

23 June 2023

### **The Power of Perseverance**

Last night we held our June CF Connect Session on Rare Gene Mutations. We were delighted to welcome Prof Adam Jaffe and Dr Shafagh Waters to the panel, each provided an incredibly insightful update of what they are working on and experiencing, within the space of rare CF gene mutations.

Key Takeaways from the session:

- Research and development efforts are underway for rare gene mutations; however, it's important to acknowledge that there are currently over 2000 mutations, each with unique responses to treatments and therapies
- Research and Development is continuously broadening the scope of cystic fibrosis (CF) treatment beyond modulators to encompass other various scientific avenues, including mRNA, gene therapy, and stem cell replacement therapies
- Shafagh's research is exploring the importance of personalized medicine for CF, particularly supporting people with rare gene mutations
- Emphasizing the need for a clear pathway to be established within Australia, to facilitate compassionate access and support the availability of alternative options for people with rare mutations. Details on the HTAi Review can be found [HERE](#)
- Acknowledging that significant progress has been made thus far and everyone on the panel expressed a strong perseverance in pursuing ongoing efforts to address the needs of all individuals affected by CF, regardless of their specific mutations.

[Watch the Recorded Session Here](#)

As mentioned in the session, Cystic Fibrosis Australia CEO Jo Armstrong will be attending the [HTAi Annual Meeting](#) over the coming week.

***“I understand the immense importance of ensuring there are changes made to our system so that there are pathways to ensure everyone has access to the therapies that will benefit them. The CF community should not have to wait so long for access***

*to affordable treatments. Australia currently has an outdated process that needs to be changed and hopefully, the HTA review will be a driving force for the change we need”.*

Cystic Fibrosis Australia understands the many difficult challenges the CF community face each day. As the peak consumer body for all Australians living with cystic fibrosis, our [strategic priorities](#) are extensive.

We can not achieve this alone and donations are critical to ensure our work continues.

There is one week left until the end of the financial year. Donate before the end of this financial year to include in your tax return. All donations over \$2 are tax-deductible.

[Thank you for your donation](#)

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