



Extended Research Article

Ivacaftor–tezacaftor–elexacaftor, tezacaftor–ivacaftor and lumacaftor–ivacaftor for treating cystic fibrosis: a systematic review and economic evaluation

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Plain language summary

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Plain language summary

This project reviewed the medical benefits, risks and costs of three treatments for cystic fibrosis: elexacaftor/tezacaftor/ivacaftor, lumacaftor/ivacaftor and tezacaftor/ivacaftor. They correct the underlying cause of cystic fibrosis in people who have a specific faulty version of the cystic fibrosis transmembrane conductance regulator gene called *F508del*.

A thorough search of medical journals and other relevant publications was undertaken to identify evidence on how well each treatment works. People treated with elexacaftor/tezacaftor/ivacaftor had large increases in lung function and other markers of overall health than people not treated with this medication, and this was expected to make them live longer. People treated with lumacaftor/ivacaftor and tezacaftor/ivacaftor also had increases in lung function, but this was not as large an improvement as with elexacaftor/tezacaftor/ivacaftor. These treatments have only been widely available in the United Kingdom since 2019 (lumacaftor/ivacaftor and tezacaftor/ivacaftor) or 2021 (elexacaftor/tezacaftor/ivacaftor), and so there is still uncertainty about their long-term effectiveness.

This project also assessed whether these treatments are likely to be considered good value for money for the National Health Service. The analysis found that based on the current prices of these treatments, they are unlikely to be considered good value for money for the National Health Service.

In summary, lumacaftor/ivacaftor and tezacaftor/ivacaftor appear to be effective, and elexacaftor/tezacaftor/ivacaftor appears to be very effective, at improving the health of people with cystic fibrosis, but they are also very expensive.

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This article

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