| Effective Date: 8/1/2026 |
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| Reviewed: 5/2026 |
| Scope: Medicaid |

# KYGEVVI (doxecitine and doxribtimine)

## POLICY

### I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications
Kygevvi is indicated for the treatment of thymidine kinase 2 deficiency (TK2d) in adults and pediatric patients with an age of symptom onset on or before 12 years.

All other indications are considered experimental/investigational and not medically necessary.

### II. CRITERIA FOR INITIAL APPROVAL

**Thymidine Kinase 2 Deficiency (TK2d)**
Authorization of 6 months may be granted for treatment of TK2d when all of the following criteria are met:

1. This medication must be prescribed by or in consultation with a geneticist, neurologist or specialist in the treatment of neuromuscular disorders
2. Documentation of genetic testing that detects biallelic pathogenic variants in the thymidine kinase 2 (TK2) gene confirming diagnosis of TK2d
3. Documentation that the member experienced initial symptom onset (e.g., muscle weakness in limbs, respiratory weakness (including mechanical ventilation or continuous non-invasive ventilation), loss in motor functions (holding head upright, sitting, standing, walking, climbing, running), etc.) on or before 12 years of age
4. Documentation of baseline liver function tests (ALT, AST, total bilirubin) less than two times the upper limit of normal per the laboratory administering the test AND member does not have a history of liver disease
5. Documentation of absence of other genetic disease, polygenic disease or concurrent inborn error of metabolism
6. Member does not have renal insufficiency requiring dialysis
7. Member does not have severe end-organ hypoperfusion syndrome secondary to cardiac failure resulting in lactic acidosis
8. Documentation that the member's dose does not exceed 800 mg/kg/day (consisting of 400 mg doxecitine and 400 mg doxribtimine). Member's current weight and prescribed dose must be provided.

### III. CONTINUATION OF THERAPY

Authorization of 6 months may be granted for continued treatment in members requesting continuation of therapy when all of the following criteria are met:

1. If member has not been approved for this drug by Neighborhood in the past, clinician must submit documentation that initial criteria are met.

2. Documentation with chart notes and/or medical records that the member has demonstrated a positive clinical response to therapy [e.g., improvement or stabilization in motor function (e.g., 6-minute walk test [6MWT], Motor Function Measure [MFM] 20 or MFM 30), pulmonary function (e.g., reduced reliance on ventilatory support), ability to meet growth and developmental milestones (e.g., sitting, standing, walking)]
3. Documentation that the member's dose does not exceed 800 mg/kg/day (consisting of 400 mg doxecitine and 400 mg doxribtimine). Member's current weight and prescribed dose must be provided.

## IV. REFERENCES

1. Kygevvi [package insert]. Smyma, GA: UCB INC.; November 2025.
2. Hernandez-Voth A, Sayas Catalan J, Corral Blanco M, Castaño Mendez A, Martin MA, De Fuenmayor Fernandez de la Hoz C, Villena Garrido V, Dominguez-Gonzalez C. Deoxynucleoside therapy for respiratory involvement in adult patients with thymidine kinase 2-deficient myopathy. BMJ Open Respir Res. 2020 Nov;7(1):e000774. doi: 10.1136/bmjresp-2020-000774. PMID: 33246973; PMCID: PMC7703425.
3. Domínguez-González C, Chiang C, Colson AO, Rebollo Mesa I, Baixauli E, Quan J, VanMeter S, Hirano M. Pyrimidine Nucleos(t)ide Therapy in Patients With Thymidine Kinase 2 Deficiency: A Multicenter Retrospective Chart Review Study. Neurology. 2025 Sep 23;105(6):e213908. doi: 10.1212/WNL.0000000000213908. Epub 2025 Sep 5. PMID: 40911819; PMCID: PMC12413740.
4. ClinicalTrials.gov [Internet]. A Retrospective Study of Subjects With Thymidine Kinase 2 Deficiency (NCT05017818). Last updated 2023 Sep 01. Available from: https://clinicaltrials.gov/study/NCT05017818 [clinicaltrials.gov]
5. ClinicalTrials.gov [Internet]. Doxecitine and Doxribtimine-Expanded Access (NCT06590493). Last updated 2025 Sep 12. Available from: https://clinicaltrials.gov/study/NCT06590493 [clinicaltrials.gov]