

Case study



Liz Brennan wants the drug for daughter Annabelle

The British Isles are home to only a hundredth of the world's population but almost a fifth of its cystic fibrosis patients. Britons are twice as likely to have the disease as the French and the Danes, and ten times as likely as the Finns.

What is going on?

The answer is in our DNA. Cystic fibrosis happens when a child inherits a set of faulty genetic instructions from each parent. Most carriers have no idea that this sequence is in their DNA, and couples who both carry the mutation will often have healthy children.

Until two and a half years ago, Liz and

Shane Brennan were one such couple. They had two sons, Finley, who is now nine, and Oscar, who is five, with no unusual health problems. Then their baby daughter, Annabelle, had cystic fibrosis diagnosed.

Annabelle is one of the children who could benefit from Orkambi. Today Mrs Brennan will travel from her home in Yate, south Gloucestershire, to the Houses of Parliament to call on the NHS to make the drug available to her daughter. "Without access to these ground-breaking drugs that tackle the root cause of the condition, instead of just treating the symptoms, Annabelle's future will continue to be uncertain and hard," she said.

Today families will gather outside the seats of government in the UK's four nations to lodge a protest on behalf of patients. In a letter to Simon Stevens, the chief executive of NHS England, the charity has warned that patients are suffering irreversible harm.

"People have been waiting since October 2015 for access to a treatment that could have prevented them developing permanent lung damage," David Ramsden, the head of the charity, wrote. "I am writing to ask you to do all in your power to change this situation

now before yet more damage is done." Orkambi is in the vanguard of a body of "precision" medicines that are forecast to cover more than 90 per cent of cystic fibrosis patients over the next five years.

The Cystic Fibrosis Trust is worried that the pricing model, which is based on the quality-adjusted life year (Qaly), will mean that the UK ends up repeatedly blocking Orkambi.

By the medicines agency's calculations Orkambi costs £216,000 per Qaly, more than seven times the £30,000 cost-benefit threshold.