Medicines Australia | Better health through research and innovation

25 January 2024

Budget Policy Division
Treasury
Langton Cres
Parkes ACT 2600

Via email: PreBudgetSubmissions@treasury.gov.au

Dear Budget Policy Division,

Re: 2024-25 Pre-Budget Submissions

Thank you for the opportunity to put forward a 2024-25 Pre-Budget Submission that advocates for reform to Australia's Pharmaceutical Benefits Scheme (PBS) in the interests of access to life-saving and life-changing medicines for all Australians.

Medicines Australia and its members are seeking to continue our collaborative and effective partnership with the Australian Government to ensure Australia's PBS continues to meet the needs of Australian patients. The PBS is an essential part of our health system which is instrumental in achieving the vision of the *National Medicines Policy* (NMP) to achieve the world's best health, social and economic outcomes for all Australians. Medicines Australia commends the Government for establishing the Health Technology Assessment Policy and Methods Review (HTA Review) and its drive for reform through this once in a generation opportunity.

Australia's HTA system was first established in 1993, and much has changed over the past three decades: patient and public expectations of our health system have risen, and medicines technology has moved beyond traditional compounds to monoclonal antibodies, cell and gene therapies and advanced vaccines technologies. These factors are all challenging the system – it can now take up to 466 days for a medicine to be listed on the PBS[1]. Australia needs to do better in providing timely and equitable access to the latest medicines.

Medicines Australia expects the HTA Review Options Paper, to be published this week for consultation, will affirm the need for fundamental reform of how new medicines and vaccines are assessed for listing on the PBS. Significant reforms are essential for a modern PBS and are supported by all stakeholders.

Medicines Australia supports the HTA Review's call for bold PBS reform to deliver equitable and timely access for all Australians to new medicines and vaccines, while encouraging the global industry to bring the latest and best medicines to Australia to address unmet patient need.

With those aims in mind, Medicines Australia and its members have developed a three-year implementation roadmap for HTA reform (see appendix for more detail). We have taken a top-down approach to reform and are proposing a suite of policy proposals that will work synergistically to achieve an ambitious but achievable aim of ensuring Australians have access to innovative medicines within 60 days of TGA registration.

The innovative medicines industry has partnered with governments over decades to ensure the continued listing of new medicines, so that Australians have access to the very best treatments. Industry has delivered billions in budget savings through successive Strategic agreements that are forever embedded in the system. The savings agreed to were: 2010 ($1.9 billion), 2015 ($6.5 billion), 2017 ($1.8 billion) and 2022 $1.9 billion). These savings were in return for agreed policy initiatives including the current HTA Review. Furthermore, PBS expenditure has grown by $3 billion over 10 years in nominal terms and has

[1] Medicines Australia *Medicines Matter 2022* https://www.medicinesaustralia.com.au/wp-content/uploads/sites/65/2023/04/Medicines-Matter-2022-FINAL.pdf Accessed 23/01/2024

shrunk as a proportion of healthcare expenditure from 20% to 17%[2]. The population continues to grow and age, while the health system continues to come under pressure to meet the population's healthcare needs. It is time for fundamental reform of the PBS to ensure its future sustainability so that it can deliver the world's best health, social and economic outcomes for all Australians.

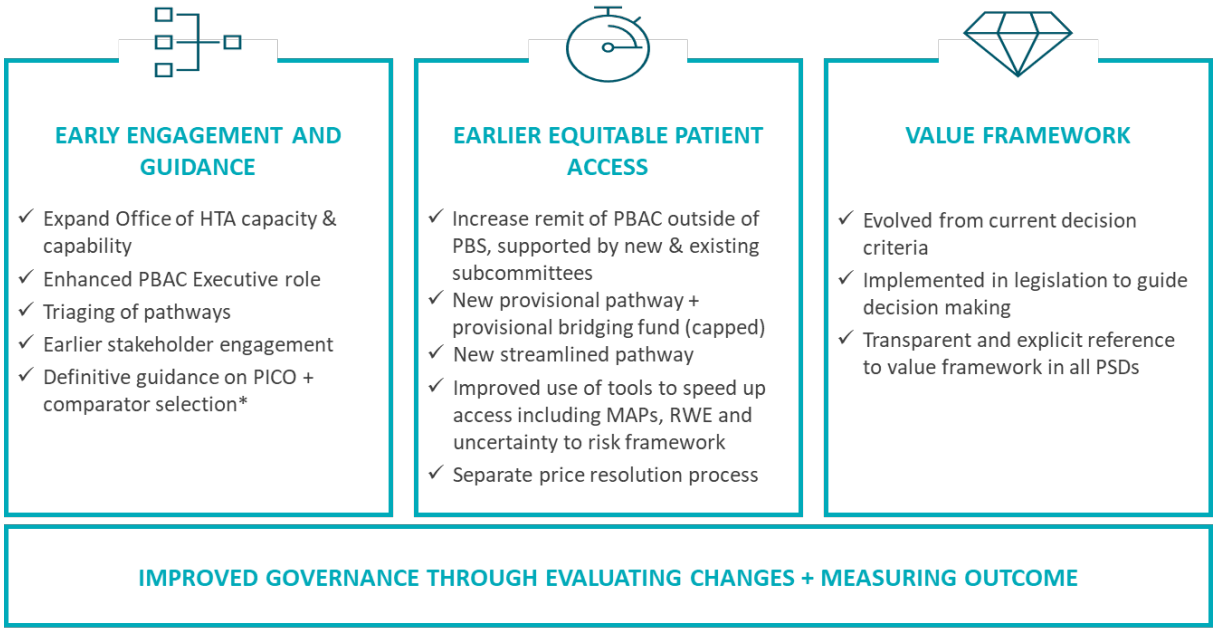
RECOMMENDATIONS

1. Implement a comprehensive suite of policy reforms to Australia's HTA system to ensure Australians have equitable and timely access to new medicines.
2. Disaggregate PBS spending in the budget papers so that investment in innovative medicines can be more easily tracked, and the impact of HTA reform can be measured.

These recommendations are discussed in more detail below.

1. A ROADMAP FOR HTA REFORM

Our reform proposals are summarised in the following diagram. Key features are explained in detail below.



*If requested by the sponsor (cost recovered)

Expanded Office of HTA

This reform proposal is focused on new responsibilities and improved capabilities within the Office of Health Technology Assessment. It is strongly aligned with the New Frontier report which includes several recommendations for greater resourcing, guidance, proactivity and capabilities within the Department.

New responsibilities for the Office of HTA could include:

- Horizon scanning
- Timely early planning and advice

[2] https://www.medicinesaustralia.com.au/wp-content/uploads/sites/65/2023/06/Funding-Innovative-Medicines-1.pdf

- Proactive patient and stakeholder engagement
- Preliminary PICO guidance (if requested by the Sponsor)
- Triage for new provisional and streamlined pathways
- Monitoring system performance

New capabilities could include:

- Permanent active role within Office of HTA for Chair of PBAC + Chairs of Sub Committees to facilitate timely and reliable advice
- Dedicated industry liaison roles to facilitate timely responses to enquiries
- Better coordination with industry through joint working groups

Earlier Stakeholder Engagement

Earlier stakeholder engagement is crucial to ensure that there is early alignment on key parameters and perspectives. This is particularly important for patients and carers but is also key for clinicians and sponsors. Early engagement is strongly aligned with recommendations in the New Frontier Report, and will also be supported by work on an enhanced consumer engagement process that is currently underway as part of Medicines Australia's Strategic Agreement with the Common wealth.

Increased Remit of the PBAC

We propose broadening the PBAC's responsibilities to assess all medical technologies uniformly, adhering to consistent timelines. To aid this process, the committee could be bolstered by specialised new or existing sub-committees focusing on areas such as rare diseases, vaccines, cell and gene therapies as well as genomics. This would mean, for example, that the PBAC could recommend drugs directly to the LSDP. We anticipate that this approach would bring about a greater level of consistency and transparency in our system. MSAC would retain its current responsibilities with the exception of co-dependent therapies, which would transition to a genomics sub-committee.

Provisional Pathway and Bridging Fund

We propose the establishment of a provisional fund for select new medicines so that patients can access them without delay. The provisional pathway would address two main issues which can slow down access to new medicines: (i) data issues (e.g. where Phase II clinical trial data has been accepted and Phase III data is still required) (ii) administrative issues (e.g. where a new therapy would be delayed because there are complex funding negotiations with the commonwealth or states and territories). The provisional pathway would only be available to products with TGA approval, and would be reserved for medical technologies with high added therapeutic value where there is an unmet clinical need. After a full health technology assessment by the PBAC they would transition to the PBS.

We envisage the provisional fund would be technology agnostic to allow for the funding of medicines, vaccines, blood products as well as cell and gene therapies. Further we would expect the fund may cover critical elements associated with delivering the technology which are not currently funded in current practice. This may include tests and other service elements critical to the delivery of the technology.

Streamlined Pathway

There is a category of PBAC submissions which are low risk because the medicines are second or third to market and are presented as non-inferior (in terms of safety and efficacy) to medicines which are already on the PBS. These medicines do not require full PBAC evaluation and efficiencies could be gained by introducing a more streamlined pathway. This enable PBAC resources to be directed to more complex submissions, and would also result in faster listings.

Price Resolution

We propose a separate process to resolve disputes around disagreements in relation to the final price or deed of agreement, after the PBAC process. This would help prevent lengthy delays resulting from "resubmission churn", where price is effectively negotiated through successive PBAC submissions. The process would be headed by an experienced dispute resolution expert, independent of the Department, PBAC and sponsor.

Value Framework

The adoption of a value framework for the public funding of medicines would offer distinct advantages for more transparent and predictable reimbursement decisions, while simultaneously fostering innovation and broadening stakeholder participation. Such a framework, co-designed by stakeholders, would:

- move beyond the current legislative focus on cost-effectiveness, allowing a more comprehensive consideration of value in decision-making processes;
- be enhanced by the inclusion of a diverse range of stakeholders, contributing varied perspectives and expertise, thereby enhancing the inclusivity of the HTA process;
- lead to a clear, transparent, and consistent decision-making process, enhancing credibility in decision making among key stakeholders;
- specifically address equity and access issues, ensuring that technology adoption decisions are fair and do not unduly favour or disadvantage any group, in line with Australia's commitment to equitable healthcare;
- provide the industry with definitive criteria for product assessment, enabling companies to more strategically allocate resources to areas that are likely to offer better returns on investment.

Implementation Timetable

The follow table shows how the proposed reforms can be implemented over a three-year period.

Year	Approach	Implementation of Reforms
Year 1 – Early Wins for Access 2024-25 Federal Budget	• Lower- or no-cost options (e.g. co-design work) • Options which benefit patients	• Empowered Office of HTA – expanded responsibilities and capabilities • Early patient engagement – co-design and implement • Streamlined pathway – co-design and implement • Value Framework – co-design • Price Resolution – establish new body • KPIs – agree on measures for success (timely access, investment etc.) • Lower the discount rate
Year 2 – Invest & Consolidate 2025-26 Federal Budget	• Programs which can be implemented with minimal investment (e.g. pilot programs)	• Provisional Pathway – co-design and legislate for provisional fund • Enhanced remit of PBAC – legislate and enact • Modernised use of data – update MAP guidance and implement RWE framework • Value Framework – pilot
Year 3 – Full Implementation 2026-27 Federal Budget	• Significant Reforms (e.g. full implementation of programs)	• Value Framework – full implementation • KPIs – evaluation of reforms and first public report on their success

2. IMPLEMENT ENHANCED REPORTING OF INVESTMENT IN F1 MEDICINES

Spending on the PBS is currently reported as an aggregate figure that includes the amount paid for innovative medicines (Formulary 1) and older, genericised (commoditised) medicines (Formulary 2); plus rebates which are subsequently repaid to the government by pharmaceutical companies which have risk sharing agreements; plus supply chain expenditure including pharmacy dispensing fees and the Community Service Obligation for wholesalers. Hence, it is not possible to tell from the total PBS spend how much has been invested in innovative medicines, where value for money has been proven. PBS spending should therefore be disaggregated in the budget into its constituent parts.

The Strategic Agreement between Medicines Australia and the Commonwealth includes a commitment to implement disaggregated reporting on the PBS spend. Delivery on this commitment would increase transparency and enable the tracking of government investment in innovative medicines.

NATIONAL WELLBEING FRAMEWORK

Our recommendations will support three of the wellbeing themes and multiple indicators from the Measuring What Matters framework:

- **Healthy**: timely access to safe and cost-effective innovative medicines, biotherapeutics and vaccines is vital for Australians to continue to seeing improvements in life expectancy and quality of life. Improved access to innovative therapeutics will also support greater gains in mental health and prevention and treatment of chronic conditions. Investing in innovative medicines reduces premature mortality and also saves hospitals money by reducing the length of hospital stays[3].
- **Sustainable**: an effective and efficient HTA system would help signal to international and local suppliers of innovative therapeutics that Australia is an available market, therefore supporting improvements in Australia's economic resilience ranking. Fiscal sustainability is also key feature of the PBS as explained above.
- **Prosperous:** as demonstrated during the COVID-19 pandemic, investment in medicines is a key driver for the economy and productivity. COVID-19 vaccines, for example, are estimated to have reduced the impact of the pandemic on the economy to an estimated $214 billion, resulting in a positive incremental benefit of $181 billion[4]. Additionally, a well functioning HTA system will promote downstream benefits of local innovation in the entire life sciences industry and supply chain.[5]

Medicines Australia looks forward to continuing to work collaboratively and transparently with the Government on a range of issues relating to the health of Australians and the industry, including the implementation of the Strategic Agreement, the funding of the TGA, the Clinical Trials National One Stop Shop, tax reform, climate change, government procurement, and a high-level government-industry forum.

Yours sincerely,

Elizabeth de Somer
CEO
Medicines Australia

ABOUT MEDICINES AUSTRALIA

Medicines Australia leads the research-based medicines industry of Australia. Our members discover, develop and manufacture prescription medicines, biotherapeutic products and vaccines that bring health, social and economic benefits to Australia.

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[3] F. Lichtenberg, *The Effect of New Medicines Provided by the PBS on Mortality and Hospital Utilisation in Australia, 2002-2-19*, 2023 https://www.medicinesaustralia.com.au/wp-content/uploads/sites/65/2023/06/From-Hospital-Beds-to-Healthy-Lives-The-Impact-of-Innovative-Medicines.pdf Accessed 23/01/2024

[4] N. Fox, P. Adams, D. Grainger, J. Herz, C. Austin. *The Value of Vaccines: A Tale of Two Parts, 2022* https://www.mdpi.com/2076-393X/10/12/2057 Access 22/1/2024

[5] Commonwealth Government. *Measuring What Matter July 2023.* https://treasury.gov.au/publication/p2023-mwm. *Accessed 22/1/2024*

APPENDIX – Roadmap for HTA Reform

The Vision for HTA Reform

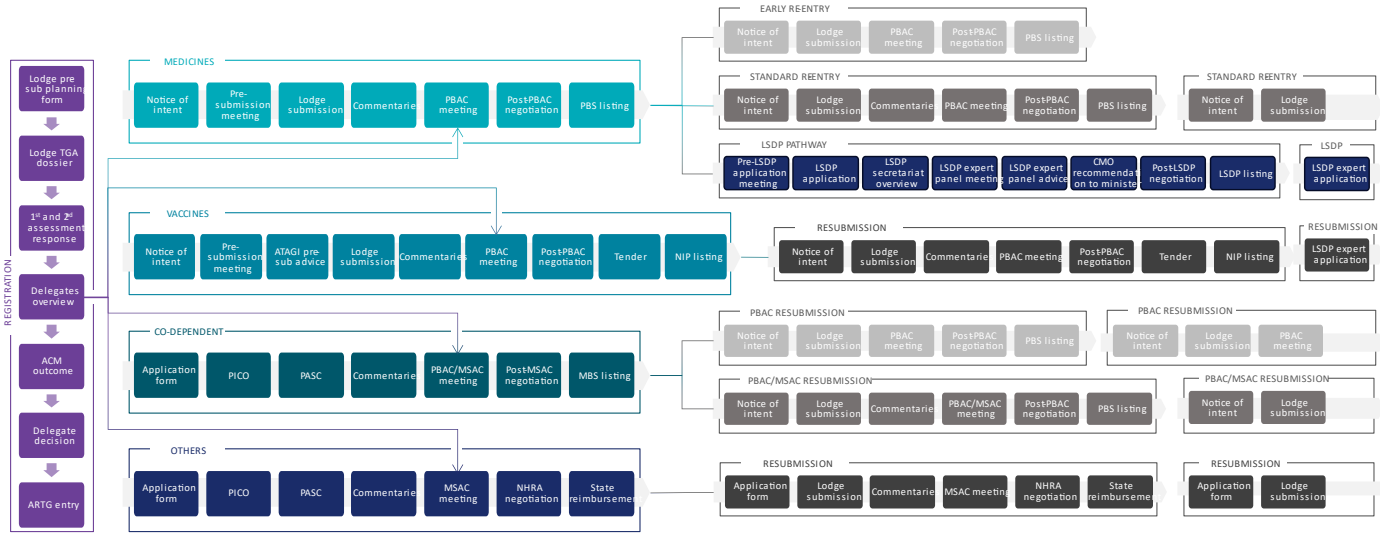
The reimbursement system should be reformed with a central vision in mind

Australians have access to the latest medical technologies within 60 days of TGA registration.

Key Terminology

WHAT WE USE	WHAT WE MEAN	OTHER SIMILAR TERMS
Provisional listing	Rapid listing based on provisional pricing before full HTA	Accelerated access Conditional listing Interim funding
Managed access program (MAP)	A range of structured agreements to manage financial and clinical uncertainty Used as tool within existing pathways	Managed access/entry Coverage with evidence development Risk-share Volume or expenditure caps Price-volume Pay-for-performance
Value framework	Transparent framework to define value, enhance equity and guide decision making	Second order effects Societal value/perspective Cost-effectiveness Cost-benefit
Work sharing	Collaboration with other international HTA bodies	
Exchange of information	A process for informal interactions between industry, evaluators + decision makers	
Real world data/evidence (RWD or RWE)	RWD refers to information collected outside of traditional clinical trials, often from sources like electronic health records, patient registries, and health apps. RWE is the clinical insight derived from analyzing this RWD, providing a broader understanding of health outcomes, treatment benefits, and potential risks in real -world settings.	Observational data/evidence

Current reimbursement ecosystem



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Recommendations for HTA Reform

EARLY ENGAGEMENT AND GUIDANCE

- ✓ Expand Office of HTA capacity & capability
- ✓ Enhanced PBAC Executive role
- ✓ Triaging of pathways
- ✓ Earlier stakeholder engagement
- ✓ Definitive guidance on PICO + comparator selection*

EARLIER EQUITABLE PATIENT ACCESS

- ✓ Increase remit of PBAC outside of PBS, supported by new & existing subcommittees
- ✓ New provisional pathway + provisional bridging fund (capped)
- ✓ New streamlined pathway
- ✓ Improved use of tools to speed up access including MAPs, RWE and uncertainty to risk framework
- ✓ Separate price resolution process

VALUE FRAMEWORK

- ✓ Evolved from current decision criteria
- ✓ Implemented in legislation to guide decision making
- ✓ Transparent and explicit reference to value framework in all PSDs

IMPROVED GOVERNANCE THROUGH EVALUATING CHANGES + MEASURING OUTCOME

*If requested by the sponsor (cost recovered)

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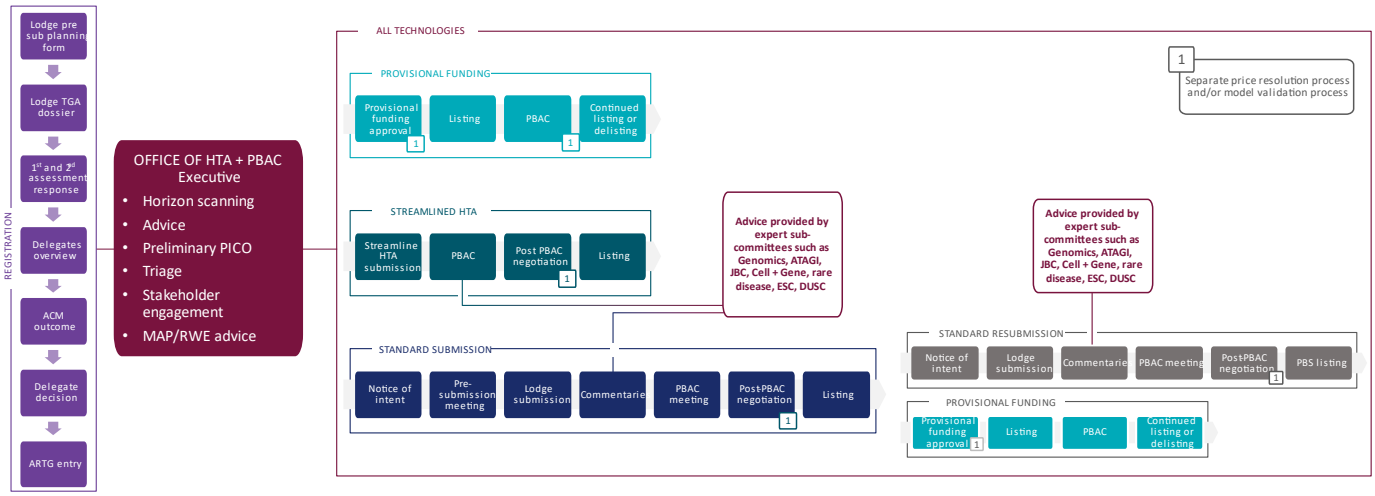
Future reimbursement ecosystem



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Empowered Office of HTA

An empowered Office of HTA will address issues earlier in the reimbursement timeline as recommended in the New Frontiers Report*

WHO

1.

Departmental staff (Office of HTA) + PBAC Executive + Experts from sub-committees

2.

Patients and other stakeholders

3.

Coordination with industry through joint working groups

WHAT

1.
Horizon scanning

2.
Timely early planning and advice + proactive patient engagement

3.
Preliminary PICO guidance**

4.
Triage for provisional & streamlined HTA

WHY

1.
Monitor system performance

2.
Early advice to prevent incorrect assumptions

3.
Early engagement with stakeholders including patients/clinicians

4.
Trigger for provisional & streamlined HTA

5.

Increase the likelihood of first-time approvals

*Several recommendations call for greater resourcing, guidance, proactivity and capabilities within the Department of Health
**Option for definitive advice including ability to adjudicate on comparator (i.e. change from lowest cost comparator)

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Provisional Access

New provisional pathway + bridging fund to enhance equitable patient access

BRIDGE TO ACCESS

New legislated bridging fund with capped budget

FLEXIBLE

Allows for provisional funding of any new medical technology meeting specific criteria*

EARLY

Funding occurs before price is agreed (see revised pathways – either before or during PBAC process)

SHORT TERM

Funding ceases following PBAC review & formal price negotiation allowing transition to traditional funding program (PBS etc)

*High clinical need; Substantial patient benefit; Limited treatment options etc.
Adjudicated by Office of HTA + PBAC Exec + expert subcommittee advice + advisory groups

Streamlined Pathway

Amended pathway capturing low risk and/or low budget impact submissions to be directly reviewed by PBAC

Triage by the Office of HTA
–
can be proposed by either Office of HTA or the Sponsor

Scope expanded to include cost-min or other low risk submissions*
–
if agreed by Sponsor

Reduced information requirements
–
similar to Category 3+ submissions

Review by PBAC only**
–
no evaluator or sub-committee commentary

*For example – rare disease products with minimal patient numbers or changes in product formulation
**PBAC can request sub-committee or advisory group advice

Value Framework

A new framework to underpin equitable reimbursement decisions, shifting conversation from cost to investment and ensuring value considerations are explicit and transparent in decision making process

Redefine cost-effectiveness within a broader value framework to include patient experience, equity, wellbeing, innovation, future potential and non-health outcomes

Consult with the community to align on the key elements that are important*

Include in legislation and policy, and link to methods. Enhanced reporting of value in PSD aligned with framework.

*Leverage new national wellbeing framework

Price Resolution

Separate process to resolve pricing disagreements

RESOLUTION

Separate resolution process for financial issues* with input from industry, Government and other stakeholders as appropriate

SPONSOR LED

Initiated by Sponsors at multiple entry points (see Future reimbursement ecosystem – slide 16)

GAURANTEED TIMEFRAMES

Legislated process that produces credible and acceptable advice within specified timeframe

*price and risk management parameters

Phased Implementation of Reforms

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