European Ombudsman

Ombudsman finds no undue delays in how EMA handled complaint about cystic fibrosis medicine authorisation

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The Ombudsman has found there were no undue delays in how the European Medicine Agency (EMA) handled a request to authorise a medicine used to treat cystic fibrosis, and commends the clear and attentive way EMA communicated with the complainant whose son has this serious illness. During the Ombudsman's inquiry the medicine was approved.

The complainant's three-year-old son has a specific form of cystic fibrosis, a serious genetic illness that affects the lungs, the digestive system and other organs. He turned to the Ombudsman in February 2020 with concerns that EMA was taking too long to authorise the medicine Kalydeco for use in treating children with the specific genetic form of cystic fibrosis that affects his son.

When the complainant first contacted EMA, in 2018, Kalydeco was not approved for use for children with the form of cystic fibrosis affecting the complainant's son; it was approved for adults with that form of the illness. The Ombudsman's inquiry showed that EMA kept in regular contact with the complainant, and informed him of what steps had to be taken for Kalydeco to be approved for children.

EMA explained to the complainant that Vertex, the company marketing the drug, would have to

apply to EMA to get its existing market authorisation extended and demonstrate the safety of this new use. EMA took the additional step of writing to Vertex, which then submitted an application to EMA for extending the approval of Kalydeco to include its use for children with this specific form of cystic fibrosis. After following the standard procedure for reviewing such applications, EMA approved the extended ‘market authorisation’ in April 2020. EMA then directly informed the complainant by email of the decision.

The Ombudsman concluded there had been no undue delays in the process and noted the care EMA had taken in being transparent about the process and keeping the complainant informed.