roundtables;
https://www.ispor.org/member-groups/councils-roundtables/patient-council/patient-representatives-roundtables

Patient Involvement in Health Technology Assessment;
Facey, Karen, Ploug Hansen, Helle, Single, Ann (Eds.), 2017

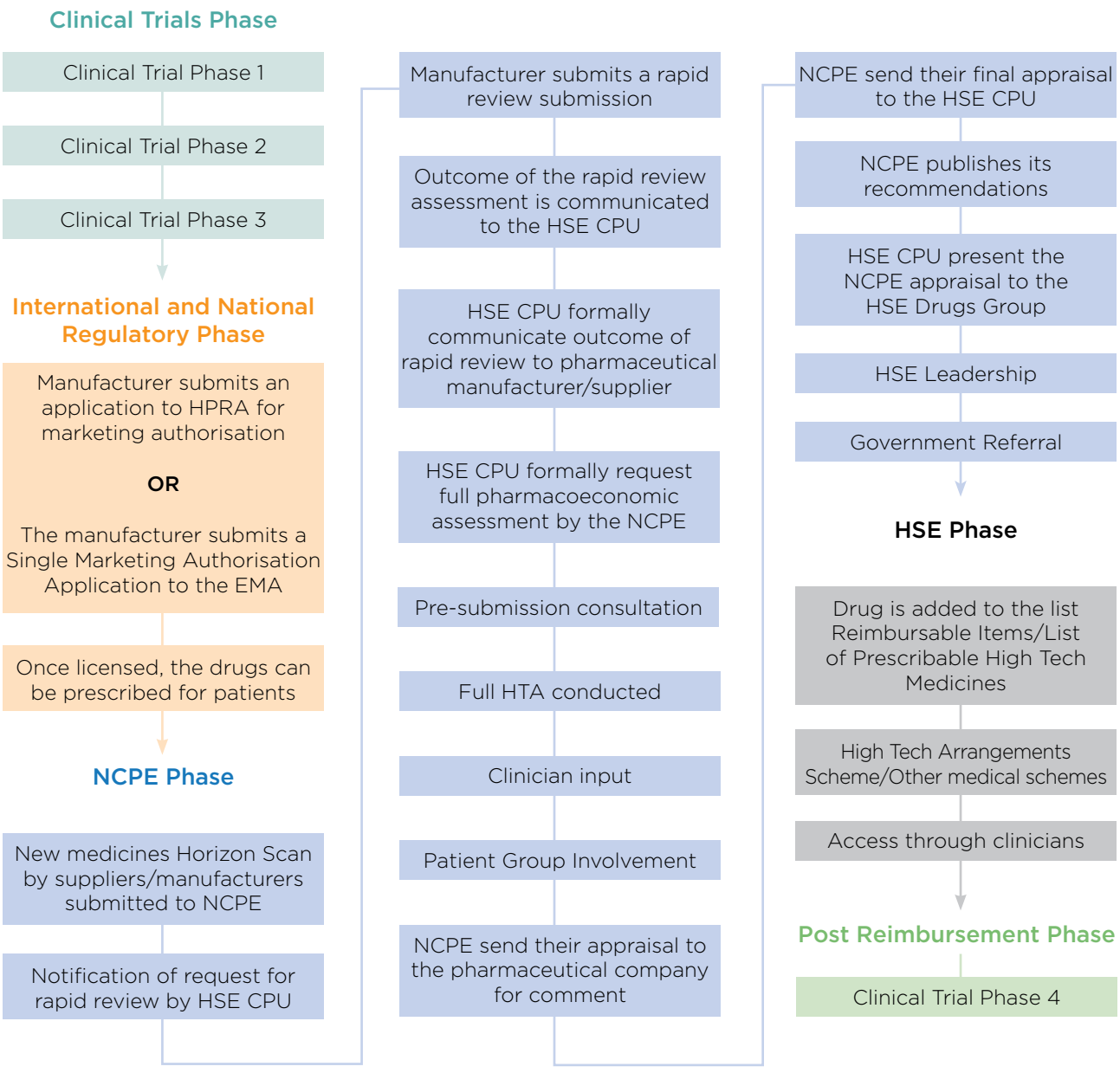
ANNEXES

ANNEX 1A: PATIENT INVOLVEMENT OPPORTUNITIES WITHIN THE HTA AND POST-HTA PROCESS



ANNEX 1B: OVERVIEW OF CURRENT IRISH HTA AND POST-HTA PROCESS[8]

Supply and Reimbursement of Drugs: From Clinical Trial to Patient Access



[8] Replicated from Annex 1 of the IPPOSI-MRCG 'Drug Iceberg Report' (2017) - http://www.ipposi.ie/wp-content/uploads/2017/09/Ice-Berg-Report-FINAL.pdf

ANNEX 2: WHAT IS A PATIENT ADVISORY PANEL (PAP)?

As the patient involvement agenda gathers momentum, more and more national agencies dealing with medicines assessment and reimbursement are opting to seek strategic advice from the very people they seek to include – patients. In many instances, mechanisms commonly known as Patient Advisory Panels or Committees are being established.

Scottish Medicines Consortium (SMC), Patient Involvement Network (PIN) Advisory Group

In 2015, SMC established the Patient Involvement Network (PIN) Advisory Group[9] comprising of four patient representatives, four public partners, an SMC committee member and a local health board representative. The Group meets for half a day, in person, three times a year. The purpose of the Group is to have patients advise the SMC on their patient involvement work. The Group has worked with the SMC to provide patients with advance notice of assessment recommendations, to revise and improve the template for patient submissions, to put in place mentoring for patients participating in PACE, and to clarify the role of public partners.

Canadian Agency for Drugs and Technologies in Health (CADTH), Patient Community Liaison Forum

Since 2013, CADTH has hosted a Patient Community Liaison Forum with the purpose of fostering communication between the national agency and patient communities in Canada. The Forum brings CADTH together with senior management from the four largest patient umbrella groups. The groups are not disease or condition specific and they represent a broad geographical area. The Forum meets once a quarter, via teleconference and members primarily share information and troubleshoot priority concerns from patient organisations. Proposals have recently been put forward which call for the Forum to be converted into a Patient Advisory Committee. The Committee would seek to incorporate a broader representation of patients including the voices of hard-to-reach or more marginalised communities such as carers, seniors, migrants. The Committee purpose would also change to focus on strategy and process change rather than communication and information. The Committee would be engaged to prepare reports on topics of thematic interest to CADTH or to review the success of an internal process or policy. Different to the PIN in Scotland, the Committee would focus on assisting the general work of the organisations rather than the specific work on patient involvement.

[9] https://www.scottishmedicines.org.uk/about-us/public-involvement/public-involvement-network-advisory-group

https://www.cadth.ca/cadth-patient-community-liaison-forum

ANNEX 3: WHY IS PATIENT-BASED EVIDENCE IMPORTANT FOR HTA?

The focus of the Charter is on patient involvement. We do not wish to confuse patient involvement with patient-based evidence. Involvement and evidence serve two distinct purposes, and while they may complement each other, one does not – and should not – replace the other.

That said, we believe that considered inclusion (and improved measurement) of patient-based evidence would make an important improvement to the assessment process in Ireland, and the resulting reimbursement decisions (i.e. in addition to clinical & economic evidence). Indeed, encouraging the measurement of patient-based evidence and the promotion of its use in sound healthcare decision-making has been put forward as an important 'third pillar' of quality in healthcare alongside clinical effectiveness and patient safety.[10] In addition Sweden, Denmark, Scotland and Germany are able to use patient-based evidence in HTA.[11]

What is patient-based evidence?

Patient-based evidence refers to the collection of patients' preferences, needs, experiences, perceptions or attitudes about their care or health.[12] Patient-based evidence can be collected through completing new research or a review of existing research studies. Examples include patient experience surveys, patient narrative data, evidence on health-related quality of life (HrQoL) measures, etc. Patient-based evidence should be peer-reviewed and grounded in robust scientific, mixed-method methodologies.

Challenges

The importance of using patient-based evidence in policy development is slowly changing in Ireland:

- The 2016 report of Major Trauma Audit compiled by the National Office of Clinical Audit recommended that patient-reported outcome measures should form part of future audits.[13]
- The first Value set based on Irish utility values for the EQ-5D-5L was published in 2018.[14]

However, challenges can arise about the use of patient-based evidence in assessment & decision-making due to the sometimes rigid application of evidence hierarchies designed for clinical research. The debate and lack of consensus between various stakeholders about what should be measured or how to measure, has muddied the waters in relation to the perceived value of patient-based evidence. Consensus among experts appears to be that it is preferable to invest in measurement of HRQoL only if a minimum level of quality of that measurement is guaranteed. So the current question in Ireland is 'what is the minimum level of quality that would be acceptable by our decision-makers'?[15]

Patient Registries

Patient registries can provide a useful tool/source of data for conducting patient-based evidence. The EMA recently qualified their opinion on the use of registries for the conduction of long-term safety and effectiveness studies that support both regulatory and reimbursement decision-making (EMA 2017).[16] However, patient organisations are often shouldered with the significant burden of resourcing and sustaining a mechanism/tool for collecting patient-based evidence (e.g. through a patient registry). Patient communities also 'lose out' due to the absence of a registry/tool for their condition.

[10] Institute of Medicine. Crossing the quality chasm: a new health system for the 21st century. Washington, DC: National Academy Press, 2001.
[11] – K. Facey et al. (eds.), "Patient Involvement in Health Technology Assessment", 2018, Chapter 4 "Patient-Based Evidence in HTA"
[12] Staniszewska S. et al. The PRIME project: developing a patient evidence-base, 2010, Health Expectations; Vol. 13, 312-322. https://doi.org/10.1111/j.1369-7625.2010.00590.x
[13] https://www.noca.ie/wp-content/uploads/2015/04/NOCA-MAJOR-TRAUMA-REPORT-2016.pdf
[14] Hobbins, A., Barry, L., Kelleher, D. et al. PharmacoEconomics (2018) 36: 1345. https://doi.org/10.1007/s40273-018-0690-x
[15] http://www.ipposi.ie/wp-content/uploads/2018/12/PCOMs-outcome-report-final-v3.pdf
[16] https://www.ema.europa.eu/documents/regulatory-procedural-guideline/qualification-opinion-european-cystic-fibrosis-society-patient-registry-ecfspr_en.pdf

ANNEX 4: INTERNATIONAL BEST PRACTICE IN INVOLVING PATIENTS IN HTA AND POST-HTA PROCESSES

PATIENT REPRESENTATION

Patient representatives on governance bodies

CANADA:
Canadian Agency for Drugs and Technologies in Health (CADTH) nominates two public representatives to its Board of Directors which provides strategic direction and guidance on how the organisation can best make informed decisions about the optimal use of health technologies.

SWEDEN:
Swedish Agency for Health Technology Assessment and Assessment of Social Services (SBU) invites a patient representative to sits on its Board which reviews reports from project groups tasked with assessing the value, risks and costs of various health and social services.

Patient representatives on advisory bodies

CANADA:
CADTH have a Patient Community Liaison Forum (see Annex 2) which provides a patient perspective on topics of interest to CADTH, including but not limited to how the organisation engages with patients.

ENGLAND:
National Institute for Health and Care Excellence (NICE) have a Citizen Council comprising of 30 members of the public which advise on a range of moral and ethical issues arising during the development of guidance about new services and treatments.

SCOTLAND:
Scottish Medicines Consortium (SMC) have a Patient Involvement Network (PIN) Advisory Group (see Annex 2) which advises on how to best involve the public, patients, their families and carers in the work of the SMC.

Patient representatives on committees making recommendations or decisions

CANADA:
CADTH's Canadian Drug Expert Committee (CDEC) has one public (lay) member, and the pan-Canadian Oncology Drug Review (pCODR) Expert Review Committee (pERC) has two patient members.

ENGLAND:
NICE have at least one public (lay) member on each of their four Technology Appraisal Committees and three public (lay) members on their Highly Specialised Technologies Evaluation Committee.

SCOTLAND:
SMC have three public partners on their Assessment Committee.

PATIENT INPUT

Patient feedback on draft recommendations or decisions

CANADA:
CADTH invites feedback on initial recommendations about new cancer treatments from invited stakeholders, including patient advocacy groups for a period of 10 days.

ENGLAND:
NICE presents all final draft guidance for consultation with registered stakeholders for a period of six weeks.

Patient submissions

CANADA:
CADTH share a compilation of all patient submissions received as part of a particular assessment, an example can be found here.

ENGLAND:
NICE provide a detailed response to each point raised by stakeholders submitting evidence or comments as part of the final guidance report, an example can be found here.

SCOTLAND:
SMC prepares to pilot the introduction of a 'SMC Decision Explained' document in late 2018 which will outline the rationale for arriving at a particular decision, an example can be found here.

PATIENT INFORMATION

Patient/public observation of committee meetings

ENGLAND:
NICE authorise public observation of a range of meetings, including advisory committee meetings, technology appraisal appeal hearings, and board meetings.

SCOTLAND:
SMC Assessment Committee meetings are open for public observation.

Patient/public access to committee minutes, agendas and documents

ENGLAND:
NICE publishes the minutes, agendas and papers relating to advisory committee meetings and technology appraisal committee meetings.

SCOTLAND:
SMC publishes the minutes of each SMC Assessment Committee meeting.

OTHER

Patient involvement payment

ENGLAND:
The National Health Service (NHS) produces detailed guidance on the reimbursement of expenses for patients and it introduces a 'patient involvement payment'.

Appeals process

ENGLAND:
NICE has developed an appeals process which allows any of the consultees participating in an assessment to appeal the final draft guidance from an assessment committee.

ANNEX 5: IPPOSI MANDATE AND MEMBERSHIP

The Irish Platform for Patient Organisations, Science and Industry (IPPOSI) is a patient-led organisation that works with patients, government, industry, science and academia to put patients at the heart of policy and innovations. IPPOSI seeks to build consensus among member groups on existing and emerging health issues and policies.

IPPOSI membership[17] includes over 100 patient organisations and medical charities, over 250 individuals from a scientific/clinical and healthcare background and 22 healthcare industry companies.

IPPOSI has been co-funded by the Health Research Board (HRB) on behalf of the Department of Health since 2007. HRB co-funding, accounts for approximately 25% of IPPOSI annual income whilst the remainder is generated from a combination of leveraged public EU and national grants as well as industry member subscriptions and sponsorships. 2018 ratio of public:private income stands at 50:50.

The development of the Charter has sought to involve and consult IPPOSI patient members with the support of the Medical Research Charities Group and Rare Disease Ireland. The 11-member IPPOSI Board Sub-Group which oversees the progress of IPPOSI work in the area of access to medicines has four industry members.

IPPOSI Annual Theme Sub-Group of IPPOSI Board members

- Ava Battles, MS Ireland
- Tomas Carroll, Alpha-1 Foundation, Ireland
- Colm Fahey, Roche
- Prof. Anne-Marie Healy, Trinity College Dublin
- Neil Johnson, Croí Heart & Stroke Charity, Ireland (Committee Chairperson)
- Robert O'Connor, Irish Cancer Society
- Karen O'Keefe, Pfizer
- Tom O'Leary, ICONplc
- Susanne O'Reilly, Novartis
- Jean Saunders, University of Limerick

IPPOSI Research & Advocacy Network Members

- Fiona Aherne, Debra Ireland
- Harriet Doig, MS Ireland
- Caitriona Dunne, Fighting Blindness
- Paul Gordon, Irish Cancer Society
- Clair Kelly, Muscular Dystrophy Ireland
- Dr. Bernadette Rock, Alzheimer Society of Ireland
- Kathryn Reilly, Irish Heart Foundation

Patient Members of the Irish EUPATI National Platform

- 12 Irish-based fellows of the EUPATI Expert Patient Training Course
- 19 graduates of the IPPOSI Patient Education Programme

[17] http://www.ipposi.ie/about-us/membership

IPPOSI

IRISH PLATFORM FOR PATIENTS ORGANISATIONS, SCIENCE & INDUSTRY

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