Alpha-1 Antitrypsin Deficiency and the Australian Context

Background

- There is only ONE treatment available to protect the lungs of people with the genetic condition known as Alpha-1 Antitrypsin Deficiency. The treatment is Alpha-1 proteinase inhibitor (also called Augmentation Therapy) and has been successfully used for over 30 years overseas.
- The Therapeutic Goods Administration (TGA) registered two proteinase inhibitors in 2017 and included both on the Australian Register of Therapeutic Goods (ARTG) but the essential treatment has never been made available at a subsidised cost, which has resulted in many people dying prematurely and others suffering with preventable lung disease, as they can't afford to privately purchase this essential treatment.
- Alpha-1 antitrypsin deficiency (Alpha-1) is a genetic disorder predisposing people to early onset lung emphysema, lung cancer, liver cirrhosis, liver cancer, bronchiectasis, inflamed blood vessels and a shortened life expectancy.
- Augmentation therapy supports proper immune function (balances lung neutrophils, reduces inflammation and stops lung disease progression).
- Alpha-1 antitrypsin is a natural protein made mostly in the liver but in individuals with Alpha-1, the antitrypsin is trapped or destroyed, so it can't travel to organs including the lungs to protect them from the innate immune system e.g. white blood cells called neutrophils which destroy the lungs if not protected by antitrypsin.
- Without normal levels of antitrypsin, neutrophils attack healthy lung tissue when they are activated by viruses, bacteria, or dust and other irritants breathed in every day.
- When the body can't make enough antitrypsin to keep neutrophils in balance, the neutrophils dominate and cause more inflammation and rapid lung destruction i.e. rapid worsening of emphysema leading to a shortened life expectancy.
- Most people with Alpha-1 are dead by age 60, as most can't afford to privately purchase augmentation therapy which has an average cost of $100,000 per annum.
- Alpha-1 proteinase inhibitor is equivalent to giving insulin to diabetics (i.e. replacing an essential protein for normal healthy body functioning).

Augmentation Therapy – Evidence Supports its Use in Severely Deficient Patients

- Augmentation therapy is made from donated plasma which contains antitrypsin.
- Augmentation therapy has been successfully used in Europe, Canada and the USA since the 1980s and more recently in other locations (e.g. Denmark).
- The price of augmentation therapy is roughly the same regardless of which company provides it in Australia (i.e. Grifols' *Prolastin-C* and CSL Behring's *Zemaira*).
- Evidence shows that augmentation therapy (i.e. weekly infusions) restores the antitrypsin/neutrophil balance and stops inflammation and further lung destruction but the Government's advisory committee (the Medical Services Advisory Committee) has not recommended government subsidy.
- One vial of augmentation therapy costs approximately 500AUD. Dosage is weight dependent. Someone weighing around 65 kgs requires 4 vials per week. It is estimated that the average cost for private purchase per patient per year is $100,000.
- The Australian Government has not subsidised the cost of augmentation after considering the evidence in relationship to clinical effectiveness and cost-effectiveness. This decision is despite the fact that augmentation therapy is successfully being used to slow lung decline and extend life to "normal life expectancy" in other countries.
- People with a severe deficiency and emphysema need urgent access to affordable treatment.
- All Alpha-1 lung disease patients have a right to the best possible health care.
- Subsidised treatment will save lives, reduce hospitalisation from lung infections and avoid lung transplant surgery.