Understanding KYGEVVI™ (doxecitine and doxribtimine)

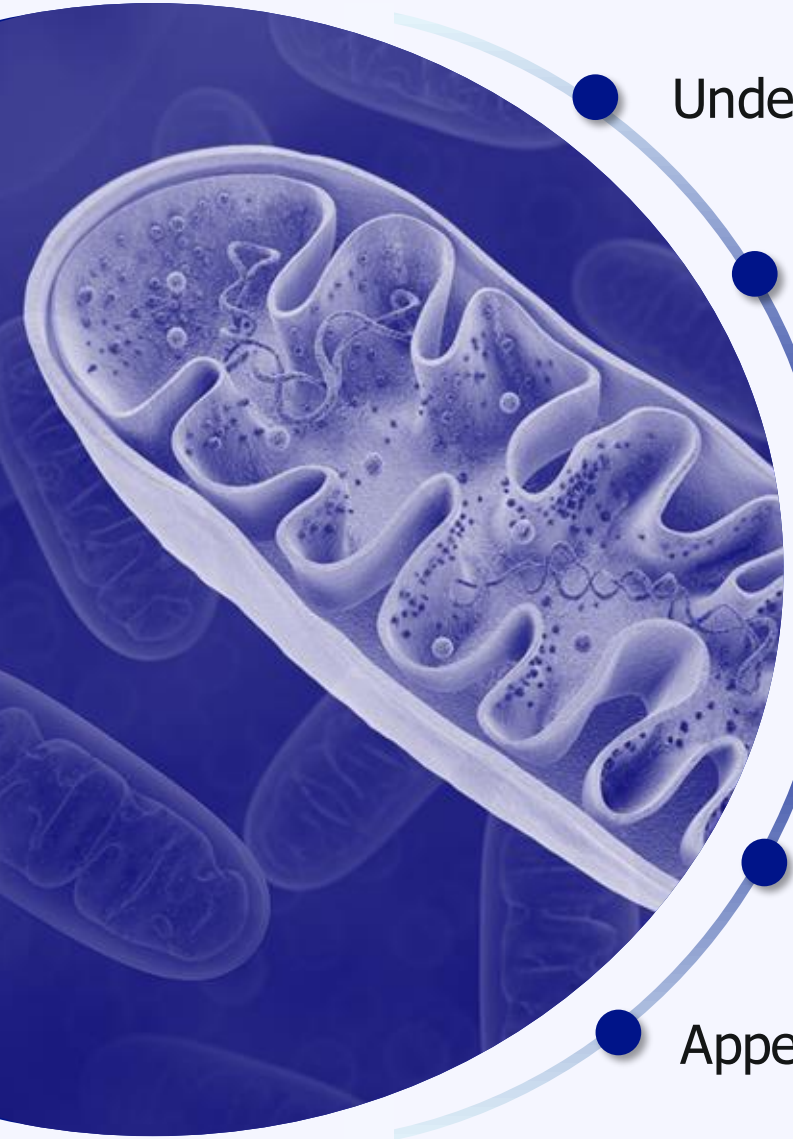
The following slides contain information about KYGEVVI.

KYGEVVI is a combination of doxecitine and doxribtimine, both pyrimidine nucleosides, indicated for the treatment of thymidine kinase 2 deficiency (TK2d) in children and adults with an age of symptom onset on or before 12 years.

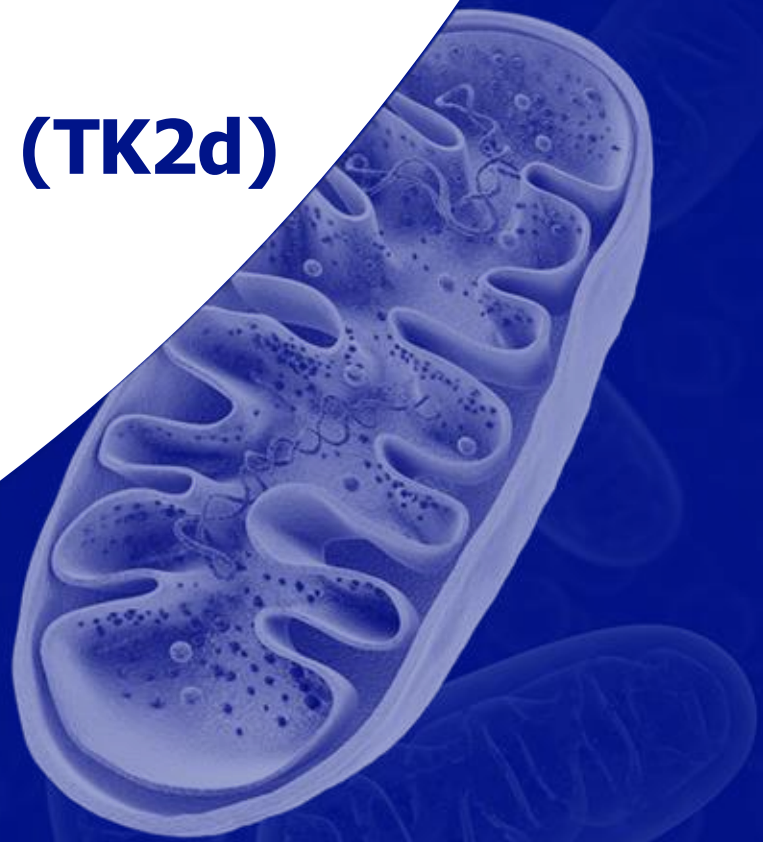
This deck is intended for reactive use by medical only.

Inspired by **patients.**
Driven by **science.**

Contents



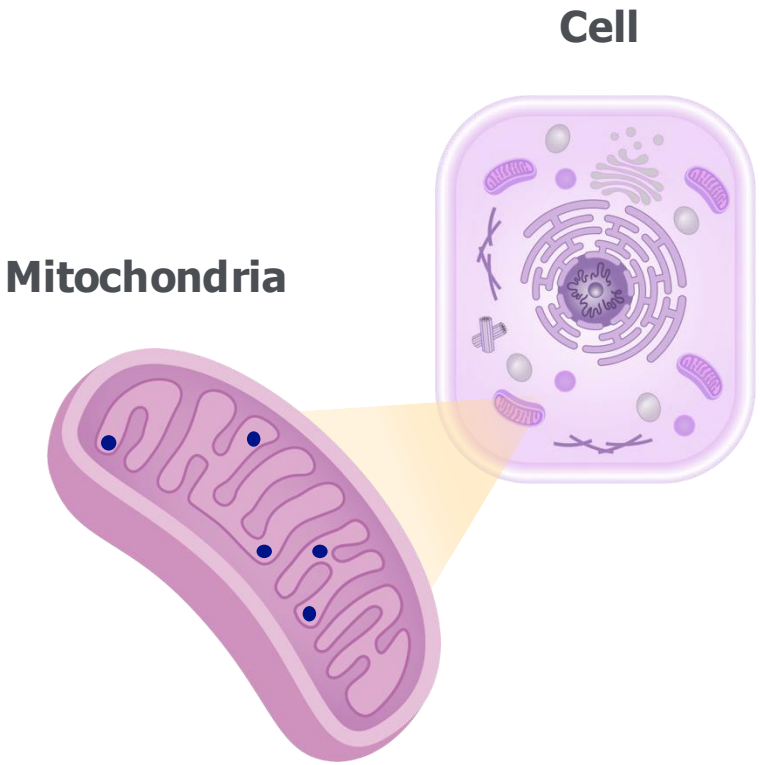
Understanding thymidine kinase 2 deficiency (TK2d)



Mitochondria are the parts of our cells responsible for producing energy

Mitochondria use the food we eat and the oxygen we breathe to **create energy** for our cells. They are **like power plants or batteries for our cells**[1]

The energy they make is needed for **important jobs** in the body, like keeping your **heart beating**[1]



1. UMDF. Mitochondrial Disease A Guide for the Newly Diagnosed. https://umdf.org/wp-content/uploads/2022/09/New-Patient-Guide-for-Mitochondrial-Disease-2022_MasterDoc.pdf. Accessed November 2025.

Mitochondrial DNA is important for mitochondria to function

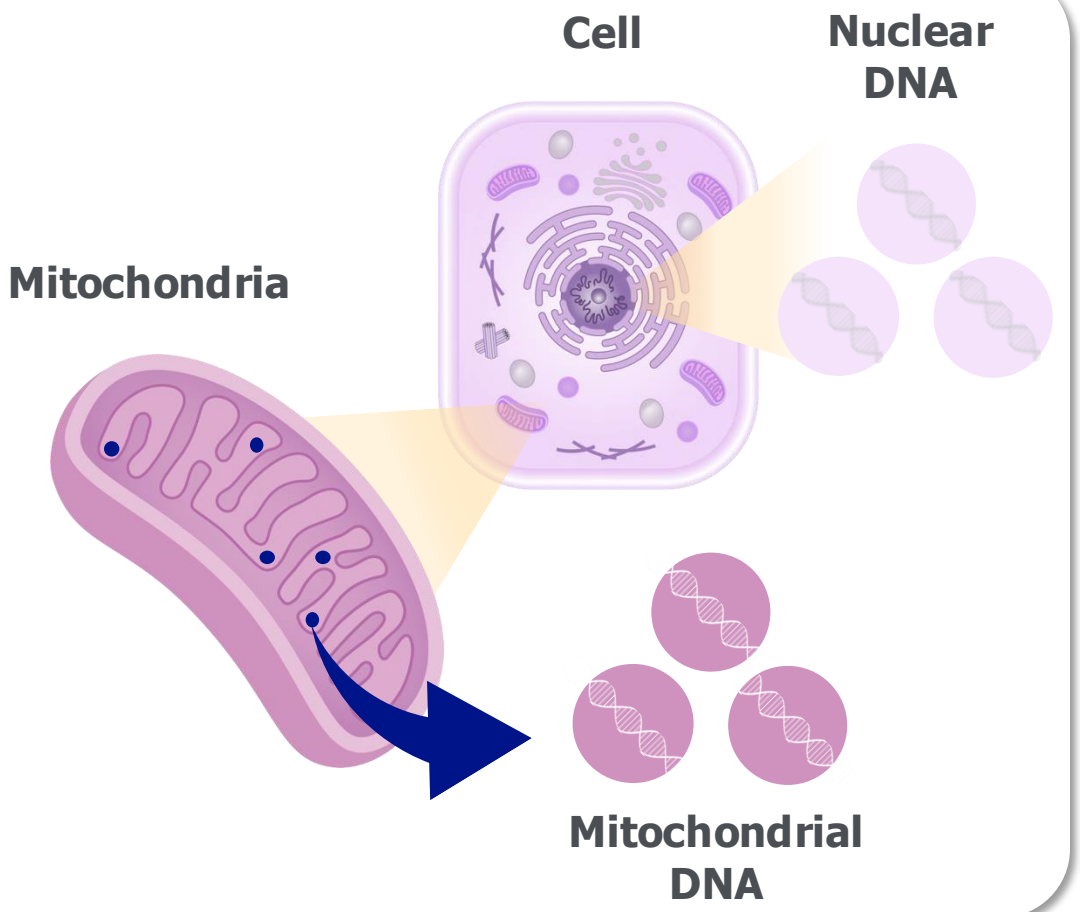
DNA is the instruction manual that tells our bodies how to **grow, develop, and function**[1]

- For example, DNA determines traits like eye color and how the lungs work[2]

Most DNA is stored inside the cellular control center (the nucleus). This DNA is called nuclear DNA. But did you know some DNA is stored in the mitochondria, known as **mitochondrial DNA** (mtDNA)?[3]

- mtDNA plays an **important role in creating energy**[4]

Mistakes (mutations) in either type of DNA can prevent mitochondria from making energy. This may **cause mitochondrial disease**[5]



DNA, deoxyribonucleic acid; mtDNA, mitochondrial DNA.

1. Cleveland Clinic. DNA. https://my.clevelandclinic.org/health/body/dna. Accessed November 2025; 2.Kids Britannica. DNA. https://kids.britannica.com/kids/article/DNA/390730. Accessed November 2025; 3. Russel OM, et al. Cell. 2020;181(1):168-88; 4. Lv J, Bhatia M, Wang X. Roles of mitochondrial DNA in energy metabolism. In: Sun H, Wang X, eds. Mitochondrial DNA and Diseases. Singapore: Springer; 2017;53-72. 5. Chinnery PF, et al. In: Adam MP, et al. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2024.

Mitochondrial diseases are rare, complex conditions with variable symptoms[1]

- An estimated **1 in 5,000 people** have a mitochondrial disease[2]
- When mitochondria don't work properly, **cells do not get enough energy.** This can make it hard for cells to do their job or to survive. **Organs that need a lot of energy**—like the brain, muscles and nerves—**are affected first**, which can cause many different symptoms[3]

Red-flag symptoms of mitochondrial diseases:[3-6]

- Reduced energy/fatigue
- Muscle weakness
- Difficulty swallowing or walking
- Neurological symptoms (e.g., epilepsy, poor coordination, hearing loss)

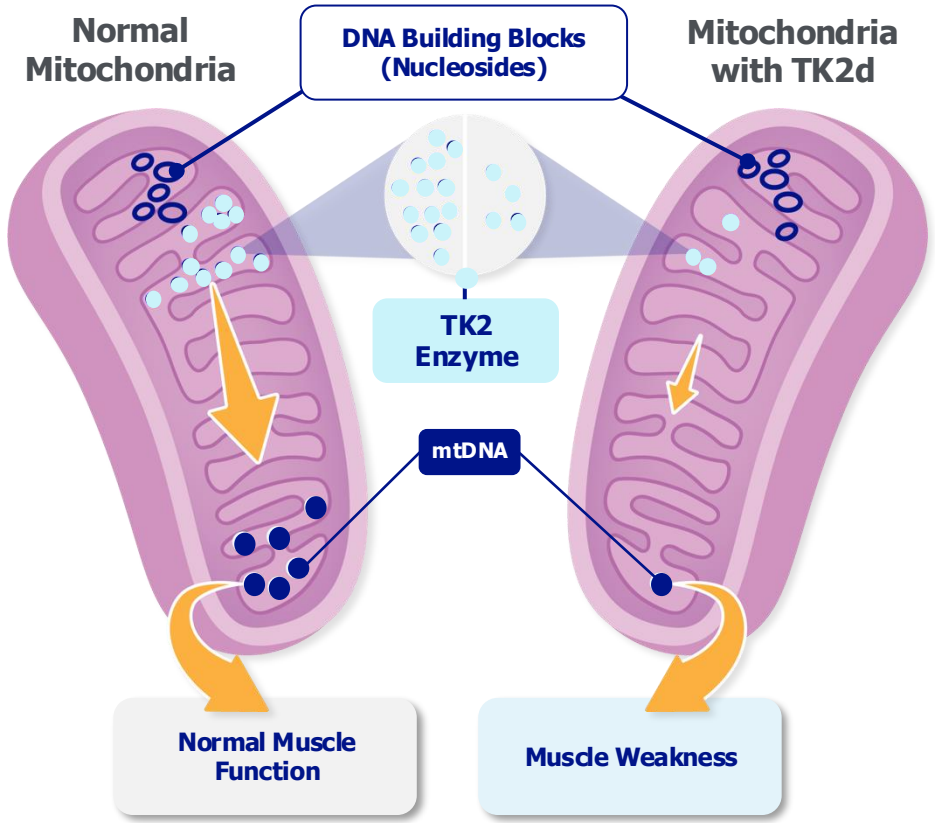
In infants:

- Low muscle tone (hypotonia)
- Weakness
- Failure to thrive
- Build up of acid in the body (metabolic acidosis)

1. Gorman GS, et al. Nat Rev Dis Primers. 2016;2:16080; 2. Gorman S, et al. ANN NEUROL. 2015;77(5):753-759; 3. UMDF. Mitochondrial Disease A Guide for the Newly Diagnosed. https://umdf.org/wp-content/uploads/2022/09/New-Patient-Guide-for-Mitochondrial-Disease-2022_MasterDoc.pdf. Accessed November 2025; 4. Thymidine kinase 2 deficiency. National Organization for Rare Disorders. https://rarediseases.org/rare-diseases/thymidine-kinase-2-deficiency/. Accessed November 2025; 5. Parikh S, et al. Genet Med. 2017;19(12):1-18; 6. Murarescu CC, et al. Curr Genet Med Rep. 2018;6(2):62-72.

Thymidine kinase 2 deficiency is a rare genetic mitochondrial disease caused by mutations in the *TK2* gene[1]

- Thymidine kinase 2 deficiency (TK2d) is a type of mitochondrial disease classified within the group of **mitochondrial DNA depletion and deletion syndromes** (MDDS)[1]
- Mutations in the ***TK2*** **nuclear gene** stop an enzyme called TK2 from helping to **make and repair your mitochondrial DNA (mtDNA)[1-3]**
- Without healthy mtDNA, mitochondria cannot work the way they should. This means muscles don't have enough energy to function properly, **causing many muscle groups to become weak[1-4]**



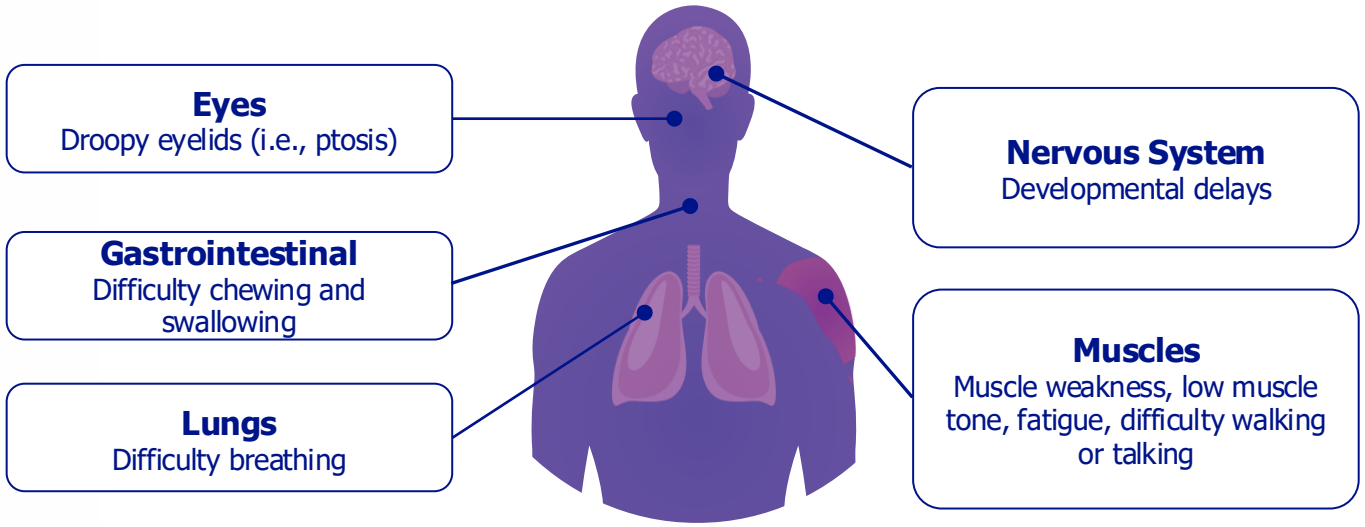
TK2(d), thymidine kinase 2 (deficiency).

1. Berardo A, et al. J Neuromuscul Dis. 2022;9(2):225-35; 2. Garone C, et al. J Med Genet. 2018;55(8):515-21 3. Lopez-Gomez C, et al. Ann Neurol. 2017;81(5):641-52; 3. Thymidine kinase 2 deficiency. National Organization for Rare Disorders. Updated September 12, 2022. https://rarediseases.org/rare-diseases/thymidine-kinase-2-deficiency/. Accessed November 2025.

TK2d can present in different ways and affect different parts of the body

- TK2d can cause a variety of symptoms, and it affects each person differently[1]
- Most people with TK2d have muscle weakness. This can make it harder to move and can cause breathing problems[1,2]
- Over time, many people may lose the ability to walk, eat, and breathe independently[2,3]

TK2d Symptoms[1-4]



TK2d, thymidine kinase 2 deficiency.

1. Berardo A, et al. J Neuromuscul Dis. 2022;9(2):225-35; 2. Garone C, et al. J Med Genet. 2018;55(8):515-21; 3. Wang J, et al. In: Adam MP, et al, eds. GeneReviews® [Internet]. University of Washington, Seattle; December 6, 2012. Updated July 26, 2018. Accessed November 2025; 4. UMDF. Mitochondrial Disease A Guide for the Newly Diagnosed. https://umdf.org/wp-content/uploads/2022/09/New-Patient-Guide-for-Mitochondrial-Disease-2022_MasterDoc.pdf. Accessed November 2025

TK2d care requires many types of healthcare providers because it affects different parts of the body

A healthcare team works together to **manage symptoms and optimize the quality of life** for people living with TK2d and may include:[1-4]

1. **Pulmonologists and respiratory therapists**
   Assist with breathing difficulties.
2. **Cardiologists**
   Assess and manage heart muscle weakness.
3. **Gastroenterologists and nutritionists**
   Manage feeding difficulties, assist in dietary guidance and address special food needs.
4. **Orthopedic specialists**
   Help with muscle weakness, bone abnormalities or joint problems.
5. **Physical therapists**
   Focus on adjusting and adapting movement, strength, and coordination.
6. **Occupational therapists**
   Focus on improving the ability to perform activities of daily living.
7. **Metabolic specialists**
   Monitor and manage energy levels.
8. **Speech therapists**
   Help with speaking difficulties and strengthening the facial muscles required to chew.
9. **Neurologists**
   Help with challenges related to movement.
10. **Clinical geneticists**
    Provide more information into the cause of health challenges.
11. **Pediatricians or primary care physicians**
    Provide regular health and wellness checkups and diagnose and treat a wide range of general health conditions.

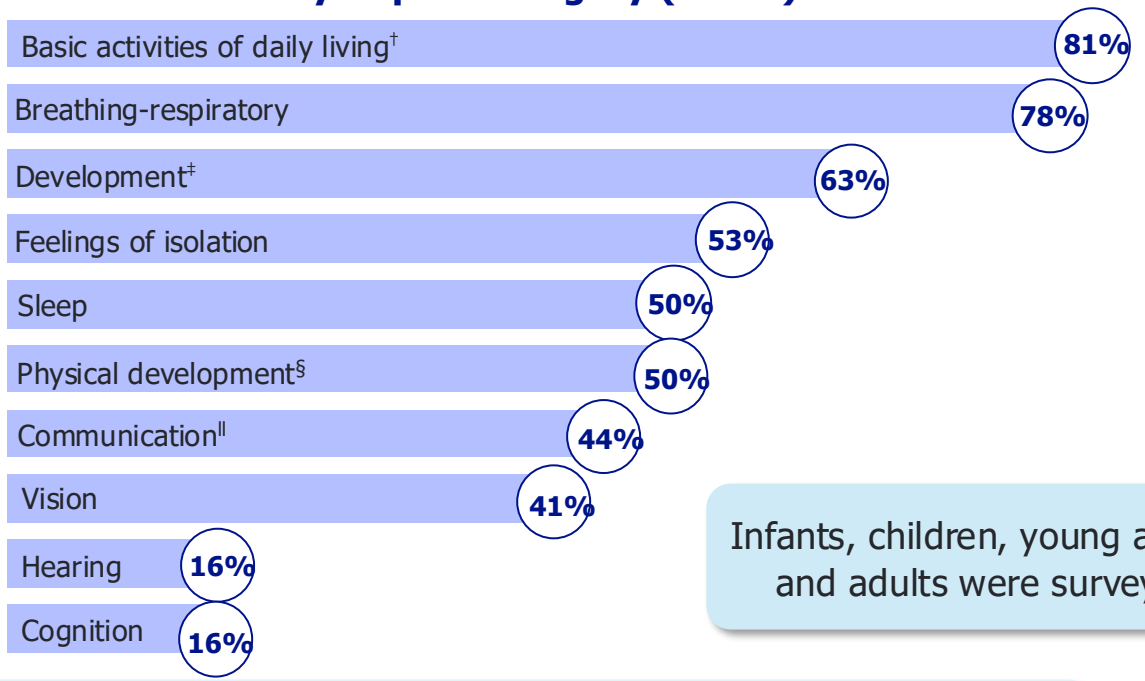
TK2d, thymidine kinase 2 deficiency.

1.. Wang J, et al. In: Adam M, et al. eds. GeneReviews® [Internet]. University of Washington, Seattle; December 6, 2012. Updated July 26, 2018. Accessed November 2025; 2. Amtmann D, et al. Mitochondrion. 2022;68:1-9; 3. Parikh S, et al. Genet Med. 2017;19(12):10.1038/gim.2017.107; 4. El-Hattab AW and Scaglia F. Neurotherapeutics. 2013;10:186-98.

TK2d affects many parts of physical and mental health

- TK2d causes substantial **physical impact** and **psychological strain,** which can affect daily life and overall well-being[1]
- These challenges affect people with TK2d of **all ages**, **regardless** of **when** their **symptoms begin**[1]

Percentage of people with TK2d experiencing impact by impact category (N=32)*



Infants, children, young adults, and adults were surveyed

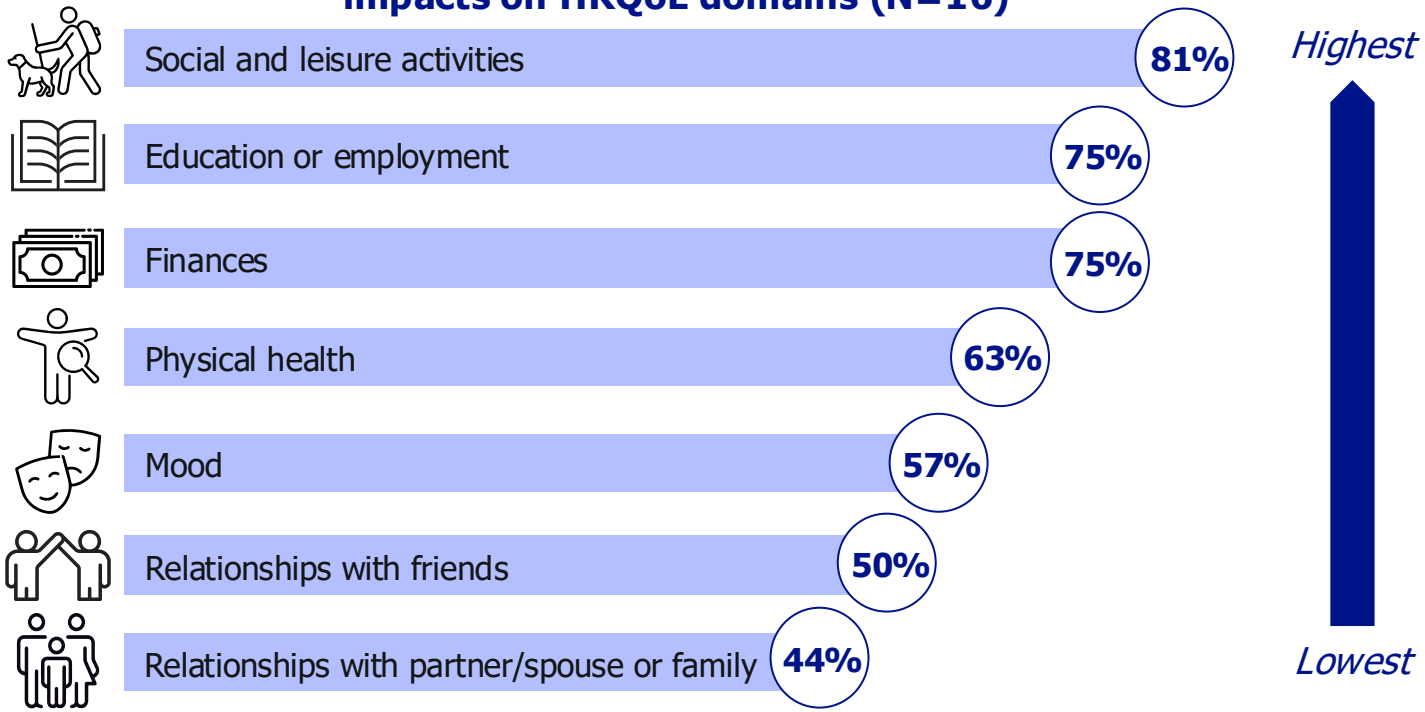
> *It is difficult to see my physical strength diminish and feel like I am losing independence. There are normal day-to-day activities that require extra planning and it's overwhelming.*
>
> **–Young adult, with age of TK2d symptom onset >2 to ≤12 years (self-report)**

*Impacts are a grouping of World Health Organization International Classification of Functioning, Disability and Health components – impairment, activity, and participation.
†Basic activities of daily living include difficulties walking and eating/swallowing, issues with control of urine and bowel movements. ‡Development includes developmental delays and loss of normal milestones and/or functions. §Physical development includes failure to thrive, losing weight or unable to maintain weight, and malnutrition. ‖Communication includes communication challenges, problems with non-verbal communication (e.g., nodding) and speech problems.
TK2d, thymidine kinase 2 deficiency.
1. Yeske P, et al. 2025. Muscular Dystrophy Association (MDA) Clinical & Scientific Conference; Dallas, TX, USA; March 16–19, 2025. P252.

Caregiving for people with TK2d imposes large emotional, financial, and physical burdens[1]

- Caregiving takes a **large emotional**, **financial**, **and physical toll**, affecting nearly every part of caregivers' lives[1]
- The strain is **greatest** when people with TK2d rely on **medical equipment** to help with breathing, eating, moving, and daily activities[1]
  - **87.5%** of caregivers (14/16) reported needing medical equipment in their homes[1]

Percentage of caregivers reporting negative impacts on HRQoL domains (N=16)*



> Sharing our struggles, seeking help, and leaning on others are not signs of weakness, but displays of strength and courage. Allowing ourselves to be vulnerable fosters deeper connections.
>
> **– Mother of young adult, with age of TK2d symptom onset after 2 years**

*Caregivers reporting positive impact in each HRQoL domain were as follows: physical health, 25.0% (n=4); social and leisure activities, 13% (n=2); finances, 6% (n=1); education or employment, 13% (n=2); mood, 38% (n=6); relationships with friends, 31% (n=5); relationships with partner/spouse or family, 31% (n=5).

TK2d, thymidine kinase 2 deficiency; HRQoL, Health related quality of life.

1. Yeske P, et al. 2025 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference; Dallas, TX, USA; March 16–19, 2025. P253

The diagnosis of TK2d is confirmed by genetic testing

- Genetic testing is the **only way** to confirm a TK2d diagnosis[1]
  - If you **suspect TK2d**, or if you or your child have received another diagnosis but haven't responded to treatment or disease management, **ask your doctor about genetic testing** - even if you've had it before

Types of genetic tests

Whole-exome sequencing/ whole-genome sequencing	Multi-gene panels that include *TK2*	Single-gene testing for *TK2*

TK2d, thymidine kinase 2 deficiency.

1. Wang J, et al. In: Adam M, et al. eds. GeneReviews® [Internet]. University of Washington, Seattle; December 6, 2012. Updated July 26, 2018. Accessed November 2025

People with TK2d often use supportive medical devices

Although TK2d can be serious and sometimes life-threatening, once it is diagnosed, healthcare providers have several ways to manage symptoms and reduce its impact[1]

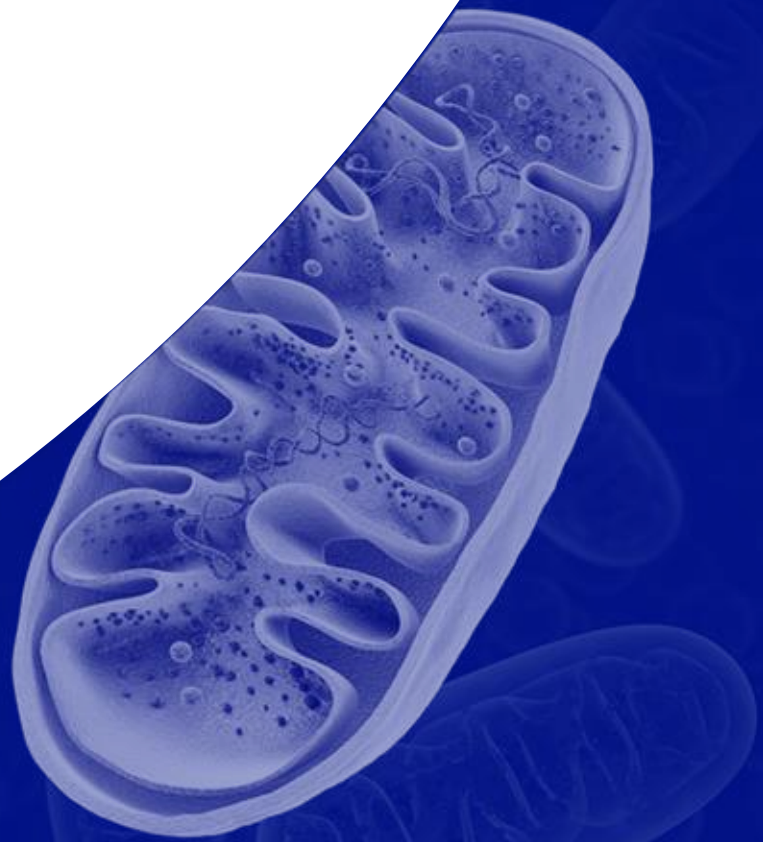
Doctors may recommend different tools and devices to make daily living easier, such as:[1-3]

- Breathing machines or masks
- Wheelchairs or walkers
- Feeding tubes
- Special utensils to help with eating
- Leg supports (braces)
- Back braces
- Glasses or other vision aids
- Communication devices (to help with speaking)

TK2d, thymidine kinase 2 deficiency.

1. Wang J, Kim E, Ting TW, et al. TK2-Related Mitochondrial DNA Maintenance Defect. GeneReviews. 2018; 2. Parikh S, et al. Genet Med. 2017;19(12):10.1038/gim.2017.107; 3. El-Hattab AW and Scaglia F. Mitochondrial DNA depletion syndromes: review and updates of genetic basis, manifestations, and therapeutic options. Neurotherapeutics. 2013;10:186-98.

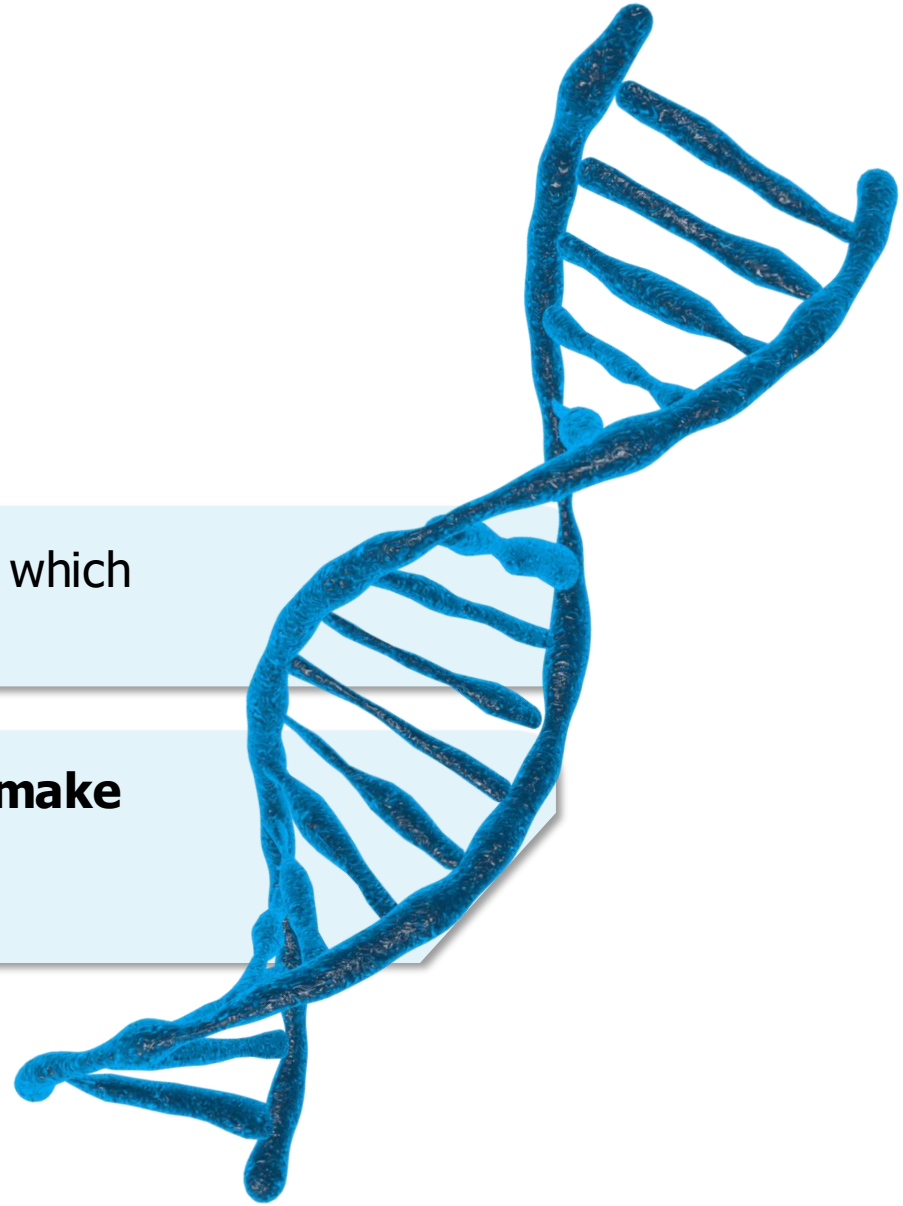
What is KYGEVVI, and how does it work?



What are pyrimidine nucleosides?

KYGEVVI is a combination of **doxecitine and doxribtimine,** which are **pyrimidine nucleosides**[1]

Pyrimidine nucleosides are small **building blocks that help make DNA.** They're **important for mtDNA** to replicate and function properly[2]



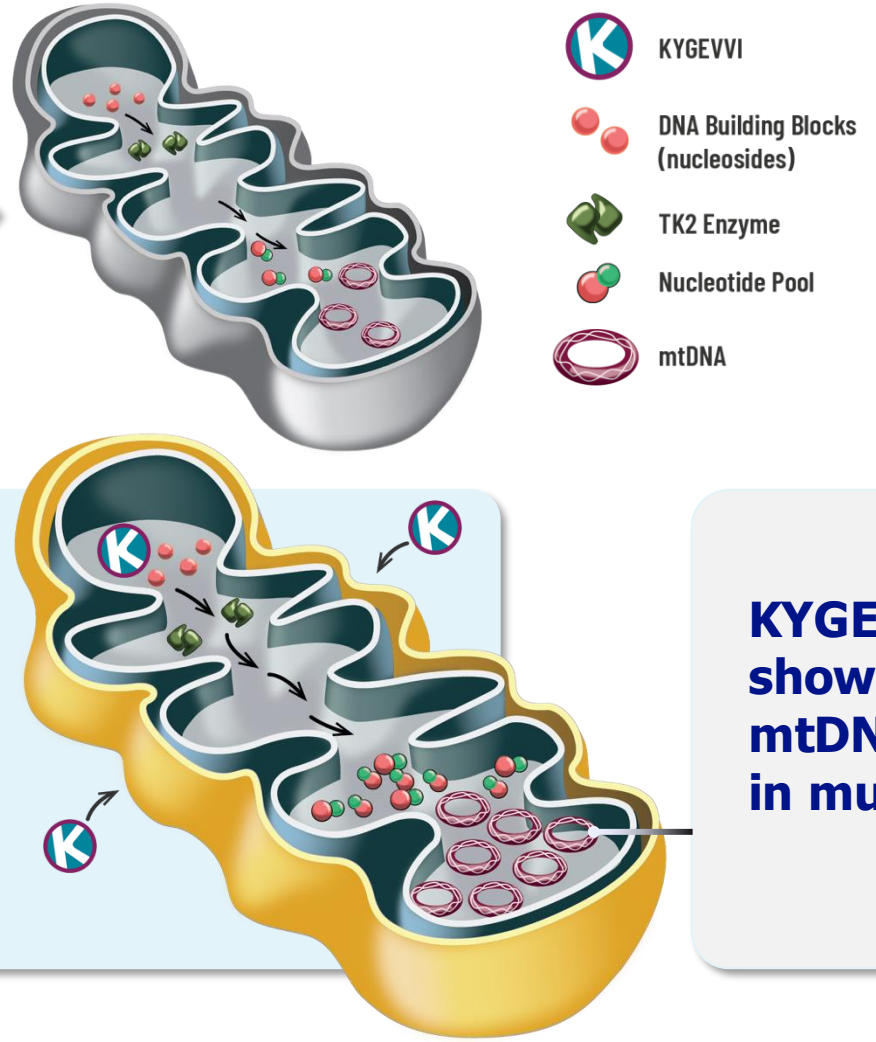
KYGEVVI is a combination of doxecitine and doxribtimine, both pyrimidine nucleosides, indicated for the treatment of thymidine kinase 2 deficiency (TK2d) in children and adults with an age of symptom onset on or before 12 years[1]

DNA, deoxyribonucleic acid; mtDNA, mitochondrial DNA.

1. KYGEVVI™ (doxecitine and doxribtimine) U.S. Prescribing Information. Smyrna, GA: UCB, Inc. Accessed November 2025. 2. Wang L, et al. Nucleos Nucleot Nucl. 2016;35(10-12):578-594.

Intended mechanism of action

People with TK2d have less mtDNA (i.e., fewer copies)[1]



Administration of KYGEVVI is intended to incorporate the pyrimidine nucleosides into the mitochondria[2]

KYGEVVI has been shown to restore the mtDNA copy number in mutant mice[2]

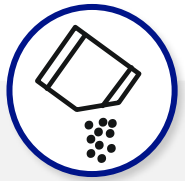
KYGEVVI is a combination of doxecitine and doxribtimine, both pyrimidine nucleosides, indicated for the treatment of thymidine kinase 2 deficiency (TK2d) in children and adults with an age of symptom onset on or before 12 years[2]

mtDNA, mitochondrial DNA; TK2(d), thymidine kinase 2 (deficiency).

1. Berardo A, et al. J Neuromuscul Dis. 2022;9(2):225-35 2. KYGEVVI™ (doxecitine and doxribtimine) U.S. Prescribing Information. Smyrna, GA: UCB, Inc. Accessed November 2025.

Who can take KYGEVVI?

KYGEVVI is a combination of doxecitine and doxribtimine, both pyrimidine nucleosides, indicated for the treatment of **thymidine kinase 2 deficiency (TK2d)** in **children and adults** with an **age of symptom onset on or before 12 years**[1]



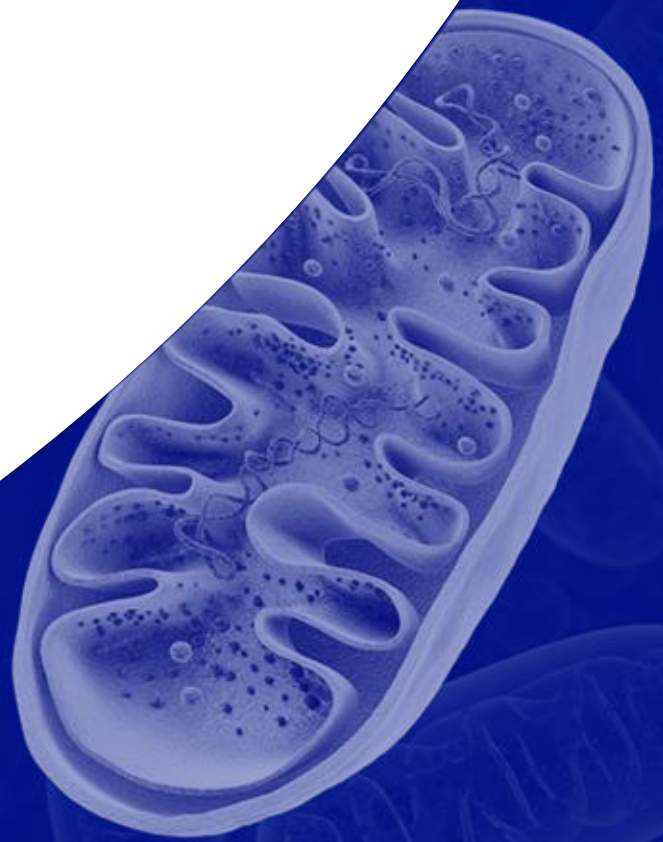
Powder for oral solution:
2 g doxecitine and 2 g doxribtimine

KYGEVVI should be **administered by an adult** in 3 equally divided doses **with food**, approximately 6 hours apart (±2 hours)

TK2d, thymidine kinase 2 deficiency.

1. KYGEVVI™ (doxecitine and doxribtimine) US Prescribing Information. UCB, Inc. Accessed November 2025.

Clinical trial design, results, and safety



Why was this study done?

To learn more about TK2d

To see how **KYGEVVI** treatment affects people with TK2d

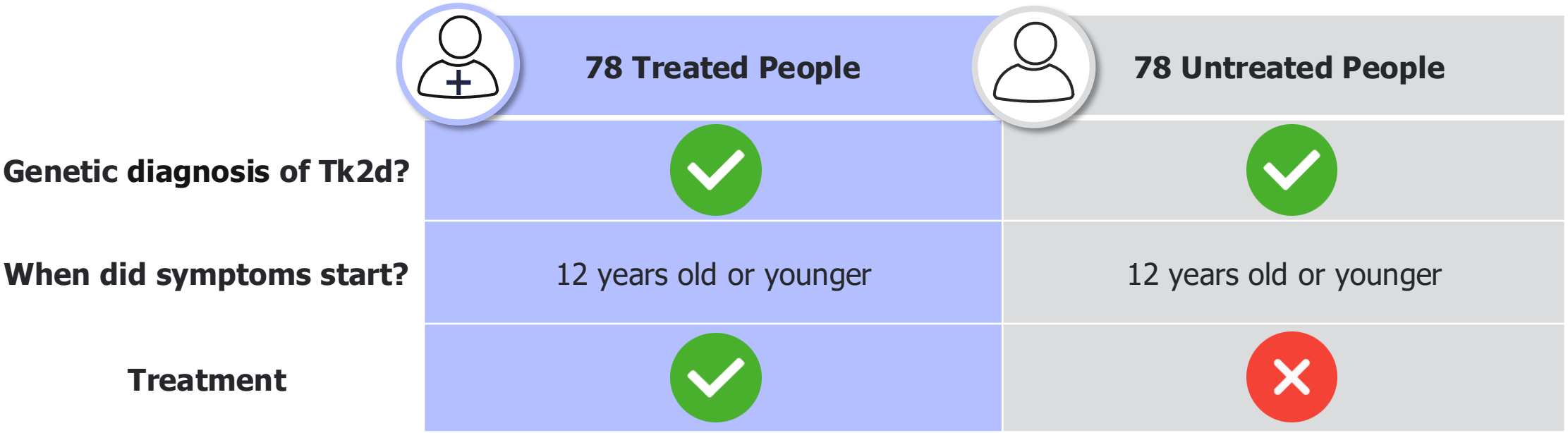
To improve care for people with TK2d

KYGEVVI is a combination of doxecitine and doxribtimine, both pyrimidine nucleosides, indicated for the treatment of thymidine kinase 2 deficiency (TK2d) in children and adults with an age of symptom onset on or before 12 years[1]

1. KYGEVVI™ (doxecitine and doxribtimine) U.S. Prescribing Information. Smyrna, GA: UCB, Inc. Accessed November 2025.

Who took part in this study?

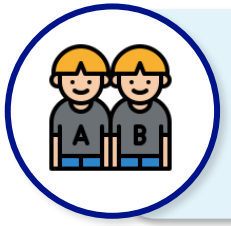
People **treated** with KYGEVVI were **matched to people from an untreated control group**



KYGEVVI is a combination of doxecitine and doxribtimine, both pyrimidine nucleosides, indicated for the treatment of thymidine kinase 2 deficiency (TK2d) in children and adults with an age of symptom onset on or before 12 years[1]

1. KYGEVVI™ (doxecitine and doxribtimine) U.S. Prescribing Information. Smyrna, GA: UCB, Inc. Accessed November 2025.

How was this study done?



Each treated person was matched to another untreated person who had symptoms that began around the same age (either on or before age 2, or between 2 to ≤12 years old)

Health record review

Open-label clinical trial

Expanded access program

Treated People (n=78)

Median duration of treatment was **4 years** (range: 1 day to 12 years)

Health record review
+
Medical literature review

Untreated People (n=78)

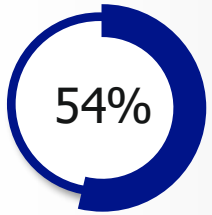
Research Question: How long did people with TK2d in each group survive?

KYGEVVI is a combination of doxecitine and doxribtimine, both pyrimidine nucleosides, indicated for the treatment of thymidine kinase 2 deficiency (TK2d) in children and adults with an age of symptom onset on or before 12 years[1]

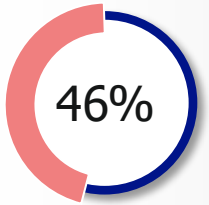
1. KYGEVVI™ (doxecitine and doxribtimine) U.S. Prescribing Information. Smyrna, GA: UCB, Inc. Accessed November 2025.

Meet the 78 treated people

Gender



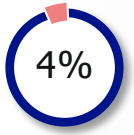
Male



Female

Race

White



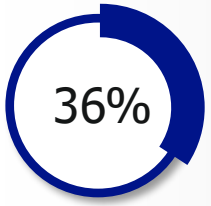
Black or African American

Asian

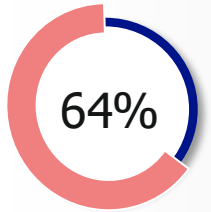
Other

American Indian or Alaska Native

Ethnicity



Hispanic or Latino



Other

The median age of TK2d symptom onset was **1.5 years** (Range: 0.01 - 12 years)

Median duration of treatment was **4 years** (Range: 1 day - 12 years)

KYGEVVI is a combination of doxecitine and doxribtimine, both pyrimidine nucleosides, indicated for the treatment of thymidine kinase 2 deficiency (TK2d) in children and adults with an age of symptom onset on or before 12 years[1]

TK2d, thymidine kinase 2 deficiency.

1. KYGEVVI™ (doxecitine and doxribtimine) U.S. Prescribing Information. Smyrna, GA: UCB, Inc. Accessed November 2025.

KYGEVVI was generally well-tolerated

The most common side effects with KYGEVVI include:*†

- Diarrhea
- Stomach (abdominal) pain including pain in the upper stomach area
- Vomiting
- Increase in liver enzymes

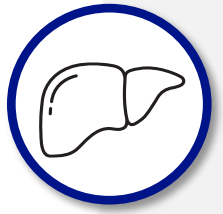
Call your doctor for medical advice about side effects. You may report side effects to UCBCares® at 1-844-599-CARE (2273)

KYGEVVI is a combination of doxecitine and doxribtimine, both pyrimidine nucleosides, indicated for the treatment of thymidine kinase 2 deficiency (TK2d) in children and adults with an age of symptom onset on or before 12 years[1]

*Open-label phase 2 clinical study, NCT03845712, TK0102. †Adverse reactions that occurred in ≥5% adult and pediatric patients with TK2d treated with KYGEVVI or pyrimidine nucleosides.

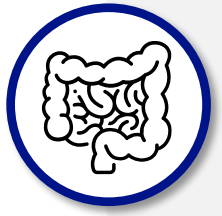
1. KYGEVVI™ (doxecitine and doxribtimine) US Prescribing Information. Smyrna, GA: UCB, Inc. Accessed November 2025.

Liver and stomach side effects were seen in some people during treatment



Elevated Liver Enzyme Levels

- Your healthcare provider will do **blood tests to check your liver enzyme levels before starting treatment and during treatment**
- Your healthcare provider **may temporarily or permanently stop your treatment if you develop liver problems**
- **Call your healthcare provider right away** if you develop any signs or symptoms of liver problems, including:
  - Loss of appetite
  - Pain on the right side of your stomach area
  - Dark, amber-colored urine
  - Yellowing of your skin or the white part of your eyes
  - Nausea and vomiting
  - Itching



Stomach and Intestinal Problems

- Contact your healthcare provider promptly if you experience **diarrhea and/or vomiting that lasts longer than a few days** while taking KYGEVVI

KYGEVVI is a combination of doxecitine and doxribtimine, both pyrimidine nucleosides, indicated for the treatment of thymidine kinase 2 deficiency (TK2d) in children and adults with an age of symptom onset on or before 12 years[1]

1. KYGEVVI™ (doxecitine and doxribtimine) US Prescribing Information. Smyrna, GA: UCB, Inc. Accessed November 2025.

KYGEVVI helped people with TK2d live longer

KYGEVVI **improved survival** from the time of treatment start

Treated people (n=78)

96% **of treated people** were alive at the end of the study

Untreated people (n=78)

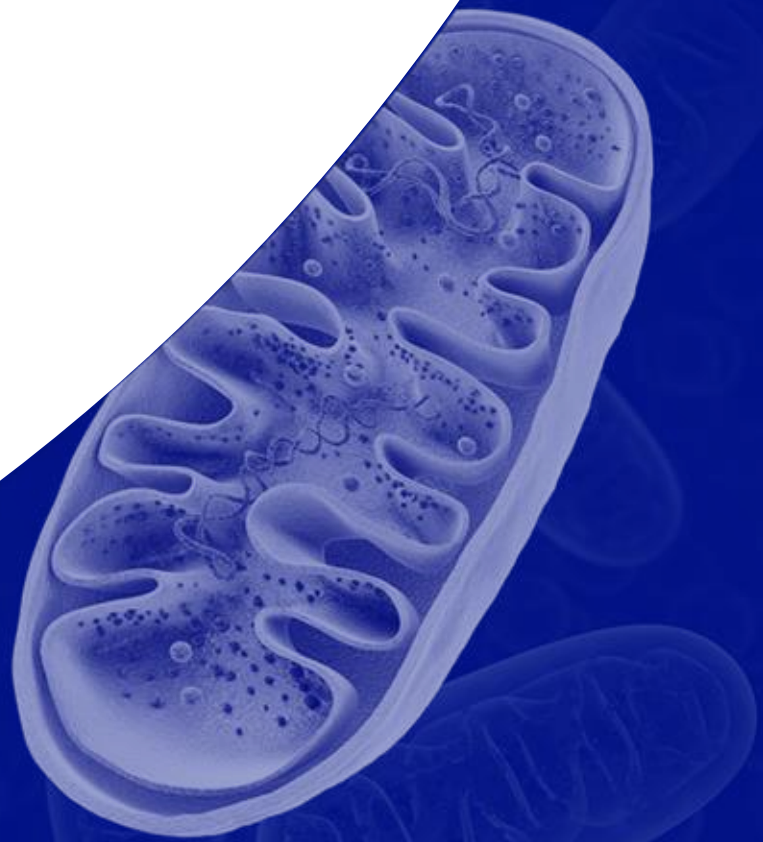
64% **of untreated people** were alive at the end of the study

KYGEVVI is a combination of doxecitine and doxribtimine, both pyrimidine nucleosides, indicated for the treatment of thymidine kinase 2 deficiency (TK2d) in children and adults with an age of symptom onset on or before 12 years[1]

TK2d, thymidine kinase 2 deficiency.

1. KYGEVVI™ (doxecitine and doxribtimine) US Prescribing Information. Smyrna, GA: UCB, Inc. Accessed November 2025.

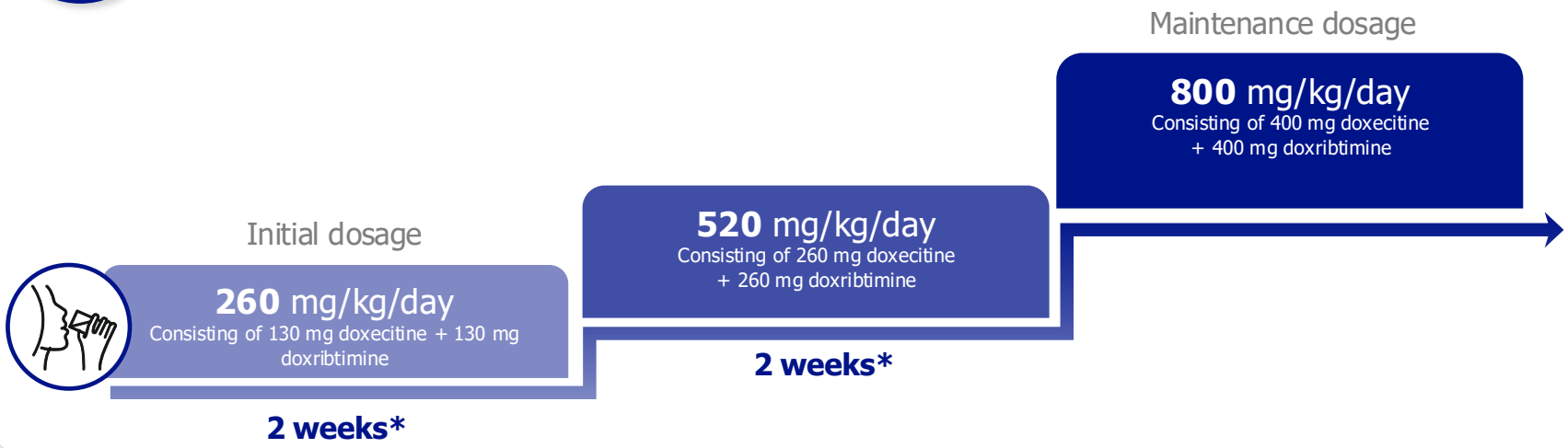
More information for people considering treatment with KYGEVVI



KYGEVVI is an oral solution taken three times a day in equally divided doses

Recommended Dosage

- The recommended dosage of KYGEVVI is based on weight.
- Dosage will be slowly increased depending on how it is tolerated.



KYGEVVI oral solution should be taken **with food 3 times a day** in equally divided doses approximately 6 hours apart (±2 hours)

If a dose is missed, take the missed dose as soon as possible but **do not take within 2 hours of the next scheduled dose**. In that case, skip the missed dose and resume the regular schedule. **A double dose should not be taken to make up for the missed dose**

KYGEVVI is a combination of doxecitine and doxribtimine, both pyrimidine nucleosides, indicated for the treatment of thymidine kinase 2 deficiency (TK2d) in children and adults with an age of symptom onset on or before 12 years[1]

*KYGEVVI is titrated based on tolerability. A minimum of 2 weeks at the current dosage is recommended before titrating to the next dosage.

1. KYGEVVI™ (doxecitine and doxribtimine): U.S. prescribing information. Smyrna, GA: UCB, Inc. Accessed November 4, 2025.

Inspired by patients. Driven by science.

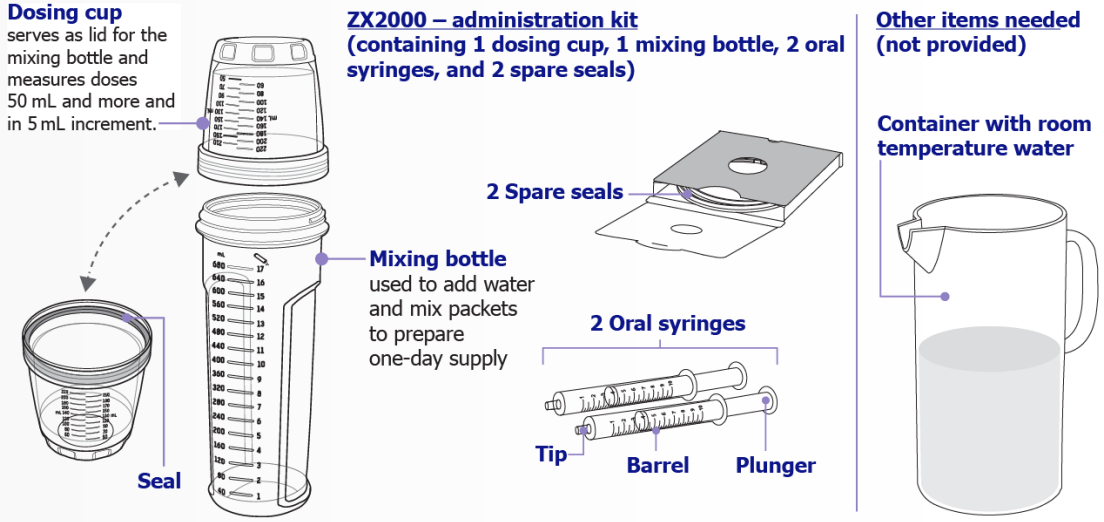
KYGEVVI preparation instructions

Preparation instructions

Only an adult should prepare a **one-day supply of oral solution** each morning **using the administration kit*** (mixing bottle, dosing cup, oral syringes and spare seals) provided.

Preparation of KYGEVVI with a liquid other than water has not been studied clinically and is not recommended.

1. Obtain the required number of KYGEVVI packets to prepare a one-day supply of solution each morning.
2. Use **40 mL of water per packet**. Pour the prescribed volume of room temperature water (between 20ºC - 25ºC or 68ºF - 77ºF) into the mixing bottle.
3. Add the powder from the required number of KYGEVVI packets into the mixing bottle.
4. Screw the dosing cup tightly onto the mixing bottle and gently invert the mixing bottle back and forth at least 20 times. If powder remains, repeat until the powder dissolves.
5. The mixed solution may appear cloudy and have some residual powder (inactive ingredients) remaining at the bottom or top.



KYGEVVI is a combination of doxecitine and doxribtimine, both pyrimidine nucleosides, indicated for the treatment of thymidine kinase 2 deficiency (TK2d) in children and adults with an age of symptom onset on or before 12 years[1]

*Household devices such as measuring cups or spoons are not adequate measuring devices.

1. KYGEVVI™ (doxecitine and doxribtimine): U.S. prescribing information. Smyrna, GA: UCB, Inc. Accessed November 4, 2025.

KYGEVVI administration instructions

Administration Instructions

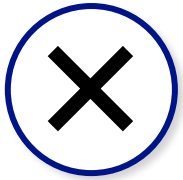
Oral Administration

1. Before each administration, **gently invert** the tightly closed mixing bottle slowly back and forth at least 3 times.
2. Use 1 of 2 methods (**dosing cup or oral syringe**) to administer KYGEVVI solution. Choose the method based on the volume of solution to be administered per dose.
3. Take KYGEVVI solution in **3 equally divided doses approximately 6 hours apart** (plus or minus 2 hours) with food.
4. **Do not administer** another dose if the dose is spit out or **if a complete dose is not taken**. Take the next dose at the next scheduled time.

Feeding Tube Administration

KYGEVVI is compatible with most commonly available feeding tubes. KYGEVVI is compatible with feeding tubes made with polyvinylchloride (PVC) free from DEHP (Phthalates), polyurethane (PUR), and silicone (SIL) material.

1. **Follow the instructions of the feeding tube manufacturer** to administer KYGEVVI.
2. **Draw up the KYGEVVI solution** using a syringe compatible with the feeding tube.
3. **Administer the solution immediately** through the feeding tube.
4. **Flush any residual solution** in the syringe or feeding tube until no solution is left. To flush the tube, a single flushing step with a volume of water equivalent to the tube's priming volume is sufficient.

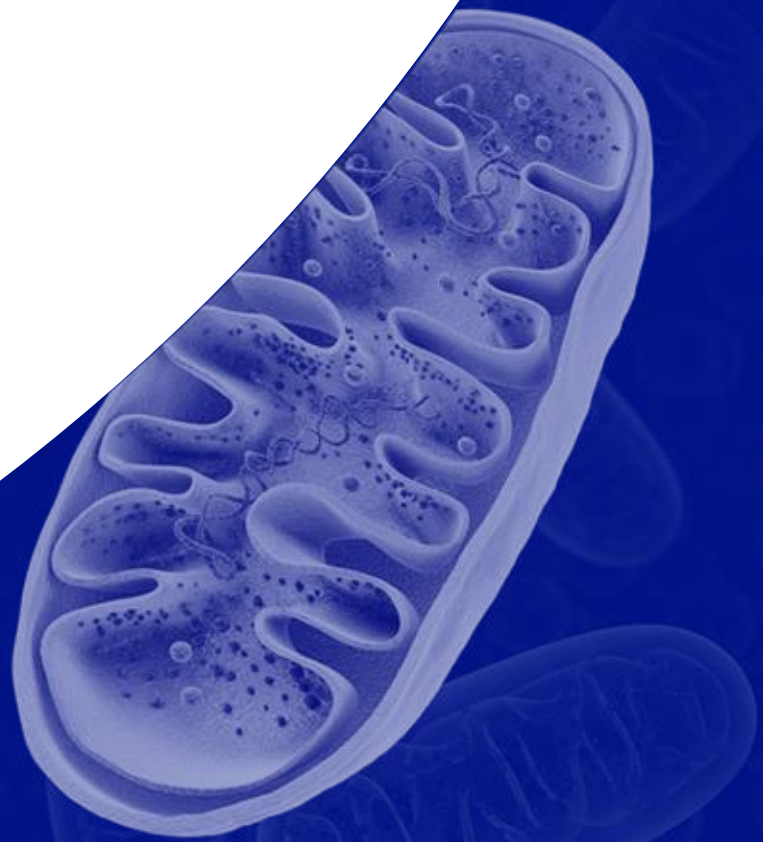


Discard any remaining KYGEVVI solution 16 hours after reconstitution or after taking or giving the 3 doses, whichever comes first.

KYGEVVI is a combination of doxecitine and doxribtimine, both pyrimidine nucleosides, indicated for the treatment of thymidine kinase 2 deficiency (TK2d) in children and adults with an age of symptom onset on or before 12 years[1]

1. KYGEVVI™ (doxecitine and doxribtimine): U.S. prescribing information. Smyrna, GA: UCB, Inc. Accessed November 4, 2025.

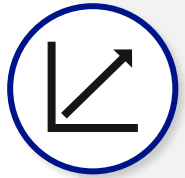
Summary



Summary of KYGEVVI

TK2d is a **rare genetic mitochondrial disease,** which results in muscle weakness[1-3]

Most common side effects of KYGEVVI are **diarrhea, stomach (abdominal) pain, vomiting,** and **increased liver enzymes**[4]



KYGEVVI **improved survival** from treatment initiation in children and adults with TK2d symptom onset on or before 12 years[4]

KYGEVVI is taken **orally 3 times a day in equally divided doses**[4]

KYGEVVI is a combination of doxecitine and doxribtimine, both pyrimidine nucleosides, indicated for the treatment of thymidine kinase 2 deficiency (TK2d) in children and adults with an age of symptom onset on or before 12 years[1]

1. Garone C, et al. J Med Genet. 2018;55(8):515-21; 2 . Grier J, et al. Neurol Genet. 2018;4(2):e230; 3. Amtmann D, et al. Mitochondrion. 2022;68:1-9; 4. KYGEVVI™ (doxecitine and doxribtimine) U.S. Prescribing Information. Smyrna, GA: UCB, Inc. Accessed November 2025.

Thank you for joining us today.

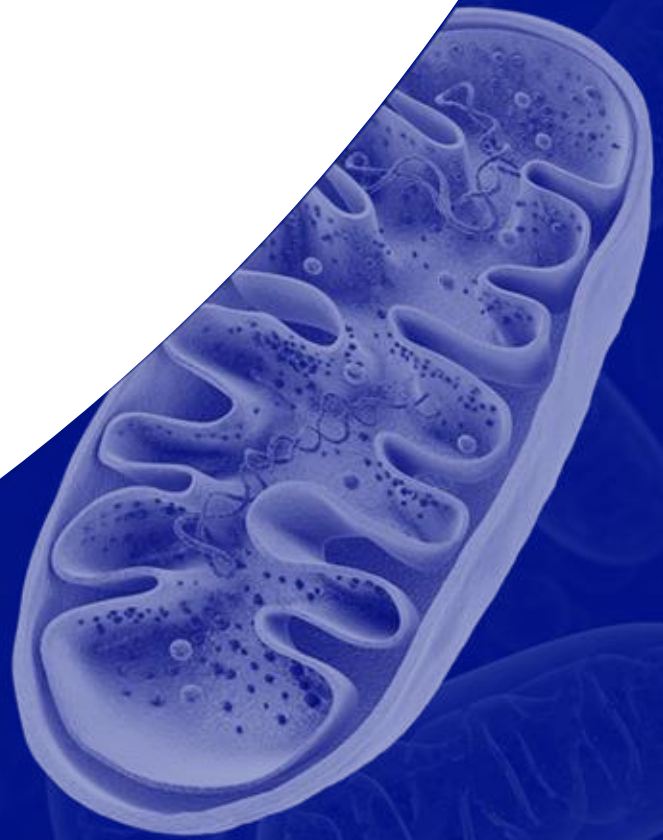
If you have questions or would like more information, please contact:

UCBCares®
About People

Hours of Operation
Monday-Thursday: 8 am-8 pm ET
Friday: 8 am-5 pm ET
Phone Number: 1-844-599-CARE (2273)
Email Address: UCBCares@ucb.com

Inspired by patients.
Driven by science.

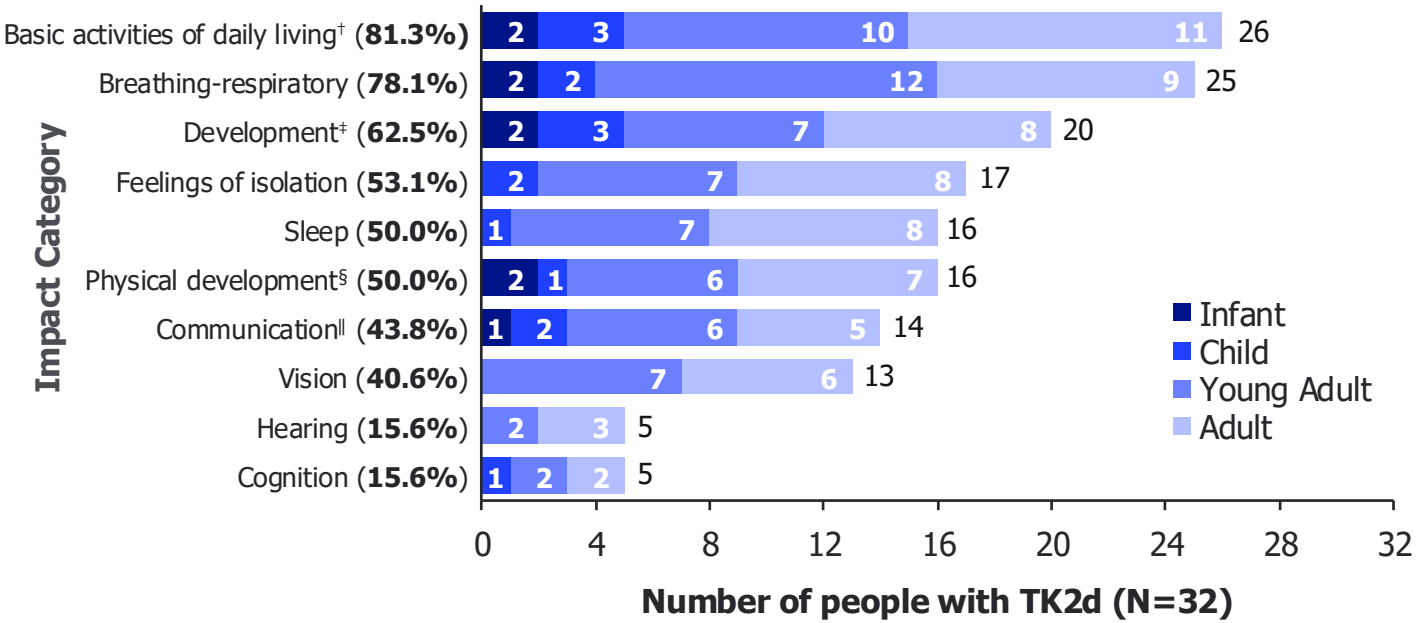
Appendix



TK2d affects many parts of physical and mental health

- TK2d causes substantial **physical impact** and **psychological strain,** which can affect daily life and overall well-being[1]
- These challenges affect people with TK2d of **all ages**, **regardless** of **when** their **symptoms begin**[1]

Number of people with TK2d experiencing impact by impact category and age group*



It is difficult to see my physical strength diminish and feel like I am losing independence. There are normal day-to-day activities that require extra planning and it's overwhelming.

–Young adult, with age of TK2d symptom onset >2 to ≤12 years (self-report)

*Impacts are a grouping of World Health Organization International Classification of Functioning, Disability and Health components – impairment, activity and participation.
†Basic activities of daily living include difficulties walking and eating/swallowing, issues with control of urine and bowel movements; ‡Development includes developmental delays and loss of normal milestones and/or functions; §Physical development includes failure to thrive, losing weight or unable to maintain weight, and malnutrition; ‖Communication includes communication challenges, problems with non-verbal communication (e.g. nodding) and speech problems.
TK2d, thymidine kinase 2 deficiency.

1. Yeske P, et al. 2025. Muscular Dystrophy Association (MDA) Clinical & Scientific Conference; Dallas, TX, USA; March 16–19, 2025. P252.

Overview of the clinical studies used to assess the efficacy and safety of KYGEVVI

Trial 1
(NCT03845712, TK0102)

A **prospective, open-label, single-arm study** in 47 patients with genetically confirmed TK2d previously treated with pyrimidine nucleosides. Thirty-eight of these 47 patients have an age of TK2d symptom onset ≤12 years; none of the 38 patients discontinued treatment. The initial oral dose of KYGEVVI was matched to the patient's pyrimidine nucleoside dose of 260-800 mg/kg/day upon entering the study in patients with an age of TK2d symptom onset ≤ 12 years, and dosage was titrated, as needed, over a maximum of 4 weeks to the maintenance dose of 800 mg/kg/day.[1]

Study 1
(NCT03701568, MT-1621-101)

A **retrospective chart review study** in 38 patients with genetically confirmed TK2d treated with pyrimidine nucleosides. Twenty-nine of these patients had an age of TK2d symptom onset ≤12 years; none of the 29 patients discontinued treatment. Thirty-five of these 38 patients were later enrolled in Trial 1 to receive treatment with KYGEVVI and one was later enrolled in Study 2. KYGEVVI was not administered in Study 1. Patients enrolled in Study 1 were receiving pyrimidine nucleoside treatment at doses 160-800 mg/kg/day.[1]

Study 2
(NCT05017818, MT-1621-107)

A **retrospective chart review study** in 61 patients with genetically confirmed TK2d (43 untreated patients and 18 patients treated with pyrimidine nucleoside therapy). Nine of these 61 patients were also included in the expanded access program and 1 patient was included in Study 1. Twenty-seven of the 43 untreated patients had an age of TK2d symptom onset ≤12 years, and 13 of the 18 treated patients had an age of TK2d symptom onset ≤12 years. Twenty-two untreated patients were included in the untreated external control group used to evaluate survival. Of the 18 treated patients, 6 (33%) discontinued treatment due to an adverse reaction. KYGEVVI was not administered in Study 2. Patients enrolled in Study 2 were receiving pyrimidine nucleoside treatment at doses 200-1200 mg/kg/day.[1]

EAP
(TK0114)

The **Expanded Access Program data** included 43 patients receiving KYGEVVI; 9 patients were included in Study 2.[1]

KYGEVVI is a combination of doxecitine and doxribtimine, both pyrimidine nucleosides, indicated for the treatment of thymidine kinase 2 deficiency (TK2d) in children and adults with an age of symptom onset on or before 12 years[1]

TK2d, thymidine kinase 2 deficiency; EAP, Expanded Access Program.

1. KYGEVVI™ (doxecitine and doxribtimine): U.S. prescribing information. Smyrna, GA: UCB, Inc. Accessed November 4, 2025.