

CF EUROPE CALLS ON CHMP TO EXPAND ACCESS TO KAFTRIO FOR ALL CF VARIANTS

CF Europe, representing cystic fibrosis (CF) patient associations across Europe, urges the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency to broaden access to Kaftrio for all individuals with CF, regardless of their genetic variant.

Currently, treatments like Kaftrio are approved based on evidence linked to specific genetic variants, leaving many patients with rare mutations excluded from life-changing therapies.

Strong clinical evidence and calls for action

A recent French study, led by Professor Pierre-Régis Burgel, demonstrated that more than 50% of CF patients whose mutations are not covered by Kaftrio's current indications benefit from the treatment. This finding has enabled patients in France to access Kaftrio through trial periods and clinical assessments, thanks to the advocacy of patient organizations and CF centers.

However, in many other European countries, patients continue to be excluded.

Francesca Farma, the mother of a young boy with CF, shared her heartbreaking experience:

"Knowing that an approved drug could transform my son's life but seeing it under review for more than a year is unbearable. Meanwhile, his condition worsens. This is inhumane."

A call for a patient-centered approach

CF Europe calls on the CHMP to adopt a more inclusive evaluation process, focusing on evidence at the CFTR protein level rather than solely on genetic variants. Such an approach would accelerate access to life-saving treatments for all those who could benefit.

"We must change how we approve innovative therapies. Behind the data and dossiers are real lives, and every day of delay can have irreversible consequences," said Hilde De Keyser, CEO of CF Europe.

CF Europe remains committed to working closely with regulatory bodies and patient organizations to ensure equal access to life-changing treatments for all individuals with CF.

About CF Europe

CF Europe is the federation of national CF associations in Europe, dedicated to improving the lives of people living with CF through advocacy, research, and collaboration.

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