# SUBMISSION TO THE PARLIAMENTARY INQUIRY INTO APPROVAL PROCESSES FOR NEW DRUGS AND NOVEL MEDICAL TECHNOLOGIES IN AUSTRALIA

## EXECUTIVE SUMMARY

Amicus Therapeutics welcomes the opportunity to submit recommendations to the Parliamentary Inquiry into approval processes for new drugs and novel medical technologies in Australia (the Inquiry).

Amicus is a global patient-dedicated biotechnology company that discovers, develops and delivers high-quality medicines for people living with rare diseases. As a company, our decisions are made as if we were living with these diseases, or are a mother or father caring for a child with these disorders. We are in a unique position to do this. Our CEO, John Crowley, has two children living with Pompe Disease and leads Amicus with a purpose of finding treatments for those in our community living with rare diseases.

We're passionate about bringing life-saving treatment to those living with rare diseases and have a strong pipeline of rare disease therapies for diseases such as Fabry Disease, Pompe Disease and Batten Disease, which we are committed to bringing to Australia.

We welcome the ambition of this Inquiry to carefully examine and recommend how Australia's health technology assessment processes evolve and keep pace with the pace of innovation. Importantly, we acknowledge, and thank, the Committee's leadership, together with the Minister for Health, the Hon. Greg Hunt MP, for the focus of this Inquiry on rare diseases. We know you share our belief that the two million Australians with a rare disease[i] have a right to timely equitable access to therapies with the same quality, efficacy and safety as those living with more common diseases. Together, we have the opportunity to bring solutions that address the challenges we face in bringing new therapies within reach of these Australians.

In acknowledging the Inquiry's Terms of Reference, our recommendations centre on three opportunities:

1. Streamlining the registration process for orphan medicines to treat rare diseases;
2. Incentivising the repurposing of medicines for rare diseases; and
3. Improving the way Australia funds medicines for rare diseases through the Life Saving Drugs Program.

We propose:

1. Connecting the TGA's Orphan Drug Designation with automatic entry to the TGA's Priority Review Pathway for registration to reduce time to register therapies for rare diseases by 75 working days.
2. Putting in place a market exclusivity arrangement of seven years that would encourage manufacturers to invest and commit to local research and clinical trials necessary to repurpose medicines for rare diseases. Where clinical trials are conducted in Australia, the period of exclusivity should be extended by a further 2 years to incentivise investment and expand Australia's clinical trial footprint.
3. Extending the waiving of PBAC and TGA submission fees for orphan drug designations to five years to encourage the repurposing of medicines.
4. Streamlining the reimbursement process to deliver clear, transparent and expedited access for LSDP medications. To achieve this, we recommend:
    - Allow the PBAC process to identify that a medicine is for a rare disease to shape the PBAC's consideration and the process thereafter.

- Introduce a streamlined information gathering process for the PBAC prior to commencement of the formal evaluation process to discuss the appropriateness of the medicine as a rare disease therapy, data requirements and implementation to outline a clearer path for assessment.
- Establish a greater and more explicit consideration of quality of life and long-term disability in rare diseases to the PBAC's consideration to balance a perceived focus on patient survival.
- Set out in the Public Summary Documents clear guidelines that indicates a drug falls under the LSDP's jurisdiction and outline key elements to form the LSDP listing.
- Strengthen the PBAC's rare disease expertise, as currently, no member of the PBAC is a rare disease expert.

In implementing these recommendations, we hope that those living with rare diseases can access medicine sooner to help provide a quality of life they deserve. We thank the Committee for their consideration of this submission and welcome the opportunity to discuss any of our recommendations further.

## 1. STREAMLINING THE REGISTRATION PROCESS FOR ORPHAN MEDICINES TO TREAT RARE DISEASES

Rare diseases are often some of the most degenerative, life-threatening and difficult to treat diseases. Delivering novel therapies to these patients as quickly as possible is essential to improving health outcomes. Yet, a 2014 McKell Institute report found that Australians often wait between two and four years longer for rare disease treatment than comparable markets do.[ii] By shortening the registration process, and in turn, time to reimbursement, we can ensure patients needing rare disease treatment have access sooner without compromising on the quality of the evaluation process.

The first piece of this puzzle is registration. Orphan drugs are therapies that treat, prevent or diagnose rare diseases with a very small patient population or that are not commercially viable for another condition. In Australia, this includes therapies for life-threatening and or seriously debilitating conditions that occur in less than 5 in 10,000 Australians.[iii]

Australia's Orphan Drug Program is effective in encouraging therapies to be brought to Australia by waiving the cost of evaluating and registering these therapies and ensures all patients have access to treatments with the potential to improve their lives. Additionally, an orphan drug designation waives fees involved in applying to the Pharmaceutical Benefits Advisory Committee (PBAC) for reimbursement.

Amicus utilised the TGA's orphan drug designation for the registration of our therapy Galafold® (migalastat), for the treatment of Fabry disease. The designation was influential in our commitment to bring this therapy to Australia. We note other jurisdictions have similar schemes including the US Food and Drug Administration's Orphan Drugs Program and the European Medicine Agency's Orphan Drugs Program.

Yet, once a product receives an orphan drug designation, it can progress through Australia's registration process at the same pace as other medicines, despite the severe unmet need amongst these patients. We believe there is an opportunity to better connect elements of the system and drive faster registration for orphan drug indications.

In 2018, in response to the Review of Medicines and Medical Devices Regulation, the Therapeutic Goods Administration (TGA) implemented a Priority Review Pathway for the registration of therapeutics with a high unmet need, which is three months shorter than the standard review process.

This Priority Review Pathway is a critical development for vital and life-saving prescription medicines, yet it hasn't been formally connected to orphan drug designation meaning orphan drugs must still apply for processing. We believe formally connecting the steps in this process will provide certainty for industry and importantly reduce the time to register therapies for rare disease from 225 working days, to 150 workings days, resulting in faster access for patients who need them.

**Recommendation 1: Connect the TGA's Orphan Drug Designation with automatic entry to the TGA's Priority Review Pathway for registration to reduce time to register therapies for rare diseases by 75 working days.**

## 2. INCENTIVISING THE REPURPOSING OF MEDICINES FOR RARE DISEASES

For many Australians living with rare diseases, the promise of repurposing therapies – or identifying new uses of existing or partially discovered therapies – is a symbol of hope that new treatments will be discovered and lead to better health outcomes. In repurposing therapies, effective treatments for diseases may be discovered in a process that is faster and less expensive than starting from scratch, and often with a reduced risk of failure as the safety profile of the medicine is typically well-established[iv].

There are currently no incentives in Australia to encourage the repurposing of medicines. While mechanisms such as orphan drug designation reduce the cost to register and reimburse a new indication, it doesn't support the medicines industry to undertake critical R&D work that enables us to repurpose a medicine.

There are examples of what other nations are doing to encourage the repurposing of therapies. For example,

- The United States set an early standard for orphan drugs program with a program that was originally created in 1983, offering incentives for companies to advance rare disease therapies. The incentives includes an Orphan Disease Tax Credit, which offers a tax credit equal to 25 per cent[1] of clinical trial costs for the development of orphan drugs,[v] seven-year market exclusivity arrangements that prevents generics entering the market for these orphan indications,[vi] as well as fast-tracked and accelerated approval processes including for paediatric indications.[vii] The FDA states the program has successfully enabled the development of over 600 therapies for rare diseases.[viii]
- The European Union offers 10 years of market exclusivity with an additional two years if the company complies with a paediatric investigation plan at the time of orphan medicine designation. Companies also receive reduced fees for regulatory activities as well as protocol assistance and access to the centralised procedure.[ix]
- Taiwan provides a three-to-five-year data exclusivity period for a new indication; depending on whether clinical trials were conducted in Taiwan, as well as a 10-year exclusivity period for orphan drugs.[viii]
- The Republic of South Korea offers de-facto data-exclusivity through its post marketing surveillance requirements, effectively combining the need for a local phase 4 study with a period of data exclusivity that can last up to 10 years, depending on the medical need.[viii]
- Hong Kong provides an eight-year data exclusivity period as part of its patent system to provide incentives for new uses.[viii]

As outlined in these case studies, there is an important opportunity for the Australian Government to work with industry to incentivise investment in treatments for rare diseases. In particular, we would encourage the Australian Government to put in place a market exclusivity arrangement for repurposed medicines that would

[1] The Tax Credit was reduced from 50 per cent to 25 per cent in 2017 as part of the US Administration's Tax Cuts and Jobs Act.

drive manufacturers to invest and commit to the local research and clinical trials necessary to repurpose medicines. This would provide a level of certainty for manufacturers to stimulate investment. Along with the United States, this is done in a number of countries including Taiwan and Korea[x].

Additionally, we would suggest extending the waiving of all PBAC and TGA submission fees related to orphan drugs from the current timeframe of 12 months to the length of the exclusivity period as we believe this would encourage and support companies to continue investigating and following up with expanded populations such as paediatric indications for a therapy.

Recommendation 2: Put in place a market exclusivity arrangement of seven years that would encourage manufacturers to invest and commit to local research and clinical trials necessary to repurpose medicines for rare diseases. Where clinical trials are conducted in Australia, the period of exclusivity should be extended by a further 2 years to incentivise investment and expand Australia's clinical trial footprint.

Recommendation 3: Extend the waiving of PBAC and TGA submission fees for orphan drug designations to five years to encourage the repurposing of medicines.

## 3. IMPROVING THE WAY AUSTRALIA FUNDS MEDICINES FOR RARE DISEASE

Australia's universal health system provides hope to Australians; whether they live with a rare disease or a more common disease, Australians can access innovative medicines that can save a life, extend life, or improve the quality of the life they live. For many Australians living with a rare disease, this hope is provided through Australia's Life Saving Drugs Program (the LDSP).

The LSDP is a critical funding mechanism that allows patients access to life-saving treatment for rare and life-threatening medical conditions, who wouldn't otherwise be able to access the therapies they need. Sixteen medicines are currently made available for 10 rare conditions, including our therapy, Galafold® for Fabry disease. Under the leadership of Minister for Health, the Hon. Greg Hunt MP, four new treatments have been made available during his tenure, which speaks to his commitment to improving the lives of those with rare conditions.

While a commitment to make these often high cost per patient therapies available is critical, the process in Australia to fund these therapies can best be characterised as a journey where we take the scenic route to get to our destination where we detour down roads that are poorly signposted unnecessarily. The opportunity we have is to draw on the strength of Australia's health technology assessment process to refine the process, increase clarity and transparency, and ultimately reduce the time it takes to fund new therapies.

At the heart of the issue is the often unnecessary number of steps involved in making a therapy available on the LDSP. In its current form, companies can not apply to have a therapeutic included on the LSDP. Instead, they traverse the same pathway as all other medicines through the PBAC. As a result of its remit, the PBAC must apply the same lens and hold the evidence up to the same account as all other medicines, despite clinical trials often being significantly smaller as a result of the size of the population affected. There is currently no option for a sponsor to submit for review with a deliberate view to LSDP listing, even if the standard of care for the disease is already funded through this mechanism.

The process in its current form sets medicines up to fail. Once the medicine inevitably fails the test of cost-effectiveness via the PBAC, only then can a therapeutic be considered for the LDSP. The Public Summary Document simply states that the medicine is not cost-effective. Sponsors of these orphan medicines know this

before application, however, they still have to proceed regardless to get to this point. This pathway could be sped up if sponsors could apply directly to the LSDP.

For patients living with a rare disease, urgency is everything as their diagnosis is often delayed, yet this process leads to inefficiencies and delays for patients who need it most. It would be faster, fairer and more rational for medicines to be reviewed by the PBAC from the start as potential candidates for the LSDP. This would allow comparisons with the existing standard of care (if it exists), and lead to clearer scientific discussion of the merits/risks of the medicine. It would also allow the PBAC to make an explicit statement as to whether the medicine meets the criteria for consideration by the CMO for inclusion in the LSDP.

To be clear, we don't expect the process to be any less stringent, or compromise the values of safety and effectiveness, but it does need to be fit for purpose to support Australians living with rare diseases.

We propose several solutions to streamline the process by strengthening the connection points to deliver clear, transparent and expedited access for these medications. A number of these recommendations align with the recommendations of the Australian Government's own Post-Market Review of the Life Saving Drugs Program but are yet to be implemented.

Recommendation 4: Streamline the reimbursement process to deliver clear, transparent and expedited access for LSDP medications. To achieve this, we recommend:

- Allow the PBAC process to identify that a medicine is for a rare disease to shape the PBAC's consideration and the process thereafter.
- Introduce a streamlined information gathering process for the PBAC prior to commencement of the formal evaluation process to discuss the appropriateness of the medicine as a rare disease therapy, data requirements and implementation to outline a clearer path for assessment.
- A greater and more explicit consideration of quality of life and long-term disability in rare diseases to the PBAC's consideration to balance a perceived focus on patient survival.
- Set out in the Public Summary Documents clear guidelines that indicates a drug falls under the LSDP's jurisdiction and outline key elements to form the LSDP listing.
- Strengthen the PBAC's rare disease expertise, as currently, no member of the PBAC is a rare disease expert.

## CONCLUSION

Amicus thanks the Committee for undertaking this critical inquiry, and for providing the opportunity to table important recommendations we believe will deliver timely access to innovative medicines for Australians living with a rare disease that improve their health and afford them a quality of life they deserve.

We share the enthusiasm of our community that this Inquiry can lead to considerable change, and look forward to the chance to work with the Committee and the Government on bringing the recommendations into reality.

At the end of the day, those living with rare disease could be any one of us, our family or our friends. We will continue to advocate for equitable access to treatment for them.

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EMAIL AND MOBILE

## About Amicus Therapeutics

Amicus Therapeutics is a global, patient-centric biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare metabolic diseases. The cornerstone of the Amicus portfolio is migalastat, an oral precision medicine for people living with Fabry disease who have amenable genetic mutations. Migalastat is currently approved under the trade name Galafold™ in the European Union, USA and Japan, with additional approvals granted and pending in several geographies. The lead biologics program in the Amicus pipeline is AT-GAA, a novel, late-stage., treatment for Pompe disease. The Company is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases.

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