

Levels of Evidence: Clinician Perspectives on the Potential of DBS for Pediatric Patients with Treatment-Resistant OCD



Pham MT¹, Campbell TA², Torgerson L³, Dorfman N³, Kostick Quenet K³, Storch EA⁴, Blumenthal-Barby J³, Lázaro-Muñoz G^{2,5}

¹Center for Bioethics and Social Justice, Michigan State University; ²Center for Bioethics, Harvard Medical School; ³Center for Medical Ethics & Health Policy, Baylor College of Medicine; ⁴Department of Psychiatry and Behavioral Science, Baylor College of Medicine; ⁵Department of Psychiatry, Massachusetts General Hospital

Background

- Deep brain stimulation (DBS) is currently used in pediatric patients with treatment-resistant dystonia, though no randomized control trials (RCTs) were conducted before this use and practitioners relied on evidence from use in adults.
- Accumulated research supports the safety and effectiveness of DBS for obsessive-compulsive disorder (OCD) in adults (Wu et al.2021). Given that approximately 10-20% of children with OCD have treatment-resistant presentations, it is likely that there will be interest in offering DBS for some children (POTS 2004).

Methods

- In-depth, semi-structured interviews were conducted with clinicians (n=24) with expertise in treating pediatric patients with treatment-resistant OCD.
- Interview transcripts were analyzed using thematic content analysis.

Results: Clinician Perspectives

- The World Society for Stereotactic and Functional Neurosurgery (WSSFN) has argued that at least two successful randomized controlled trials (RCTs) should be available before DBS treatment for a psychiatric disorder is considered “established.”
- Clinicians were asked: **whether two successful RCTs is a necessary level of evidence to offer DBS for treatment-resistant OCD in pediatric patients.**
- Nearly half of clinicians (n=11; 45%) were in favor of two successful RCTs:

RCTs constitute appropriate level of evidence

“Yeah, I think we should have **at least that level of evidence** [two RCTs].” (003)

“I would like to have evidence. [...] **And I would love to see at least two trials, preferably independent trials..**” (023)

Evidence to Justify Efficacy of Treatment

“We have to study it. I think we do, because **otherwise we don’t know it’s effectiveness or efficacy.**” (011)

“[W]e probably should not be offering it without **some solid evidence that there are reasonable benefits to expect.**” (012)

- Some clinicians (n=4; 17%) identified challenges with pursuing two successful RCTs

Few centers with capacity

- “It would be very difficult to get two separate randomized controlled trials. **There’s so few centers that really have the capacity to do this.**” (001)

Not enough participants

- “[T]here’s **no way you’re going to get an adequate number of patients to participate in a clinical trial** that will have enough power to actually be useful. If we wait for that, it’s just never going to happen.” (014)

Take too long

- “Two randomized controlled trials, **that’s going to take a very long time to do..**” (020)

- Some clinicians (n=5; 21%) proposed **alternative standards** to offer DBS to pediatric patients

1 RCT

- “My inclination is to say **one randomized blinded, well-conducted study** should be sufficient.” (001)

Family informed

- [A]s long as families and caregivers and parents **knew that there hadn’t been those trials, and that whatever the level of risk was,** I think it would be reasonable that they could pursue it without the trials. (022)

Safety

- “I guess I would want to make sure that [...] there was **adequate evidence of safety.** [...] So I guess it would depend on the safety of it in children and adolescents [...]” (024)

Conclusion

- These preliminary results on clinician perspectives will be useful for researchers, ethicists, and policy-makers as interest in using DBS for treatment-resistant conditions in pediatric patients grow. Additional research is needed to understand other important stakeholder perspectives (e.g., prospective patients, caregivers).

References

POTS. 2004. “Cognitive-Behavior Therapy, Sertraline, and Their Combination for Children and Adolescents With Obsessive-Compulsive Disorder.” *JAMA* 292 (16): 1969.

Wu, Hemmings, Marwan Hariz, Veerle Visser-Vandewalle, Ludvic Zrinzo, Volker A. Coenen, Sameer A. Sheth, Chris Bervoets, et al. 2021. “Deep Brain Stimulation for Refractory Obsessive-Compulsive Disorder (OCD): Emerging or Established Therapy?” *Molecular Psychiatry* 26 (1): 60–65.

Acknowledgements

Research for this work was supported by a BRAIN Initiative grants from the US National Institutes of Health under Award Number **R01MH121371**. The views expressed are those of the authors alone, and do not necessarily reflect views of the NIH, Harvard Medical School, Massachusetts General Hospital, or Baylor College of Medicine.