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FDA doesn't approve Pharmaxis' Bronchitol

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Singapore: Pharmaxis received a complete response letter from the US FDA confirming that Bronchitol cannot yet be approved for marketing for the treatment of cystic fibrosis in the US.

The FDA concluded in its review of the Bronchitol New Drug Application (NDA) and recommended Pharmaxis conduct an additional clinical trial to obtain an approval for Bronchitol. The complete response letter stated: "The submitted data do not provide a favourable benefit-risk balance to support the use of inhaled mannitol in patients with cystic fibrosis 6 years of age and older. The determination of efficacy based on the two clinical trials are not adequate because of the treatment-related frequent early dropouts in trial 301 for which the primary statistical analyses did not account and the lack of statistical significance in trial 302 for the primary endpoint". In relation to safety, the FDA stated its concern with the occurrence of haemoptysis, particularly in paediatric patients.

Pharmaxis CEO, Mr Gary Phillips, said, "We are clearly disappointed that Bronchitol cannot yet be made available to patients in the US. The FDA has provided guidance on the necessary measures to gain approval and Pharmaxis will now have a follow up meeting with the FDA. This will be a Type A meeting which I expect will take place next quarter and will examine the parameters of an additional clinical trial including how best to incorporate both adult and paediatric patients.

"At the recent Pulmonary-Allergy Drugs Advisory Committee (PADAC) meeting we received strong support from the US CF Foundation, leading CF clinicians and patients who spoke passionately about the need for Bronchitol. The company remains committed to bringing Bronchitol to CF patients in the US and the onus is now on Pharmaxis to work with the FDA to ensure Bronchitol is approved as soon as possible."

The FDA has previously granted Bronchitol Orphan Drug designation for the treatment of patients with cystic fibrosis. The product is approved for marketing for patients aged six years and over in Australia and for patients aged 18 years and over throughout the European Union.