



# Beyond Seizures: Addressing Neurodevelopment, Quality of Life and Emerging Therapies in Dravet Syndrome Patients

## REFERENCES

Andrade DM, Berg AT, Hood V, et al. Dravet syndrome: A quick transition guide for the adult neurologist. *Epilepsy Res.* 2021;177:106743.

Bacq A, Depaulis A, Castagne V, Le Guern ME, Wirrell EC, Verley M. An update on stiripentol mechanisms of action: A narrative review. *Adv Ther.* 2024;41:1351-1371.

Balestrini S, Scheffer IE. Ameliorating seizures in Dravet syndrome: A review of newly approved and investigational drugs, RNA and gene-based therapies. *CNS Drugs.* 2026;40(4):535-548.

Britch SC, Bablonis S, Walsh SL. Cannabidiol: pharmacology and therapeutic targets. *Psychopharmacology (Berl).* 2021;238(1):9-28.

Campbell JD, Whittington MD, Kim CH, VanderVeen GR, Knupp KG, Gammaitoni A. Assessing the impact of caring for a child with Dravet syndrome: Results of a caregiver survey. *Epilepsy Behav.* 2018;80:152-156.

Clayton LM, Azadi B, Eldred C, Wilson G, Robinson R, Sisodiya SM. Feeding difficulties and gastrostomy in Dravet syndrome. *Neurol Clin Pract.* 2024;14:e200288.

Devinsky O, Cross JH, Laux L, et al. et al. Trial of cannabidiol for drug-resistant seizures in the Dravet syndrome. *N Engl J Med.* 2017;376(21):2011-2020.

Diacomit. Prescribing information. Biocodex. Published 2022. Accessed February 15, 2026. [https://www.diacomit.com/wp-content/uploads/2022/10/DIACOMIT\\_US\\_PI\\_2022.pdf](https://www.diacomit.com/wp-content/uploads/2022/10/DIACOMIT_US_PI_2022.pdf)

Ding J, Li X, Tian H, et al. SCN1A mutation-Beyond Dravet syndrome: A systematic review and narrative synthesis. *Front Neurol.* 2021;12:743726.

Dlugos DJ, Scheffer IE, French JA, et al. Bexicaserin for the treatment of seizures in developmental and epileptic encephalopathies: A phase 1b/2a trial (PACIFIC). *Epilepsia.* 2025;67:646-659.

Dravet Syndrome Foundation. Genetics of Dravet syndrome. Published 2025. Accessed February 16, 2026. <https://dravetfoundation.org/what-is-dravet-syndrome/genetics-of-dravet-syndrome/>

Dravet Syndrome Foundation. Transition Guide. Published 2021. Accessed February 16, 2026. <https://dravetfoundation.org/new-dravet-syndrome-transition-guide-2021-10-08/>

Epidiolex. Prescribing information. Jazz Pharmaceuticals, Inc.; June 2025. Accessed February 15, 2026. [https://www.accessdata.fda.gov/drugsatfda\\_docs/label/2025/210365s023lbl.pdf](https://www.accessdata.fda.gov/drugsatfda_docs/label/2025/210365s023lbl.pdf)

Fenfluramine. Prescribing information. UCB, Inc. Published 2025. Accessed February 15, 2026. <https://www.ucb-usa.com/fintepla-prescribing-information.pdf>

Garcia-Peñas JJ, Calvo-Medina R, Garcia-Ron A, Gil-Nagel A, Villanueva VV, Sánchez-Capintero R. Use of stiripentol in patients with Dravet syndrome: Common practice among experts in Spain. *Neurol Ther.* 2025;14:27-43.

Griffin A, Hamling KR, Knupp K, Hong SG, Lee LP, Baraban SC. Clemizole and modulators of serotonin signaling suppress seizures in Dravet syndrome. *Brain.* 2017;140:669-683.

Guerrini R, Chancharme L, Serraz B, Chiron C. Additional results from two randomized, placebo- controlled trials of stiripentol in Dravet syndrome highlight a rapid antiseizure efficacy with longer seizure-free periods. *Neurol Ther.* 2024;13:869-884.



## Beyond Seizures: Addressing Neurodevelopment, Quality of Life and Emerging Therapies in Dravet Syndrome Patients

Knupp KG, Scarbro S, Wilkening G, Juarez-Colunga E, Kempe A, Dempsey A. Parental perception of comorbidities in children with Dravet syndrome. *Pediatr Neurol.* 2017;76:60-65.

Knupp K, Sullivan J, Perry MS, et al. Zorevunersen demonstrates disease-modifying potential in patients with Dravet syndrome with increases in seizure-free days, improvements in quality of life, and benefits in overall functioning. Presented at: 2025 American Epilepsy Society Annual Meeting; December 5-9, 2025; Atlanta, Georgia. Abstract 1.379.

Lagae L, Sullivan J, Knupp K, et al. Fenfluramine hydrochloride for the treatment of seizures in Dravet syndrome: a randomized, double-blind, placebo-controlled trial. *Lancet.* 2019;394(10216):2243-2254.

Laux L, Sullivan J, Perry MS, et al. Zorevunersen in children and adolescents with Dravet syndrome. *N Engl J Med.* 2026;394:969-982.

Li W, Schneider AL, Scheffer IE. Defining Dravet syndrome: An essential pre-requisite for precision medicine trials. *Epilepsia.* 2021;62:2205-2217.

Licheni SH, McMahon JM, Schneider AL, Davey MJ, Scheffer IE. Sleep problems in Dravet syndrome: a modifiable comorbidity. *Dev Med Child Neurol.* 2018;60(2):192-198.

Lim KH, Han Z, Jeon HY, et al. Antisense oligonucleotide modulation of non-productive alternative splicing upregulates gene expression. *Nat Commun.* 2020;11(1):3501.

Miller I, Scheffer IE, Gunning B, et al. Dose-ranging effect of adjunctive oral cannabidiol vs placebo on convulsive seizure frequency in Dravet syndrome: A randomized clinical trial. *JAMA Neurol.* 2020;77(5):613-621.

Nabbout R, Mistry A, Zuberi S, et al. Fenfluramine for treatment-resistant seizures in patients with Dravet syndrome receiving stiripentol-inclusive regimens: A randomized clinical trial. *JAMA Neurol.* 2020;77(3):300-308.

Ray A, Ko S, Donner E, et al. EPX-100 as adjunctive therapy in patients with Dravet syndrome: Preliminary results from the open-label extension phase of the ARGUS study. Presented at: 2025 American Epilepsy Society Annual Meeting; December 5-9, 2025; Atlanta, Georgia. Abstract 3.353.

Rodda JM, Scheffer IE, McMahon JM, et al. Progressive gait deterioration in adolescents with Dravet syndrome. *JAMA Neurol.* 2012;69(7):873-878.

Scheffer IE, Halford JJ, Miller I, et al. Add-on cannabidiol in patients with Dravet syndrome: Results of long-term open-label extension trial. *Epilepsia.* 2021;62:2505-2517.

Shao W, Liu L, Gu J, et al. Spotlight on mechanism of sudden unexpected death in epilepsy in Dravet syndrome. *Transl Psychiatry.* 2025;15(1):84.

Shmueli S, Sisodiya SM, Gunning WB, Sander JW, Thijs RD. Mortality in Dravet syndrome: A review. *Epilepsy Behav.* 2016;64:69-74.

Sourbron J, Lagae L. Fenfluramine: a plethora of mechanisms? *Front Pharmacol.* 2023;14:1192022.

Sullivan J, Deighton AM, Vila MC, et al. The clinical, economic, and humanistic burden of Dravet syndrome – A systematic literature review. *Epilepsy Behav.* 2022;130:108661.

Sullivan J, Lagae L, Cross JH, et al. Fenfluramine in the treatment of Dravet syndrome: Results of a third randomized, placebo-controlled clinical trial. *Epilepsia.* 2023;64(10):2653-2666.



## Beyond Seizures: Addressing Neurodevelopment, Quality of Life and Emerging Therapies in Dravet Syndrome Patients

Sullivan J, Knupp K, Wang F, et al. EMPEROR phase 3 study design: Evaluation of zorevunersen as a potential disease-modifying therapy for Dravet Syndrome. Presented at: International Epilepsy Congress; August 30- September 3, 2025; Lisbon, Portugal. Abstract P358.

Sullivan J, Perry MS, Brunklaus A, et al. Zorevunersen continues to demonstrate potential as a disease-modifying therapy in long-term open-label extension studies of patients with Dravet syndrome. Presented at: 2025 American Epilepsy Society Annual Meeting; December 5-9, 2025; Atlanta, Georgia. Abstract 2.341.

Sullivan J, Wirrell EC, Knupp KG, et al. Natural history of children and adolescents with Dravet syndrome. *Neurology*. 2025;105:e214388.

Sullivan J, Scheffer I, Howell K, et al. POLARIS phase 1/2 program interim safety and preliminary efficacy results of ETX101, a one-time gene regulation therapy, in young children with Dravet syndrome. Presented at: 2025 American Epilepsy Society Annual Meeting; December 5-9, 2025; Atlanta, Georgia. Abstract 1.308.


Sylvia C, Polega S, Knowles N, et al. Bexicaserin for the treatment of seizures in developmental and epileptic encephalopathies: Interim analysis of an expanded access program for participants on treatment for up to 2 years. Presented at: 2025 American Epilepsy Society Annual Meeting; December 5-9, 2025; Atlanta, Georgia. Abstract 1.564.

The SCN1A-Epilepsy Prediction Model. Published 2022. Accessed March 18, 2026. <https://scn1a-prediction-model.lalresearchgroup.org>

Wang YQ, Fang ZX, Zhang YW, et al. Efficacy of the ketogenic diet in patients with Dravet syndrome: A meta-analysis. *Seizure*. 2020;81:36-42.

Wirrell E. Treatment of Dravet syndrome. *Can J Neurol Sci*. 2016;43:S13-S18.

Wirrell EC, Hood V, Knupp KG, et al. International consensus on diagnosis and management of Dravet syndrome. *Epilepsia*. 2022;63:1761-1777.

Xu X, Yang X, Wu Q, et al. Amplicon resequencing identified parental mosaicism for approximately 10% of "de novo" SCN1A mutations in children with Dravet syndrome. *Hum Mutat*. 2015;36:861-872. 

Ziobro J, Eschbach K, Sullivan JE, Knupp KG. Current treatment strategies and future treatment options for Dravet syndrome. *Curr Treat Options Neurol*. 2018;20(12):52.