Cystic Fibrosis Australia Submission
Health Technology Assessment Parliamentary Inquiry
29 September 2020

Introduction	Cystic Fibrosis Australia (CFA) welcomes the Parliamentary Inquiry into new drugs and novel technologies and it is pleasing that the Inquiry will include a focus on access to treatment for rare diseases and conditions where there is high and unmet need. The Cystic Fibrosis (CF) Community is collectively an active and forceful advocate for CF drugs and treatments that go through the HTA process and we would all like it to be a swifter process where consumers are involved from the very beginning. The CF Community has a wealth of knowledge and real-life experiences that should be considered and will help ensure the Inquiry's outcomes benefit those in need. Consumer organisations are not a mouth piece for Pharma but rather offer personal stories and heartfelt experience that are incredibly important and should be integrated into each step of the HTA process.
Background	Cystic Fibrosis Australia is the peak patient body in Australia and our remit is to support the broad cystic fibrosis (CF) community through Advocacy, Research and Clinical Improvement. CFA is committed to improving clinical practice and patient outcomes with the aim of extending life expectancy to 50 years by 2025. Every four days a baby is born in Australia with CF and more than one million Australians are carriers of cystic fibrosis. CFA advocates for timely drug access, consumer co-design in all activities that impact patients, CF-specific mental health supports, improvements in infection control, gene testing, reduction of the burden of disease and access to the best medical and allied health services. With no government funding CFA is responsible for creating innovative and enduring funding streams, cost-efficient and service-effective business practices and a positive culture of support, respect and celebration. CFA works ceaselessly to fulfil our mission to deliver research and information as the national body, supporting state and territory members with their goals to enhance the quality of life of people affected by CF. CFA will not rest until people with CF have access to the best treatments and medicines and we have been very involved in the HTA process since 2015. New CF treatments add decades of life to people with CF and as Australia has one of the best health systems in the world we should also have access to the best drugs and treatments. For further information please visit our website at https://www.cysticfibrosis.org.au/

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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.*** **Current Issues:** • Speed to market in Australia when drugs are available overseas • Lack of transparency by pharmaceutical companies • The HTA process and submission timing relating to a specific drug or treatment are not agreed to by Government and sponsor and are not shared with consumers to manage expectations • No use of Patient Reported Outcomes (PROs) or Patient Experience Outcomes (PREMs) • No consumer consultation at TGA stage or prior. • Lack of clinician education about access for patients via the TGA • Sponsor registration in Australia and their intent to seek reimbursement in Australia • Diagnostics approvals to help with treatment choices not always approved. **Solution:** • Work collaboratively with … ○ Consumers and consumer organisations to access and understand real world evidence plus PREMs and PROMs ○ International regulatory agencies to speed up and share the approval process ○ Pharmaceutical companies and demand their commitment to providing their products in a timely and cost-effective manner. ○ Consumers must be part of the registration process ○ Establish a HTA communication and education process for clinicians. • Establish an Office for Rare Disease.
	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. **Current Issues:** • Innovative clinical trial not encouraged and incentivised – N of 1, adaptive, organoids and basket trials are all available in Australia • Australia not seen as a country of choice due to population number • Rare diseases that don't meet the 'orphan drug' definition have no special approval pathways • Double blind placebo clinical trials are still seen as the only option or 'best practice' method, especially in rare diseases • Treatments are not personalised and precision medicine is not embraced and encouraged

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	• Research into 'evidence gaps' for rare diseases should be funded • Patient Reported Outcomes (PROs) or Patient Experience Outcomes (PREMs) are not collected during research and clinical trials • Repurposing of existing treatments are not incentivised and acceleration pathways don't exist • Patient organisations are not always involved in research and funding does not go to what consumers really want and need. **Solution:** • Insist on consumer co-design and consumer consultation • Streamline and nationalise ethics processes – establish a Central (National) Ethics Committee for research and clinical trials • Embrace new research technologies and encourage them as part of the drug approval process - N of 1, adaptive, organoids, basket trials – establish a government supported program • Establish a consumer consultation process to highlight where research is needed • Establish an Office for Rare Disease
	• ***Measures that could make Australia a more attractive location for clinical trials for new drugs and novel medical technologies.*** **Current Issues:** • There is no government support for disease specific clinical trial networks. • International clinical trials are not incentivised to include Australia by providing benefits such as an expedited HTA approval process when Australian data is available • National infrastructure for clinical trials • No streamlined national ethics approval process • Every Australian should have access to clinical trials – • Not for Profit (NFP) consumer bodies should be supported to aid clinical trial participant recruitment • Embrace innovative clinical trials that include rural and remote communities. **Solution:** • Insist on consumer co-design • Encourage and incentivise patient organisations to establish Clinical Trial Networks. Patient organisations are very effective CI and participant recruiters. • Clinical trials to be discussed with patients – location barriers to be identified in the trial design • Place added value or a 'positive weighting' on clinical trials done in Australia • Establish a Central (National) Ethics Committee for research and clinical trials to streamline and nationalise processes • Review outcomes of trials as part of funding process • Embrace and encourage the use of new research technologies and encourage them in clinical trials - N of 1, adaptive, organoids, basket trials

	• Establish an Office for Rare Disease
	• ***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.*** **Current Issues:** • Open collaboration with FDA, EMA and FDA is not apparent • Clinical registries should be accredited and then supported by Government for use in the drug evaluation process (pre/post marketing) • International reimbursement contract negotiations are not apparent. • Immediate access to life saving drugs following TGA approval should be allowed. The PBAC process and negotiations can then run while consumers benefit from treatments. This would also provide valuable data through post marketing surveillance. • Pathways established for submissions where there is no sponsor but benefit and patient need can be demonstrated • Lack of transparency with sponsor submissions. Not enough information is available in the public domain • No set time limits for commercial (pricing) negotiations • New clinical trial techniques are not valued in the reimbursement process • Consumers are not part the HTA process from the beginning • Patient Reported Outcomes (PROs) or Patient Experience Outcomes (PREMs) are not part of HTA submission • Consumer comments are not part of a pharmaceutical company's TGA and PBAC submissions • Consumers are not provided support, education and updates throughout the process by either the sponsors or Government • Compassionate access is not available for a great number of people who are critically ill. **Solution:** • Insist on consumer consultation and sharing of real-life evidence 'up front' in the process • Encourage and incentivise patient organisation to be involved in the process • Provide consumers with more information about the submission • Encourage pharmaceutical companies to collaborate with patients when preparing their HTA submissions • Embrace new research technologies to streamline and speed up the assessment of safety, quality and efficacy - N of 1, adaptive, organoids, basket trials • Establish an Office for Rare Disease • Work with countries like UK, Canada and NZ to secure a 'value for money' reimbursement contract • Use Consumer Organisation Data Registries for pre and post marketing surveillance • Consider the German model of immediate access once EMA has approved a drug. Financial negotiations take place after that when quality and efficacy can be gauged in the whole population.

Who	Cystic Fibrosis Australia and the CF Community were involved in the HTA process when Kalydeco, Orkambi and Symdeko were submitted by Vertex. We are now embarking on our Trikafta advocacy and hope this Parliamentary Inquiry will speed up the process. Every day that people are denied access to life saving drugs their lung health declines and it is important to keep in mind that lung damage is a key 'death indicator'.
What	The CF Community is asking for three things: 1. More consumer involvement in the HTA process by Government Departments and Pharmaceutical companies - Nothing About Us, Without Us! 2. A national body for Rare Diseases and one for central ethics and clinical trial regulatory affairs - Every Australian should have access to clinical trials. 3. Consider immediate access to drugs and treatments following safety and efficacy approval. Reimbursement and 'value for money' conversation could be held when the drug is 'in market' and truly representative data is available through local data registries. We admit that our regulatory process (TGA and PBAC) is robust and trustworthy but a patient's access to life saving drugs is often slow and hampered by commercial considerations.
When	Changes to the HTA process that will ... • embed consumers in the total process • speed up the process and access to drugs and treatments and • result in better outcomes for patients ... is a health emergency and must happen immediately. Cystic Fibrosis Australia is keen to assist in any way and if a trial of new protocols and procedures is planned we would love to be part of it. Our community is engaged and experienced in the HTA process.
How	Consumers and their patient organisations see the HTA process from a unique angle and we must be part of the Parliamentary Inquiry and subsequent HTA review if health outcomes for all Australians are to improve.

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Why	Let's not forget a key tenet of our national anthem … **With courage let us all combine to Advance Australia Fair** And when it comes to rare disease … **you can't equate "rare" or "small numbers" with "insignificant"**
Outcomes	1. A patient centric HTA process 2. Millions of Australians living with a rare diseases are not and seen as "insignificant" 3. Australia leads the world in innovative research, clinical trials and timely drug access 4. International pharmaceutical companies add Australia to their trial ledger and more Australians get access to clinical trials 5. Suffering is reduced and lives are saved.
Recommendation	**The CF Community is asking for three things …** **1. Involve consumers at all points in the HTA process** **2. Establish a Rare Diseases Office and an Office for Central (National) Ethics and Clinical Trial Regulatory Affairs** **3. Provide patients with immediate access to drugs and treatments following safety and efficacy approval by TGA. Commence reimbursement discussion when the drug is 'in market'.**
Conclusion	Cystic Fibrosis Australia is thrilled that the House of Representatives Parliamentary Inquiry is underway, and we believe Trent Zimmerman and Mike Freelander are very appropriate Chairs. Over the years, through the HTA process, I have had the opportunity to work with some outstanding Department of Health, TGA, PBAC and Ministerial staff. I know that they all highly regard consumers and the value that they can bring to a discussion or negotiation. Further engagement with consumers could only enhance the HTA process so I hope that we will be included at every step of the way. Kind regards [redacted] Nettie Burke, CEO Cystic Fibrosis Australia

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