



April 21, 2021

Matt Anderson
Assistant Commissioner and Medicaid Director
Minnesota Health Care Administration

Dear Director Anderson,

On behalf of the approximately 700 people in Minnesota living with cystic fibrosis (CF) and their families, we thank Minnesota Medicaid for including elexacaftor/tezacaftor/ivacaftor (Trikafta®) on its drug formulary. We appreciate the inclusion of this transformative drug to your state's Medicaid formulary; however, we write to express our concerns regarding the requirement that adult patients aged 18 and older must have an eye exam prior to elexacaftor/tezacaftor/ivacaftor reauthorization.

Cystic fibrosis experts agree that, for adult patients, elexacaftor/tezacaftor/ivacaftor access should not be contingent on regular eye exams – this consensus is also reflected in the Food and Drug Administration's (FDA) label. Furthermore, this requirement results in Minnesota Medicaid paying for additional unnecessary services. As such, the CF Foundation requests the removal of this requirement for elexacaftor/tezacaftor/ivacaftor reauthorization. We are available to you to assist in this revision.

About Elexacaftor/Tezacaftor/Ivacaftor

For eligible patients, elexacaftor/tezacaftor/ivacaftor is the most significant therapeutic advance in CF to date. This oral therapy addresses the underlying cause of cystic fibrosis – CFTR protein defects – in individuals with at least one copy of the *F508del* mutation in the *CFTR* gene. Among the modulator class, elexacaftor/tezacaftor/ivacaftor is considered a highly effective therapy. Restoring CFTR function would preserve health and lung function, reduce costly hospitalizations, improve quality of life, and ultimately delay premature death. Longer-term data from first-generation modulators show these improvements are sustained over time; however, modulators cannot reverse existing organ damage.ⁱ For these reasons, elexacaftor/tezacaftor/ivacaftor should be initiated as soon as patients and their physicians determine it is medically necessary and delays in reauthorization should be avoided when possible.

Concerns with adult eye exam criteria

As is indicated in the FDA label, cases of non-congenital lens opacities have been reported in some pediatric patients treated with ivacaftor. However, no such cases have been reported in adults with CF. Recognizing that there are no findings substantiating increased risk to adults, the FDA only recommends baseline and follow up eye exams for patients under 18.ⁱⁱ In your current initial authorization criteria, this is recognized by only mandating an initial eye exam for pediatric patients. In contrast, the reauthorization criteria does not have this distinction and requires all patients (adult and pediatric) prove they do not have cataracts or lens opacities. Requiring this documentation for adult patients adds undue burden to adults with CF by necessitating an eye exam and may result in delays or gaps in treatment that could further disease progression and irreversible organ damage

caused by CF. Additionally, requiring these eye exams results in unnecessary costs to the state and health care system. Removing this requirement would both better reflect the FDA label for elexacaftor/tezacaftor/ivacaftor and save Minnesota Medicaid the costs of unnecessary eye exams for adults with CF.

We stand ready to answer any questions about elexacaftor/tezacaftor/ivacaftor or other CF treatments. Please contact Adam Kellermann, State Policy Specialist, at akellermann@cff.org or (240) 200-3713. We would be happy to connect you with local CF experts to further discuss this important issue.

Sincerely,

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ⁱ Volkova, N et al. "Disease Progression in patients with cystic fibrosis treated with ivacaftor: Data from national US and UK registries". *Journal of Cystic Fibrosis* (2019): [e-pub]

ⁱⁱ Trikafta™ (elexacaftor/tezacaftor/ivacaftor) [package insert]. Boston, MA: Vertex Pharmaceuticals, Inc.; 2019.