

15 June 2023



BEYOND THE DIAGNOSIS

From the moment a person is diagnosed with cystic fibrosis (CF), family and friends are continually searching for ways to help support their loved one, so they can be and feel better.

The evolution of CF care has come a long way, with the life expectancy of a child born with CF steadily increasing. However, this isn't to say there are no challenges within the CF Community. Many people with CF are ineligible for current 'life-changing' treatments or won't respond to them. This is not okay, and we are actively working to ensure all people living with CF have the effective therapies they need.

Supporting researchers and advocating for effective therapies that are **accessible to all people** who will benefit from them is a priority for Cystic Fibrosis Australia, alongside the hope for a cure.

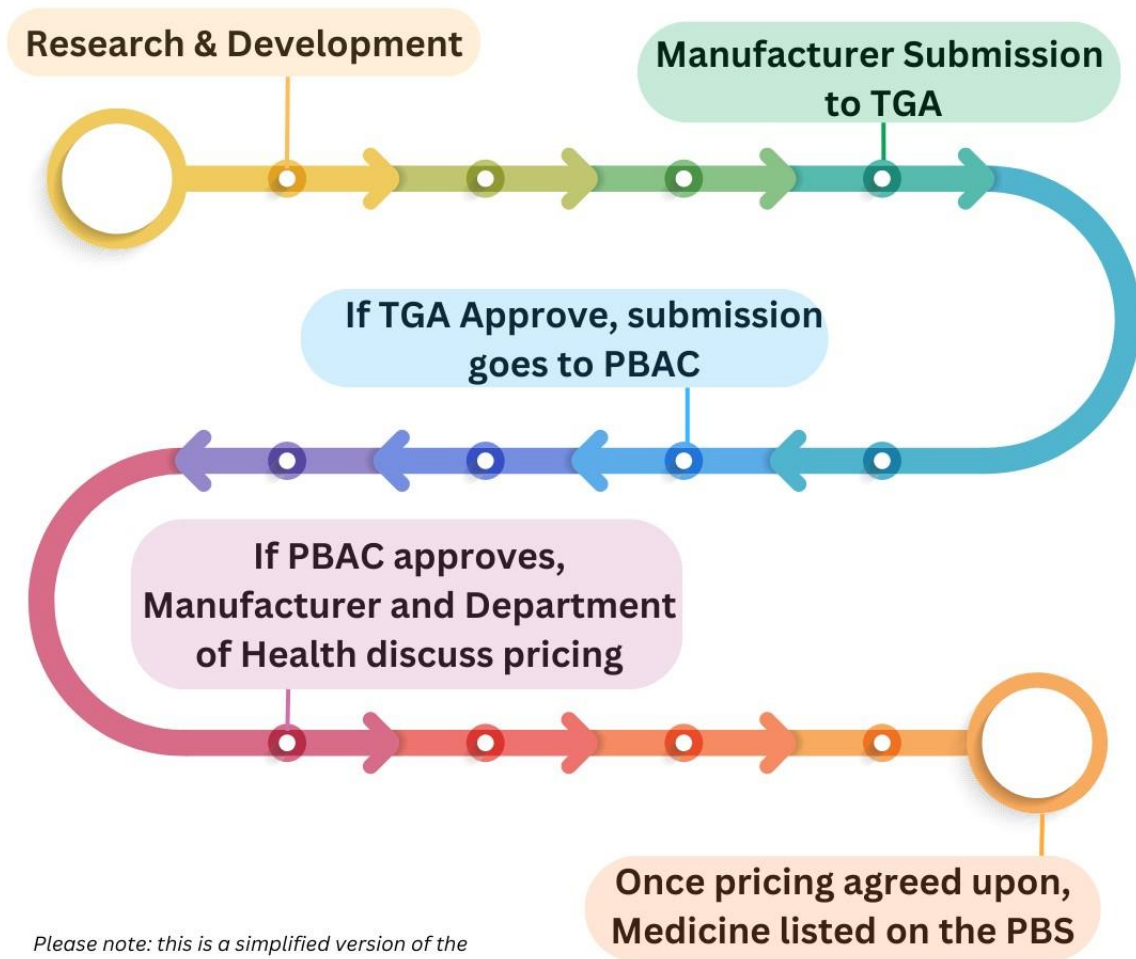
Research and Development (R&D) is the foundation of the medicines industry; however, the process of R&D is not only time-consuming but also very costly. Teams of researchers run clinical trials to develop new therapies and different phases are conducted to assess safety and effectiveness.

Rare CF Gene Mutations make this process challenging.

Whilst there is no typical length of time it takes a drug to go through the R&D stages, it can often take years (even decades), and only a small percentage of these make it from discovery (the first step in R&D) to market (in the patient's hands).

Having a medicine listed on the market and on the Pharmaceutical Benefits Scheme (PBS) means it is available to be dispensed to patients at a government-subsidised price.

The process of getting medicines from laboratory testing to patients can also be complex and time-consuming.



Please note: this is a simplified version of the steps involved for a medicine to be listed on the PBS. Many variables can change this process.

Once a manufacturer (pharmaceutical companies) has completed their R&D, they must have their medicine approved by the Therapeutic Goods of Administration (TGA). The TGA is responsible for monitoring the quality, safety, and efficacy of medicines on the Australian market.

If the manufacturer's submission of their medicine shows effectiveness, safety, and high quality, the TGA will approve the medicine.

This approval takes the manufacturer's submission to the next stage where it is assessed by the Pharmaceutical Benefits Advisory Committee (PBAC). The [PBAC has its own set of requirements](#) the submission must meet including (but not limited to):

- Comparative Health Gain
- Comparative cost-effectiveness
- Patient affordability in the absence of PBS subsidy
- Predicted use in practice and financial implications for the PBS

- Predicted use in practice and financial implications for the Australian Government health budget.

These requirements must be discussed before the medicine can be recommended to the Health Minister or be listed on the PBS.

Unfortunately, because there is limited funding in the Australian health budget, not all medicines will be subsidised.

Once a medicine is approved by the PBAC, the medicine Manufacturers then enter into pricing discussions with the Department of Health.

Once Pricing arrangements have been agreed upon, the medicine is then listed on the PBS for all Australians.

The process of getting medicines listed on the PBS is currently [under review](#), and Cystic Fibrosis Australia is part of this process. We value the importance of reducing the time it takes for Australians to access new health technologies, and that the current process must be re-assessed.

The creation of new medicines and getting them into the patient's hands is time-consuming and costly. At Cystic Fibrosis Australia, we understand these difficulties, particularly when people with CF need support today. We believe the process of accessing new therapies should be accelerated and streamlined and hope that with the current review, there will be significant progress to help all people have access to the medicines they need as soon as they are available.

It is crucial to understand that by donating to Cystic Fibrosis Australia today, you are investing in the future of CF.

Our ongoing advocacy, support of research, and involvement in varied collaborative efforts both locally and internationally are fundamental for the prospects of the Australian CF Community.

Please support our work by donating today.

All donations above \$2 are tax-deductible.

DONATE TODAY