SUBMISSION TO THE STANDING COMMITTEE ON HEALTH, AGED CARE AND SPORT
Inquiry into approval processes for new drugs and novel medical technologies in Australia
13 October 2020

Executive summary:
Sanofi is a leading global biopharmaceutical company focused on human health. We are one of the top five pharmaceutical companies in Australia, employing more than 700 people across every state and territory.

Our portfolio spans rare diseases, oncology, rare blood disorders, chronic conditions, vaccines and consumer healthcare solutions. This includes:

- Sixty medicines on the Pharmaceutical Benefits Scheme (PBS);
- Nine on the Repatriation Schedule of Pharmaceutical Benefits (RPBS);
- Seven vaccines on the National Immunisation Program (NIP);
- Five treatments on the Life Saving Drugs Program (LSDP);
- Two treatments on the National Blood Authority (NBA) tender; and
- An extensive portfolio of consumer healthcare products.

The breadth of our experience and commitment to innovation, targeted specifically towards rare conditions and areas of unmet clinical need, provides us with a unique perspective on the access environment and approval processes that impact a broad range of medicines, vaccines and medical technologies. As such, we are well placed to provide comment to this inquiry.

The right policy settings are vital if Australia is to retain its position as a first wave country for registration and reimbursement of new medicines and vaccines, and to support R&D investment. This in turn will contribute to a strong economy, since it has been shown quality use of medicines is a good investment in health, productivity and economic prosperity.

To date, policy reform relating to access to medicines has generally occurred at an individual reimbursement program level, rather than via holistic reform. As demonstrated by recent cell therapy experiences, the innovations of the future will not be best served by this discrete approach. While the introduction of the National Health Reform Agreement (NHRA) is a welcome step in addressing the issue of funding high-cost therapies, which are initiated in hospital, consideration needs to be given to developing seamless access and funding pathways to support patients as they transition between treatment settings. Further, despite the absence of an explicitly specified ICER threshold, current precedents based on Pharmaceutical Benefits Advisory Committee (PBAC) recommendations for vaccines compared to pharmaceuticals, and within pharmaceuticals - between treatments which offer survival benefits versus those which treat chronic diseases - warrant closer scrutiny as to whether they reflect an appropriate level of willingness to pay for innovation and truly reflect societal preferences.

It is Sanofi's recommendation that the review of the National Medicines Policy (NMP) provides the ideal mechanism to achieve the integrated and comprehensive reform required to ensure Australia's approval processes remain efficient, fit-for-purpose and equipped to appropriately inform decision-making about how best to allocate investment to optimise health outcomes for all Australians.

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Sanofi believes there are three key pillars where reform should be focussed:

1. Ensure Australia adopts the right access and pricing policy settings to appropriately value the benefits that innovative therapies deliver, including those which accrue beyond the health system, to ensure we remain a first wave country for launch of innovative therapies and to support ongoing industry investment, jobs and long-term sustainability;
2. Accelerate the application and assessment process and reduce churn by aspiring to have world-leading access pathways that provide transparency, certainty and process efficiency for industry and will ensure accelerated access to life changing medicines for Australians while bringing times to access back in line with comparable OECD countries; and
3. Support development of the most innovative medicines e.g. develop new and adaptable frameworks to accommodate new scientific findings; support market entry of innovative treatments; and appropriately consider and value their complex impacts on health outcomes.

Response to Terms of Reference:

Terms of Reference #1:

The range of new drugs and emerging novel medical technologies in development in Australia and globally, including areas of innovation where there is an interface between drugs and novel therapies.

Unfortunately Australia is not considered a world leader when it comes timely patient access to pharmaceutical medicines and vaccines.[1] Currently, lack of integration and predictability across regulatory and reimbursement processes involving multiple bodies within the Department of Health extends timelines needed to reach an outcome that enables patient access. As a result, it is vital the healthcare system continues to evolve and is appropriately structured to take advantage of the potentially lifechanging, and even curative, vaccines and therapies that are already available in overseas jurisdictions. See our recommendations made in response to TOR 4 for further information.

Terms of Reference #2:

Incentives to research, develop and commercialise new drugs and novel medical technologies for conditions where there is an unmet need, in particular orphan, personalised drugs and off-patent that could be repurposed and used to treat new conditions.

The reinstatement of the Life Saving Drugs Program Expert Panel has been a positive step forward with respect to patient access to medicines for rare diseases. However, the ongoing requirement for innovative treatments for rare diseases to first be rejected by the PBAC before being able to be considered for funding via the LSDP results in a significantly longer and resource intensive path to reimbursed access compared to other innovative therapies.

In addition, there are currently several services that patients with rare disease require as part of the diagnosis and management of their condition, which are not funded by the Commonwealth or State governments. Consequently, these are subsidised by the sponsors of the treatments for rare diseases.

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The ability to continue to fund these services in the face of ongoing price erosion for rare disease treatments brings to light the current challenges resulting from the cross subsidisation required in the absence of appropriate funding pathways, which consider the totality of the patient journey from diagnosis to initiation of treatment and ongoing disease management.

Recommendations:

- The Therapeutic Goods Administration (TGA) and Office of Health Technology Assessment (OHTA) to develop clear and more inclusive processes, including the acceptability of several sources of scientific evidence, such real world evidence (RWE) to capture the value for patients and their families, and pathways for sponsors considering submitting applications for the repurposing of medicines already approved for use in treatment of other conditions.
- Develop and implement, as per other OECD countries, special approaches for evaluation of rare disease treatments/orphan medicines, including expedited processes, as well as consideration of wider elements of value and requirements for additional data collection with later reassessment.
- Ensure sustainability of funding for treatments for rare diseases is not undermined by the cumulative impact of LSDP reviews, as well as the recent application of "PBS-like" pricing policy to a group of products with very different market dynamics to the typical products these polices were designed; and
- Incorporate best practice from international jurisdictions e.g. opportunity to leverage project RENEWAL being undertaken by the FDA to update labelling for older cancer medicines.

Terms of Reference #3:

Measures that could make Australia a more attractive location for clinical trials for new drugs and novel medical technologies.

In Australia, Sanofi is currently running over 40 clinical trials helping more than 800 people gain access to cutting edge therapies. Our local pharmaceutical clinical studies unit employs 70 people and invests a significant amount in R&D each year. Our investment in clinical trials enables early patient access to cutting-edge therapies, delivering improvements in their health and quality of life.

Globally, Sanofi's R&D pipeline contains 83 projects, including 33 new molecular entities in clinical development (or that have been submitted to the regulatory authorities). 34 projects are in phase 3 or have been submitted to the regulatory authorities for approval. As such, new drug and vaccine development policies are important to Sanofi's ongoing ability to conduct research in Australia.

Notwithstanding the advantages making Australia a favourable location for clinical trials, the right policy setting with respect to longer term patient access remain crucial. As experiences in New Zealand demonstrate, conducting clinical trials in countries that are unlikely to support sustainable, affordable access for patients' post-study completion raises a range of ethical considerations. See also Sanofi's response to TOR 4 below.

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Recommendations:

- Streamline ethics committee approvals (like the New Zealand model) with central committees and an online portal for all sites, regardless of whether they are public or private; remove state specific requirements.

Terms of Reference #4:

Without compromising the assessment of safety, quality, efficacy or cost-effectiveness, whether the approval process for new drugs and novel medical technologies, could be made more efficient, including through greater use of international approval processes, greater alignment of registration and reimbursement processes or post market assessment.

It has been shown that quality use of medicines is a good investment in health, productivity and economic prosperity. As such, Sanofi welcomed the government's announcement in the 2020-21 federal budget committing $2.8 billion over the forward estimates in new funding for new medicine listings – and in doing so effectively removing the offsets policy to a value of approximately $700 million per annum.

The recent reforms to the regulatory approval processes to more closely reflect the evolution in the types of products under development and the available evidence base for these products via the introduction of the provisional registration pathway, as well as the willingness to collaborate across jurisdictions via work sharing and comparable overseas regulator pathways, has resulted in a more efficient regulatory environment for therapeutic goods. However, this unfortunately has not yet translated into faster reimbursement timelines and patient access for medicines via the PBS and LSDP, new blood products via the NBA or for vaccines via the NIP, despite the introduction of PBS Streamlining initiatives and LSDP reform. In recognition of the complexity and interdependencies of the healthcare system, a more integrated approach to policy reform is needed.

Recommendations:

- Prioritise a multi-stakeholder review of the NMP to ensure breakthrough medicines, vaccines and therapies can be effectively accommodated and reimbursed in line with current societal expectation, while maintaining choice for Australian patients. As part of the NPM review:
    - Implement a transparent HTA priority setting system with formal horizon scanning processes;
    - Review current HTA processes, including the role and jurisdictions of the current Expert HTA Committees of the PBAC and MSAC, to accommodate treatments which do not neatly fit into one of the existing federally funded schemes of the PBS, MBS, NIP, LSDP or NBA;
    - Consider ways to improve the speed and efficiency of HTA processes by leveraging opportunities to collaborate across global jurisdictions and revisit the way cost-

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effectiveness is measured and adjudged in Australia compared with the methods of other HTA authorities;

- Reduce submission churn for high added therapeutic value treatments by allowing earlier and more meaningful interactions between sponsors and decision makers, such as the PBAC and other key stakeholders, such as treating physicians and patient groups;
- Examine ways to provide equitable funding of combination therapies, particularly as it relates to treatments for oncology, including rare cancers;
- Implement an open and transparent tracking system designed to measure speed to access from registration to reimbursement for new therapies. This system should include benchmarks to other comparable countries and healthcare systems;
- Implement and adhere to review timelines for new medications through the NBA tender process; establish more transparency with the process ; and
- Review and adapt current review processes to allow appropriate and timely assessment of new applications to the NBA.

References:

1. https://medicinesaustralia.com.au/wp-content/uploads/sites/52/2018/10/MA_Compare-final.pdf Accessed October 2020

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