

RAVICTI FOR DUMMIES®



A Reference for the Rest of Us!



Department of Neurology

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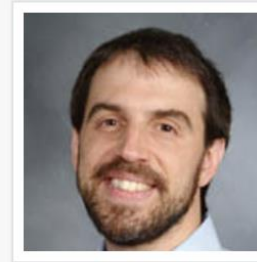
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VANDERBILT  **UNIVERSITY**
MEDICAL CENTER

ClinicalTrials.gov

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PEDIATRIC NEUROLOGY

Zachary Grinspan, M.D., M.S.

PROVIDING WORLD-CLASS CARE WITH  **NewYork-Presbyterian**


Specialties: Pediatric Neurology, [more](#) ▾

 Accepts New Patients  Treats Children

Phenylbutyrate for Monogenetic Developmental and Epileptic Encephalopathy

ClinicalTrials.gov ID  NCT04937062

Sponsor  Weill Medical College of Cornell University

Information provided by  Weill Medical College of Cornell University (Responsible Party)

Last Update Posted  2024-08-20

The drug has been **VERY PROMISING** for SLC6A1

Ravicti Pros

Seizure Reduction

Behavior Improvement

Development Improvement

Consolidation of AEDs

Little Side Effects

Not a “Dirty” Epilepsy Drug

Ravicti Cons

⚠️ Kroger Rx: We received a new RX from your prescriber. Est cost \$56942.99. Call [972-377-0840](tel:972-377-0840) to confirm you would like it filled. Txt STOP to optout



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Text Message



Ravicti is **EXPENSIVE**

Ravicti has Gone Generic

TRANSITIONING TO A GENERIC:
Preparing for Generic Glycerol Phenylbutyrate



WHAT CHANGE IS COMING FOR PATIENTS CURRENTLY TAKING RAVICTI® (glycerol phenylbutyrate)?

As early as July, generic glycerol phenylbutyrate is expected to become available.

Note that this timing may shift; Amgen does not control the timing of generic medicine availability.

Price Decline of ~10%
~\$785,000/Year



- Insurance **Might be More** Inclined to Cover
- **Expect Denials** Because the Drug is Still **Off Label**



Possible Alternatives

- Frequent **Shortages**
- **Hard to Monitor**
- An *Official* Trial is **Difficult**

amazon

Access Remains Too Difficult

Feedback I Commonly Hear

- My Doctor **Requires More Research** to Prescribe
- The Drug is **Too Expensive**
- Insurance is **Rough**

Irreconcilable Differences We Must Overcome

- There is **NO Financial Incentive** for Amgen to Seek an Additional Indication
- There is **NO Practical Regulatory Pathway** for the Non-Profit to Seek an **Additional Indication**
- **Insurance** will Always be **Jerks**
- There will Always be **More Studies** that **Could be Done**

Compromises

- We Need **Peer Reviewed Publications** ASAP
- We can **Continue the Trial** but Patients must **Buy Their Own Drug** at around ~\$20,000/year
- Ravicti needs to be Submitted into **Compendiums**
- **I Wish Doctors would Simply Try – if this were Prozac, there would be Zero Hesitation.**

We Must look Past the Price

**If this were a Cheap Drug — Would We Really
Still be Spending this Much Time Debating?**

Patient Organization Quandary

We have Funded Roughly \$350,000 in Studies for a SAFE FDA Approved Drug

No Pathway to Add an Existing Indication

There is no Central Insurance Authority for Drug Coverage

Additional Studies are Impractical Due to Cost

We Can't Control the Timing of Publications

Trying is Becoming Easier

- **Our Resident PharmD
Created an Entire
Playbook**

Phenylbutyrate Play Book for SLC6A1 (May 2025)

Pilot Study Pre-Print Link/Benefits from Pilot Study	1
Side effects and Risks with Phenylbutyrate	1 - 2
Drug Interactions	2
Exclusions for Pilot Study	2 -3
Off-label Explanation	3
Informed Consent	3 - 4
Tips for Insurance Approvals	4
Formulations of Phenylbutyrate	

Appendices:

Appendix A: SOAP (Clinical Note) for Prescriber

Appendix B: Provider Medical Necessity Cover Letter Template

Appendix C: Supportive Medical Necessity Letter Template from Z Grinspan

Appendix D: Glycerol Phenylbutyrate Dosing Guidance

Appendix E: Metabolic Acidosis protocol

Appendix F: Phenylbutyrate References

What's Next?

**Doctors
Prescribe Off-
Label**

**Clinical Trial
Continues***

**Publications &
Compendium
Submissions**