

Collective Voices in Gut1 Deficiency Project Survey – Report

The Glut1 Deficiency Collective Voices Project was designed to have a better understanding of the patient and family experience across a broad range of areas. The goals for the survey were to better define the range of symptoms, identify gaps in treatment and patient care, identify the gaps in knowledge and understanding of this disease, better understand disease burdens, identify the most important components of a future natural history study, develop and prioritize a patient-led strategic research plan, and to develop better and more effective clinical trials for potential future treatments.

The report will be presented in sections, and the first section reported is **Other Treatments**.

Questions:

- 1. Have you tried any treatments, medications, or supplements (prescribed or over the counter) that have been effective for seizure control?**

52% of the respondents report not having tried other treatments for seizure control, while 36% report as trying other treatments. 12% report not having this symptom.

Please see below the list of reported treatments for seizure control and the percentage of respondents reporting use of the treatment. It is important to point out that some people have tried more than one treatment.

Levetiracetam (Keppra) **27%**
Clonazepam (Klonopin) **1%**
Lamictal (Lamotrigine) **12%**
Sodium Valproate (Valproic Acid, Depakine, Epilim, Depakote) **15%**
Zonisamide (Zonegran) **3%**
Ethosuximide (Zarontin, Petinimid) **11%**
Acetazolamide (Diamox) **11%**
Epidiolex **3%**
KetoVie **1%**
Triheptanoin (C7 oil) **11%**
Diazoxide (Proglycem) **4%**
Apydan **1%**
Metformin (Glucophage) **1%**
Trileptal (Oxcarbazepine) **1%**
Clobazam (Frisium, Onfi) **4%**
Vagal Nerve Stimulation (VNS) **1%**
Lacosamide (Vimpat) **1%**
Cortisone boost therapy **1%**

Vigabatrin (Sabril) **1%**
Mesuximid (Petinutin, Celontin) **1%**
Levebon (Newest form of Levetiracetam) **1%**
Topiramate (Topamax) **1%**
Synacthen depot (Tetracosactide) **1%**
Homeopathic Medicine **1%**
CBD Oil **3%**
Cyproheptadine (Periactin) **1%**
Phenobarbital (Luminal) **1%**
Carbamazepine (Tegretol) **1%**

2. Have you tried any treatments, medications, or supplements (prescribed or over the counter) that have been effective for movement issues?

72% of respondents report as not having tried other treatments for movement issues and 16% report trying other treatments. 12% report not having this symptom.

Please see below the list of reported treatments for movement issues and the percentage of respondents reporting use of the treatment. It is important to point out that some people have tried more than one treatment.

Baclofen (Lioresal) **~11%**
Ativan (Lorazepam) **~3%**
Clobazam (Onfi) **~3%**
Levetiracetam (Keppra) **~3%**
Sinemet **~7%**
Trihexyphenidyl **~3%**
CBD Oil **~7%**
Diazoxide (Proglycem) **~3%**
Acetazolamide (Diamox) **~23%**
Metformin (Glucophage) **~3%**
Rehabilitation Therapies **~7%**
Baclofen pump **~3%**
Valproic Acid (Sodium Valproate, Depakine, Epilim, Depakote) **~3%**
Electrolytes **~3%**
Amantadine (Gocovri) **~3%**
Synacthen depot (Tetracosactide) **~3%**
Arch supports **~3%**
C7 Oil **10%**

3. **Have you tried any treatments, medications, or supplements (prescribed or over the counter) that have been effective for ADD/ADHD or focus?**

48% of respondents have not tried other treatments for ADD/ADHD or focus, while 16% report having tried other treatments. 36% report not having this symptom.

Please see below the list of reported treatments for ADD/ADHD or focus and the percentage of respondents reporting use of the treatment. It is important to point out that some people have tried more than one treatment.

Ritalin (Concerta, Methylphenidate) ~33%
Vyvanse (lisdexamfetamine) ~ 13%
Adderall (Mixes amphetamine salts) ~7%
Strattera (Atomoxetine) ~3%
Risperidone (Risperdal) ~3%
Amino Acid supplement (BAM) ~3%
Focalin (Desmethylphenidate) ~3%
Gliatilin ~3%
Carnitine ~3%
Medikinet ~3%
Occupational Therapy ~3%
Theory of Mind Therapy ~3%
Dexamfetamine (Dextroamphetamine) ~3%
4 Life transfer factor ~3%
Behavior Therapy ~3%

4. **Have you tried any treatments, medications, or supplements (prescribed or over the counter) that have been effective for frequent headaches/migraines?**

37% of the respondents report not trying any other treatments for frequent headaches/migraines while 49% report not having this symptom. 14% Report trying other treatments.

Please see below the list of reported treatments for frequent headache/migraines and the percentage of respondents reporting use of the treatment. It is important to point out that some people have tried more than one treatment.

Ibuprofen ~19%
Tylenol ~19%
Lamotrigine (Lamictal) ~4%
Naproxen ~4%
Natural ointments and essential oils ~4%
Migraine cocktail of zofran, Tylenol, benadryl, and gabapentin ~4%
C7 Oil (Triheptanoin)

Sumatriptan (Imitrex, Treximeb) ~4%
Ondasentron (Zofran) ~4%
Sodium Valproate (Valproic Acid, Depakine, Epilim, Depakote) ~4%
Excedrin ~4%
Propranolol (Inderal) ~4%
Pizotifen (Sandomigran) ~4%
B-2 ~4%
Periactin (Cyproheptadine) ~4%
Paracetamol (Acetaminophen) ~7%

5. **Have you tried any treatments, medications, or supplements (prescribed or over the counter) that have been effective for anxiety?**

Most of the respondents(44%) report not trying other treatments for anxiety, 46% report not having this symptom and 10% report trying other treatments.

Please see below the list of reported treatments for frequent headache/migraines and the percentage of respondents reporting use of the treatment. It is important to point out that some people have tried more than one treatment.

Fluoxetine (Prozac, Sarafem) ~10%
Paroxetine (Paxil, Serotax) ~10%
Medical Marijuana ~5%
CBD Oil ~10%
Sertraline (Zoloft) ~21%
Lemon balm ~5%
Diazoxide (Proglycem) ~5%
Wellbutrin (Bupropion, Zyban) ~5%
Psychological therapy ~10%
Celexa (Citalopram) ~10%
Biofeedback ~5%

6. **Do you have a Vagal Nerve Stimulator (VNS)?**

96 % of respondents don't have a VNS while 4% have one. 12% Patients who have a VNS report it has not been effective for seizure control.

7. **Have you tried C7 oil (triheptanoin)?**

85% of respondents report not having tried C7 oil, while 15% report having tried it. Patients who have tried C7 Oil were asked how effective this treatment was for a number of different symptoms, their responses are shown in the table below.

Symptom	Very effective	Somewhat effective	Not effective	Made symptom worse	I do not have this symptom
Seizures	29.6%	25.9%	33.3%	3.7%	7.4%
Movement issues	18.5%	25.9%	33.3%	7.4%	14.8%
Cognition	22.2%	40.7%	37%	0	0
Attention/focus	22.2%	33.3%	40.7%	0	3.7%
Energy/Stamina	29.6%	33.3%	33.3%	0	3.7%
Headaches	7.4%	11.1%	48.1%	0	33.3%
Behavior	18.5%	7.4%	44.4%	3.7%	25.9%
Speech/Communication	14.8%	29.6%	48.1%	0	7.4%
Other symptom	14.8%	0	29.6%	7.4%	48.1%

Most of the respondents who have tried C7 Oil report it being very effective or somehow effective treating seizures, movement issues, cognition, attention/focus and helping with energy/stamina. On the other hand, most of the respondents report C7 oil not being effective to treat headaches, helping with behavior, speech/communication or other symptoms.

In addition, most (44%) of the patients who have tried C7 Oil decided to stop taking it for various reasons, including that it did not help with their symptoms or that it caused side effects that made it difficult to continue its use.

8. Have you tried corn starch as a treatment?

The great majority of respondents (94%) report not trying corn starch, only 6% have tried it. However, most of them (60%) report it as not being helpful for symptom control.

9. Have you tried CBD Oil as a treatment?

The great majority of respondents (92%) report not trying corn starch, only 8% have tried it. Most of them (53%) report it as being helpful for symptom control and 27% are not sure if it has been helpful.

Here are the symptoms that people report CBD Oil helping with:

Anxiety, movement episodes, dystonia, sleeping, focus, feeling steadier, less foggy, memory, energy levels, muscle cramps and ataxia.