HTA Policy and Methods Review Consultation 2- Options Paper
Submission from Alexion, AstraZeneca Rare Disease, Australasia

Executive summary

Alexion, AstraZeneca Rare Disease, Australasia ("Alexion") welcomes the opportunity to provide comments in response to the Health Technology Assessment (HTA) Policy and Methods Review Consultation 2 options paper (the "options paper").

Alexion is a global biopharmaceutical company and rare disease leader with over 30 years of experience in developing first-in-class therapies for the treatment of rare diseases. Alexion works closely with governments and health agencies around the world, including in Australia, to ensure rare disease patients have access to equitable, timely and sustainable health care.

Alexion supports reform to the HTA process to better achieve the objective of equitable, timely and sustainable access to medicines for Australians living with a rare disease. At present, the HTA system is only partially delivering on this goal, with access to rare diseases often taking longer than comparable countries or missing out entirely on rare disease treatments available overseas.

We are committed to working with the government on improving the HTA system.

The options paper presents industry, patients, and the broader community, with a roadmap with some welcome changes. It does, however, leave many necessary reforms either subject to further processes or with options that are unclear in their impact which could have unforeseen consequences for patient access to rare disease therapies.

It also presents options that would negatively impact on investment in Australia by the global pharmaceutical industry, resulting in less choice for Australian patients and prescribing physicians compared with other comparable nations. Such options fail to reflect the realities of how the global marketplace works in the medicines sector and the interconnectedness of decision-making based on country health and reimbursement policy settings. This must be avoided if Australians are to have equitable and universal care from the government-funded health system.

Alexion's analysis of the critical issues for rare disease patients and their access to medicines is presented in this submission.

Current HTA – important features to retain.

There are many features of the current HTA system that have been important in delivering access to new therapies for rare diseases and ensuring Australia is an attractive country for new therapy launches in the global marketplace. Some of these features are only partially considered in the options paper, and Alexion asks that the final report to government strongly recommend their ongoing continuance as a central part of the HTA system. These features are identified in the table below.

Feature	Comments
Special Pricing Arrangements and confidential pricing	This is crucial to enable support from global businesses for cost-effective prices in an environment where non-confidential agreements could lead to global reference pricing and threaten innovative treatments coming to Australia. It is important to note that Australia is a lower-cost market for many pharmaceutical products. Decisions about our HTA processes cannot be taken in isolation from global factors in what is a multinational marketplace.
Confidential indication-based pricing	Indication based pricing ensures that HTA decisions reflect the unique characteristics of disease specific populations, clinical data and pricing. This also enables Australian affiliates to bring products for specific indications into Australia which may not be possible if the price were to be blended across different indications.

No fixed ICER thresholds	This is crucial for innovative rare disease therapies that will often not meet the thresholds of medicines for more common diseases because of small population sizes and life-long treatments, often commencing in paediatric populations. It is a positive feature of the current Australian system and sets us ahead of many other comparable jurisdictions.
LSDP criteria – (cost-effectiveness not met)	The Australian Life Savings Drug Program is intended to treat patients with ultra-rare and life threatening (modifying) diseases and should not be subject to the same evaluation for cost-effectiveness as other technologies listed on the PBS.

Abbreviations: HTA: Health Technology Assessment; ICER: Incremental cost-effectiveness ratio; LSDP: Life Savings Drug Program; PBS: Pharmaceutical Benefits Scheme

Key changes for equitable, timely and sustainable access to innovative rare disease therapies for Australian patients.

Based on our extensive experience in the Australian market and with the existing HTA system, there are four areas of reform which would make a significant difference to equitable and timely access for Australian patients with rare diseases. These areas are summarised in the table below.

Feature	Comments
Discount rate	The options paper does not recommend a clear pathway for change to the current application of discount rates. While acknowledging the impact of the discount rate in areas like vaccines it fails to recognise the impact of discount rates for rare disease therapies, which are considerable. Alexion recommends a model which would incorporate different discounting methodologies in the base case of the economic evaluation, namely the application of differential discount rates (i.e. 5% on cost and 1.5% on benefits) for lifelong technologies (with paediatric onset) with weight-based dosing (see 3.3 Economic evaluation – valuing of long-term benefits for more detailed comments).
Comparator selection	The options paper presents no definitive options for reform despite comparator selection being a crucial issue for rare disease therapy access in Australia. An appropriate approach to comparator selection would be to reinstate the intent of the original PBAC guidelines in policy. This would mean that in most instances the comparator would be the medicine most likely to be replaced in clinical practice, which would be consistent with the original interpretation of the National Health Act (pre-2016) and the practice of other international HTA organisations (see 3.3 Economic evaluation – Selection of the comparator for more detailed comments). For cost-minimisation submissions, the comparator should be what is most likely to be replaced rather than the lowest cost-comparator which in some instances may no longer be the treatment of choice. In disease areas that do not have any targeted innovative treatments and where the use of off-patent therapies have been utilised, the ideal comparator selected for cost-effectiveness should be SoC (excluding the off-patented therapies) and in instances where a RCT has been conducted in this disease area, it should be acceptable to evaluate cost-effectiveness against the comparator in these trials. It should not be considered reasonable to assess cost-effectiveness against alternate therapies which are often commoditised following a patent expiry and have a very lost cost (making achieving cost-effectiveness more difficult) with a poor to weak evidence base for use in the selected indication.
Broader value	Alexion recommends a commitment to a staged process to incorporate broader values, commencing with value to carers and the impact of decisions on other areas of government expenditure (eg NDIS). There should be an explicit commitment to develop workable methodologies to consider second order effects into the HTA value assessment. This includes amending the PBAC guidelines to include the caregiver benefit in the base case and the economic evaluation should consider the relevant downstream costs as a result of funding the intervention (i.e. NHRA. NDIS, carer supplement, aged care admissions, disability support).
LSDP reform	A single pathway for the LSDP is positive. However, this could be achieved by the proposed initial gateway triaging process. The introduction of PBAC consideration of cost effectiveness undermines the intent of the LSDP and the rationale for the establishment of the program. A key reform Alexion recommends is the broadening of the LSDP criteria to include severe morbidity (ie not just mortality).

Abbreviations: HTA: Health Technology Assessment; LSDP: Life Savings Drug Program; NDIS: National Disability Insurance Scheme; NHA: National Healthcare Agreement; NHRA: National Health Reform Agreement; PBAC: Pharmaceutical Benefits Advisory Committee; RCT: Randomised Controlled Trials

Key HTA changes warranting further consideration.

Alexion recommends the HTA review reference committee recommend reforms in the following areas that have not been specifically canvassed in the options paper.

Feature	Comments
Fee waivers for orphan drug resubmissions	While the intent of recommendations in the HTA options paper is to provide streamlined pathways which reduce the need for resubmissions, the possibility remains that resubmissions may be required. With a staged approach, the full benefits of streamlined assessment may not be realised for several years. Orphan drug submissions to the PBAC currently receive a fee waiver for first submissions. However, only 13 per cent of orphan drug submissions were recommended on their first submission (based on IQVIA analysis of January 2018 to June 2023 period). This considerably diminishes the benefit of fee waivers. Such waivers are in place to ensure innovative medicines for rare diseases do not face cost barriers which may deter sponsors from lodging submissions in Australia. This principle and the fee waiver should extend to resubmissions.
Pre-PBAC facilitated meetings with decision maker in addition to the existing Department stakeholders. -Meetings should result in more binding outcomes	The options paper canvasses options which would allow earlier engagement with various bodies involved in the HTA process – for example, upfront triaging or early discussions and consideration by the economic subcommittee of PBAC. However, what is missing is an option that allows facilitated workshops with decision-makers (crucially, representatives of PBAC) before formal PBAC consideration. This is currently possible in some circumstances following a negative PBAC recommendation (and at PBAC's initiation) and Alexion's experience with these has been positive. However, they occur towards the end of the HTA assessment process. Alongside other options, upfront facilitated workshops before PBAC consideration would greatly assist in reducing the risks of negative recommendations and resubmissions. Such an option should be able to be requested by a sponsor to help streamline and ensure there is a current need for the entry of their technology into the Australian market
Impact of cost recovery.	Several of the options envisage processes which could result in increased cost-recovery fees for sponsors. An expansion of cost-recovery would have negative impacts on attracting submissions, particularly for companies specialising in rare disease therapies. This would undermine the goal of delivering more timely access to innovative therapies for Australian patients. The comment in the options paper that sponsors would carry the cost for collection of outcome data under cost recovery is concerning as these databases can cost millions of dollars to establish and maintain.

Abbreviations: HTA: Health Technology Assessment, PBAC: Pharmaceutical Benefits Advisory Committee

Detailed response to HTA options paper - key issues for rare diseases.

1.1 Transparency & communication of HTA pathways, processes and decisions

Alexion supports the publishing of plain language summaries of the patient/population, intervention, comparison, outcomes (PICO) at the time that the PBAC agenda is released. All elements relating to net pricing and risk share arrangements must, however, remain commercial-in-confidence.

1.2 Consumer, clinician and other stakeholder engagement and consideration in HTA

Alexion supports this proposal in principle, however the role of stakeholders in relation to the PICO needs to be clarified. Consideration also needs to be given on the role of stakeholders in clinical trials that might not be conducted in Australia. This is particularly important in rare diseases where it may not be feasible to conduct trials in Australia due to low patient numbers.

1.4 State and territory collaboration

In principle, Alexion supports the development of a central standardised data sharing system for utilisation and outcome data. However, the proposed implementation plans should be adopted for all high-cost specialised therapies delivered in an inpatient setting (not just CAR-T therapies) to ensure a fair and equitable technology funding system.

- The next National Health Reform Agreement (NHRA) should consider funding arrangements with an increase in federal contribution if new inpatient therapies are replacing those that would have previously been delivered through the PBS.
- Consideration should be given to the benefits of a medicine/technology on the total health spend, not just the impact on the PBS.
- Data collection and sharing costs must be borne by government.

2.1 Streamlining and aligning HTA pathways and advisory committees- Pathway for drugs for ultra-rare diseases (Life Saving Drugs Program (LSDP)

Alexion supports a single and faster pathway to achieve LSDP listings (i.e. we support removal of the current requirement for a PBAC rejection prior to applying for an LSDP listing). This could be achieved by an initial gateway triaging process following a request from the sponsor, as applicants will understand if a submission is unlikely to meet the cost-effectiveness requirements of a standard HTA assessment.

Crucially, the existing LSDP criteria should be broadened to include severe morbidity as well as mortality, to recognise that many ultra-rare diseases may not be life threatening but can profoundly affect quality of life. Therefore, criterion A3 and A4 of the LSDP guidelines should be updated to also include therapies for diseases with significant morbidity. Alexion proposes that, medicines should qualify for LSDP eligibility if they:

- Are proven to create a significant life extension; OR
- Significantly improve morbidity, or significantly reduce levels of disability

LSDP submissions are intrinsically for therapies where cost effectiveness will not meet normal PBAC requirements. The options paper implies cost effectiveness would be considered by the PBAC for LSDP submissions. PBAC consideration of cost effectiveness as part of its decision-making process is not supported as it undermines the very rationale of the LSDP. The PBAC should provide advice on the clinical evidence presented by the LSDP expert panel and follow the LSDP listing criteria in making their assessment.

The decision for determining a LSDP listing should remain with the Minister for Health following consultation with the Chief Medical Officer. The LSDP should also continue to be funded through a separate funding stream to the PBS.

2.2 Proportionate appraisal pathways: Development of pathways to calibrate the level of appraisal required for HTA submissions to the level of risk (levels of uncertainty and potential fiscal impact) and clinical need that the submission represents.

In principle, Alexion supports the development of streamlined and efficient pathways to achieve PBS listing sooner but does not support the development of disease specific common models and strongly opposes any streamlined cost-minimisation pathway that requires a discounted price to achieve a listing.

Streamlined pathway for cost-minimisation submissions:

- Alexion is, in principle, supportive of the earlier sharing of relevant comparator pricing to enable sponsors to make a decision as to whether it is feasible to pursue a listing. It is critical that sharing of this pricing information must follow existing strict confidentiality deeds with penalties applying for any breaches (to avoid price phishing).
- There should also be strict guidelines in place on the timing of when the price is made available i.e. it should only occur when a sponsor has lodged their TGA submission or at the time when a notice of intent for a HTA submission is made.
- Alexion recommends the proposed arrangements for earlier access to price sharing be trialed for a two-year period to ensure there are no unintended consequence from the implementation of this change (i.e. does not lead to companies gaming the system).
- Alexion strongly opposes any streamlined cost-minimisation pathway that leads to the inclusion of the requirement of a discounted price to achieve listing, this is further explained in “4.1 Approaches to funding or purchasing new health technologies”

Early resolution mechanism:

- The requirement for submissions to be lodged within six months of first international registration is unrealistic and would severely curtail the effectiveness of the proposed scheme and the ability of sponsors to participate. Any requirement to create a nexus between international registration and Australian listing timeframes should be removed to ensure sponsors can more confidently enter the HTA appraisal process ensuring all requirements of a successful submission can be made. Arbitrary time requirements such as that proposed could mean sponsors may not be able to lodge a submission in Australia using a new pathway given international considerations.
- Of the options proposed under this pathway, Alexion supports Option 4 (optional resolution step after PBAC consideration but before advice is finalised) with the addition of allowing a sponsor to request a facilitated workshop with representatives of PBAC before its consideration of a submission.
- We do not support restricting the capacity for a sponsor to resubmit proposals to the HTA committee – this would potentially and arbitrarily limit access to new therapies for Australian patients.

Development of a disease specific common model:

Alexion does not believe the development of a disease specific common model will make the HTA evaluation process more streamlined. The development of disease models are not appropriate for the evaluation of technologies for rare diseases.

Case manager:

Alexion supports the resourcing of a case manager, however this case manager must be appropriately trained and empowered to facilitate decisions and not be assigned solely as an administrative support.

It would significantly streamline processes during pricing negotiations following a PBAC recommendation if sponsors had an opportunity to meet or directly discuss issues with the PBS pricing team more frequently. This would help resolve issues more efficiently and hasten the time between application and listing.

3.1 Determination of the Population, intervention, comparator and outcome (PICO)- increased early stakeholder input and transparency

Alexion supports early stakeholder input and increasing the transparency of the process for stakeholders. Earlier determination and agreement on the PICO for use in submissions will make the HTA process more efficient. This should be a binding agreement. However, processes such as that used by the Medical Services Advisory Committee (MSAC) should be avoided as they create a year-long process that would further delay HTA decisions.

3.2 Clinical evaluation methods – Methods for the assessment of nonrandomized and observational evidence

Non-randomised studies:

Alexion supports greater flexibility in the assessment of nonrandomised and observational evidence to support the clinical and safety claims proposed in a HTA submission.

However, the PBAC guidelines need to accommodate and maintain flexibility in assessing clinical data in the context they are presented (i.e. literature based submissions). In instances where an indirect treatment comparison (ITC) is required, the sponsor should have the ability to present any ITC methodology (I.e. Bucher, Match adjusted or stimulated treatment comparison) as long as the approach is justified.

The PBAC guidelines need to permit flexibility especially when assessing technologies that target rare and ultra-rare diseases as its often impossible to design a “perfect” trial due to ethical and sampling considerations. This flexibility also needs to flow into the economic evaluation.

As an example, in March 2022, the PBAC recommended the listing of trientine on the PBS for the treatment of Wilson's disease despite limitations in the clinical studies available. The PBAC considered in the specific context of the therapy and "rare disease, the lack of randomised trials for trientine was understandable." This flexibility should be maintained in the clinical appraisal of HTA evaluations and also needs to be carried into the economic evaluation. For HTA submissions with a clinical claim of superiority, the low tolerance for uncertainty associated with clinical evidence and its subsequent conservative application in modelled economic evaluations impacts PBAC decision-making, often resulting in resubmissions and delayed access for patients.

Real World Evidence/Data:

Alexion is supportive of including Real World Data ("RWD") and Real World Evidence ("RWE") in the HTA appraisal process and supports the development of multi-stakeholder advisory committees to oversee the development of enabling systems and pathways to support the use of RWD. The inclusion of RWE in the critical appraisal is helpful in rare diseases where there are often limitations in trial designs. In these circumstances, RWE/RWD are appropriate sources to help reduce uncertainty in the clinical claims presented in HTA submissions. This is further described under Section "4.3 Understanding the performance of health technologies in practice."

3.2 Clinical evaluation methods – Develop an explicit qualitative value framework

Alexion supports the development of a broader value framework so long as flexibility in the appraisal process remains. Broader value should flow into the base case of the economic models, the care giver benefits and where the therapeutic benefit improves functional benefit/morbidity. Explicit commitment should be given to develop and include second order benefits in the value assessment where appropriate.

Alexion proposes the following immediate/short- and medium-term outcomes:

Immediate /short-term:

- Development of agreed criteria for situations where indirect benefits (second order effects) for patients and their caregivers should be included in the HTA assessment process.
- Broadening the current HTA perspective, which is currently limited to direct health sector/patient impacts. This should consider the costs and benefits affecting the patient and their carers/dependent including consideration of the caregiver burden for life long chronic rare diseases, particularly in paediatric diseases as the burden is often more profound in these groups of patients. This needs to occur as part of the base case economic analysis, not as a sensitivity analysis as is currently permitted.

Outcomes: Failure to consider carer effects means the economic evaluation is incomplete and may provide misleading information on the impact of a therapy on societal health or wellbeing. Where the condition has a substantial effect on the quality of life of parents and carers there should be an option to include this benefit in the base case economic evaluation. In these cases, a carer-based utility decrement should be considered acceptable as part of the base case. This would bring Australia in line with other HTA Agencies (e.g. NICE, CADTH and the Netherlands)

Medium term

Costs: Evaluations should include relevant costs incurred by the health, ageing or welfare budgets (federal and state). For example, all Commonwealth costs related to treating a disease/condition - direct (non-medical) and indirect (i.e. NHRA, NDIS, carer supplement, aged care admissions, disability support) should be assessed. This would reflect the fact that the listing of a medicine might demonstrably reduce a patient's use and reliance on other government funded programs like the NDIS.

Currently, the Section 4 budget impact estimates are limited in scope to direct federal health costs (PBS/MBS). This does not capture the true cost to government or consider the savings generated to other state and federal government portfolios. Guidelines for the development of methodology to calculate the true net cost of a new PBS listing should be developed in consultation with stakeholders.

Importantly the onus would be on the sponsor to collect appropriate evidence to support inclusion of these additional costs and benefits. This will require additional work to identify data sources and the appropriate way to extract and analyse these data sets.

3.3 Economic evaluation – Selection of the comparator

Alexion recommends the HTA review committee strengthen options for the selection of comparators for superior and non-inferior health technologies. This is a critical issue for ensuring timely access to innovative rare disease therapies.

Superior health technologies

The appropriate approach for comparator selection for superior therapies would be to reinstate the intent of the original PBAC guidelines. This would mean the comparator would be the medicine most likely to be replaced in clinical practice.This is consistent with the original interpretation of the National Health Act (pre-2016) and the practice of other international HTA organisations.

It is inherently difficult for pharmaceutical sponsors to introduce innovate, tested and safe therapies into Australia if cost-effectiveness needs to be assessed against alternate therapies that have been commoditised following patent expiry and are often very low cost.

Non-inferior health technologies

Alexion recommends the PBAC guidelines be updated to reflect the selection of the most utilised comparator for the purpose of a cost-minimisation analysis where multiple products are available. It is disadvantageous and limits pharmaceutical sponsors from entering the Australian market with innovative therapies if they are expected to be cost-minimised to the lowest cost comparator (often the one that is near the end of its lifecycle and has been subject to multiple price reductions).

If a new medicine is non-inferior to multiple comparators that it could replace, and a cost-minimisation approach is appropriate, the cost-minimised price of the new medicine should be the average price of the other alternative medicines weighted by market share, rather than the price of the lowest priced alternative. The weighted comparator pricing approach is a fair and balanced solution that achieves a comparator price which more accurately represents the average cost to the PBS of current treatment.

3.3 Economic evaluation – valuing of long-term benefits

With the Australian therapeutic landscape expected to increasingly see innovative cell and gene therapies and personalised medicines offering new treatment possibilities, the current uniform discount rate of 5% risks significantly reducing patient access to cutting edge therapies.

Below is an example on the impact on the ICER when different discounting scenarios are applied to a chronic disease where therapy initiates in infancy; whilst benefits are maintained lifelong.

Table 1 Impact of ICER applying different discount rates

Scenario	Cost discount	Health discount	Result on ICER*
1 (Base case)	5%	5%	
2 (lower rates)	1.5%	1.5%	17% increase in ICER
3 (differential rates)	5%	1.5%	57% decrease in ICER

Evaluating new therapies with a high discount rate can lead to the creation of a generational effect where particular medicines are perpetually disadvantaged, locking in decision making that undervalues the long-term benefits.

Alexion acknowledges that the PBAC Guidelines (v.5; 2016 – section 3A.1.5) allow for submissions to use other discounting methodologies such as a different uniform rate, differential rates and time-varying rates presented in a scenario analyses. However, Alexion recommends that the PBAC guidelines be amended to include the application of differential and time varying rates in the base case of submissions that meet the following circumstances:

(i) vaccines;
(ii) high-cost life-long medicines with weight-based dosing; and
(iii) other therapies (such as cell and gene therapies)

4.1 Approaches to funding or purchasing new health technologies – recognising competition between new health technologies that deliver similar outcomes

Competition between new technologies that deliver similar outcomes:

Alexion strongly opposes the scenarios presented in the options paper which would require cost-minimisation submissions to offer a lower price to be successfully listed on the PBS. This approach will lead to fewer technologies being brought to Australia, limiting clinician and patient choice for treatments.

There is often added value of new health technologies (e.g. dosing convenience, reduced pill burden) leading to better compliance and patient-related outcomes that are not usually considered when conducting a cost-minimisation analysis.

For example, in July 2021, the PBAC recommended once daily orally-administered opicapone for the treatment of Parkinson's disease on a cost-minimisation basis when compared to multiple dosed (up to 5 times daily) entacapone which was already listed on the PBS. The cost-minimisation analysis was conducted on the mean doses of each technology based on the key clinical trial data. The listing of opicapone on the PBS was preferred by Parkinson's Australia as it noted the convenience and adherence benefits of a once daily treatment given the nature of Parkinson's disease and its impact on speech and swallowing capacity. If the options paper scenarios had been in place, a lower price would have been required to facilitate a listing of opicapone and the sponsor could have been less inclined to bring the technology to Australia. This would have meant patients did not have access to a more conveniently dosed medication.

Technologies that deliver superior outcomes - unintended consequences for innovative treatments:

The options paper scenarios would also lead to unintended negative consequences on the cost effectiveness submission pathway for new technologies that deliver superior outcomes to existing technologies. It is challenging to be cost-effective when compared to a lower cost comparator despite demonstrating superior outcomes and the proposed options would either deter or delay sponsors from bringing innovative therapies into Australia. This will limit clinician and patient choice of treatments that demonstrate superior outcomes.

Disinvestment:

An explicit disinvestment framework is unnecessary and not supported. Delisting from the PBS should continue to be a disallowable instrument so that parliamentary scrutiny and transparency remains a central component of delisting decisions.

Bridging funding:
Alexion in principle supports the options for bridging funding, however considers:

- A six-month requirement from first international registration is unworkable and would severely restrict the effectiveness of the scheme and should be removed.
- Sponsors should be able to apply outside the constraints of a priority list where horizon scanning has not identified a particular critical and innovative therapy that would benefit Australian patients.
- Any bridging scheme needs to recognise the intrinsic difficulties of data collection for rare diseases with small patient cohorts and long periods of treatment. This is quite different to the capacity for data collection in other health fields like oncology.
- Greater clarity needs to be provided in relation to risk-sharing to ensure that the bridging fund avoids the disincentives with existing managed access programs (both in Australia and other jurisdictions).

4.3 Understanding the performance of health technologies in practice. Oversight – reforms to optimise access to and use of RWD in HTA:

Establish a multi-stakeholder advisory group, reporting to government, to co-design and oversee the development and implementation of enabling systems, pathways, evaluation, and research to optimise access and use of RWD in HTA.
Alexion supports the establishment of multi-stakeholder advisory group. Consideration needs to be given to establishing programs that allow access to data more efficiently. For this pathway to succeed data sharing between states and the federal system or between international registries, sponsor and the government needs to significantly improve to achieve best practice.

Develop a strategic approach to increase confidence, awareness, and acceptance of cross-jurisdictional and cross-sectoral RWD access and use of RWE
Alexion is supportive of this option and recommends its development by the committee overseeing the development and implementation of systems to optimise access to RWD/RWE.

Develop whole of government data infrastructure to accelerate use of RWE.
Alexion is supportive of a national approach but for it to be successful significant investment will be necessary to establish a national database where data can be collected and shared between federal and state health departments. For instance, currently Victoria is the only state that has data shared between the immunisation registry and GPs general health data sets.

Collection of utilisation and outcome data for provisionally listed health technologies
In principle, Alexion supports the creation of registries and databases to collect outcome data for provisionally listed therapies. However, the options paper suggests that sponsors would carry the cost of this activity. This would be an unreasonable cost burden for sponsors noting that such data collection could cost several million dollars to establish and maintain.

Different registries collect different data and often it is difficult to have one registry encompassing all data sets (i.e. there is no one “best” registry). Therefore, an approach where a specific PBS registry is created may be the most efficient for this intended purpose.

The options paper notes that “in the case of ultra-rare diseases, international registries should be utilised” which is sensible however it is unclear how Australian health authorities would determine which international registries would be accredited for these purposes. Non-governmental databases have usually been set up by academics and may not collect data in a form useful to PBAC deliberations. Government-operated registries would require an accreditation process which may face similar difficulties if the operating parameters of those registries varies significantly between jurisdictions.

Therefore, implementing these options will require some pragmatism and may require allowing company databases, allowing more than one international database, understanding the limitations around likely data collection (i.e. needs to be clinically relevant for physicians, not just for HTA assessment) to be utilised.

Alexion recommends the development of databases which collects data from clinicians in a similar manner to the existing approach where Services Australia records and the approval for patients meeting the continuation criteria for existing therapies.

A pilot program should be trialed with stakeholder agreement on what data should be collected and how this data should be interpreted, this should include the involvement of the PBAC, clinicians and pharmaceutical sponsors at the time of establishment and review.

5.1 Addressing areas of unmet clinical need and gaps in the PBS – a systematic approach encompassing five interdependent new mechanisms:

Alexion supports this approach in principle. However, a priority list should avoid any movement towards a Pharmac-style system which has severely limited access to therapies for New Zealanders. An IQVIA study (September 2023) found that there are 131 modern medicines available through public funding for Australian patients that are not available to New Zealand patients.

5.3 Environmental impacts in the HTA:

Further stakeholder consultation will be required to implement new environmental standards in the HTA system. Any scheme should be consistent and developed in parallel with international practice in large similar markets to avoid additional red tape and costs for industry.

5.6 Strengthen international partnerships and work-sharing:

Alexion opposes this in principle. If Australia were to join a buying group with other markets, it is expected that manufacturers would need to waive rights to confidential pricing among the payers within the buying group to generate a common price. This would have detrimental international reference pricing implications that would be unviable for manufacturers. It would ultimately result in new health technologies simply not coming to Australia