Original Investigation

Family-Based Treatment of Early Childhood Obsessive-Compulsive Disorder

The Pediatric Obsessive-Compulsive Disorder Treatment Study for Young Children (POTS Jr)—A Randomized Clinical Trial

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IMPORTANCE Cognitive behavior therapy (CBT) has been established as efficacious for obsessive-compulsive disorder (OCD) among older children and adolescents, yet its effect on young children has not been evaluated sufficiently.

OBJECTIVE To examine the relative efficacy of family-based CBT (FB-CBT) involving exposure plus response prevention vs an FB relaxation treatment (FB-RT) control condition for children 5 to 8 years of age.

DESIGN, SETTING, AND PARTICIPANTS A 14-week randomized clinical trial (Pediatric Obsessive-Compulsive Disorder Treatment Study for Young Children [POTS Jr]) conducted at 3 academic medical centers between 2006 and 2011, involving 127 pediatric outpatients 5 to 8 years of age who received a primary diagnosis of OCD and a Children's Yale-Brown Obsessive Compulsive Scale total score of 16 or higher.

INTERVENTIONS Participants were randomly assigned to 14 weeks of (1) FB-CBT, including exposure plus response prevention, or (2) FB-RT.

MAIN OUTCOMES AND MEASURES Responder status defined as an independent evaluator–rated Clinical Global Impression–Improvement scale score of 1 (very much improved) or 2 (much improved) and change in independent evaluator–rated continuous Children's Yale-Brown Obsessive Compulsive Scale total score.

RESULTS Family-based CBT was superior to FB-RT on both primary outcome measures. The percentages of children who were rated as 1 (very much improved) or 2 (much improved) on the Clinical Global Impression–Improvement scale at 14 weeks were 72% for FB-CBT and 41% for FB-RT. The effect size difference between FB-CBT and FB-RT on the Clinical Global Impression–Improvement scale was 0.31 (95% CI, 0.17-0.45). The number needed to treat (NNT) with FB-CBT vs FB-RT was estimated as 3.2 (95% CI, 2.2-5.8). The effect size difference between FB-CBT and FB-RT on the Children's Yale-Brown Obsessive Compulsive Scale at week 14 was 0.84 (95% CI, 0.62-1.06).

CONCLUSIONS AND RELEVANCE A comprehensive FB-CBT program was superior to a relaxation program with a similar format in reducing OCD symptoms and functional impairment in young children (5-8 years of age) with OCD.

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Early childhood-onset obsessive-compulsive disorder (OCD) disrupts social, family, and academic functioning, compromising achievement of normal developmental milestones.[1-4] The efficacy of cognitive behavior therapy (CBT), selective serotonin reuptake inhibitors (SRIs), and their combination has been established for older children and adolescents with OCD,[5-10] yet little is known about their efficacy among younger children. Young children with OCD have been found to have similar clinical profiles, including comparable obsession/compulsion types and multiple comorbidities.[11-15] Only religious/scrupulosity obsessions and an increased likelihood of depressive disorders are more common among older children with OCD.[11,12,14] Because pediatric OCD's pernicious effect on functioning extends into adulthood,[16-18] developing effective, developmentally sensitive interventions for early-emerging OCD is a public health imperative.

Contemporary CBTs for older children do not adequately address the unique features of OCD in young children (5-8 years of age), especially developmental differences, family context, unique symptom correlates, and the family's initial contact with the mental health system. Although CBT for older children allows for parent involvement, it often does not provide explicit, systematic instructions for structuring parental participation. It also does not provide specific strategies for explaining concepts to patients with less advanced cognitive abilities.

An evaluation of our developmentally sensitive, family-based CBT (FB-CBT) protocol adapted for children 5 to 8 years of age provided promising results.[19,20] Adaptations addressed cognitive, socioemotional, and family contextual differences for young children, while maintaining emphasis on exposure plus response prevention (EX/RP), the CBT component with the most empirical support for treating OCD.[21] A small randomized clinical trial[20] yielded moderate and large treatment effects for FB-CBT for intent-to-treat (Cohen d = 0.53) and completer (d = 0.85) samples, respectively, when compared with FB relaxation treatment (FB-RT), a credible psychosocial control condition. Demonstrating acute efficacy definitively, however, requires a larger sample, multiple sites to permit examination of generalizability, and a broader evaluation of change across OCD symptoms, functional impairment, and quality of life.

Toward these ends, our collective research group, which has already examined the efficacy of CBT, pharmacotherapy, and their combination,[9] as well as CBT's efficacy in augmenting SRI partial response,[10] initiated the Pediatric Obsessive-Compulsive Disorder Treatment Study for Young Children (POTS Jr). In the present study, we hypothesized that FB-CBT would yield a greater response rate and improvements in continuous measures of OCD and related dysfunction compared with a relaxation protocol with a similar format and similar developmental considerations.

Methods

Design

The POTS Jr was a 14-week, parallel-group, randomized clinical trial that involved 3 sites (Brown University in Providence, Rhode Island, the University of Pennsylvania in Philadelphia, and Duke University in Durham, North Carolina) and 2 treatment conditions (FB-CBT vs FB-RT). Acute-phase outcomes were measured at the week 0 baseline visit and again at weeks 5, 9, and 14. Detailed descriptions of design considerations, measurement psychometrics, recruitment procedures, and adaptations designed to increase the developmental sensitivity of the assessment and treatment are published elsewhere.[4] A summary of the trial protocol is available at http://www.clinicaltrials.gov (NCT00533806), and the full protocol is available from the authors. Each site's institutional review board approved the study. Parents provided written informed consent, and children provided written assent; families were compensated $20 for each assessment visit during acute treatment (weeks 0, 5, 9, and 14).

Participants

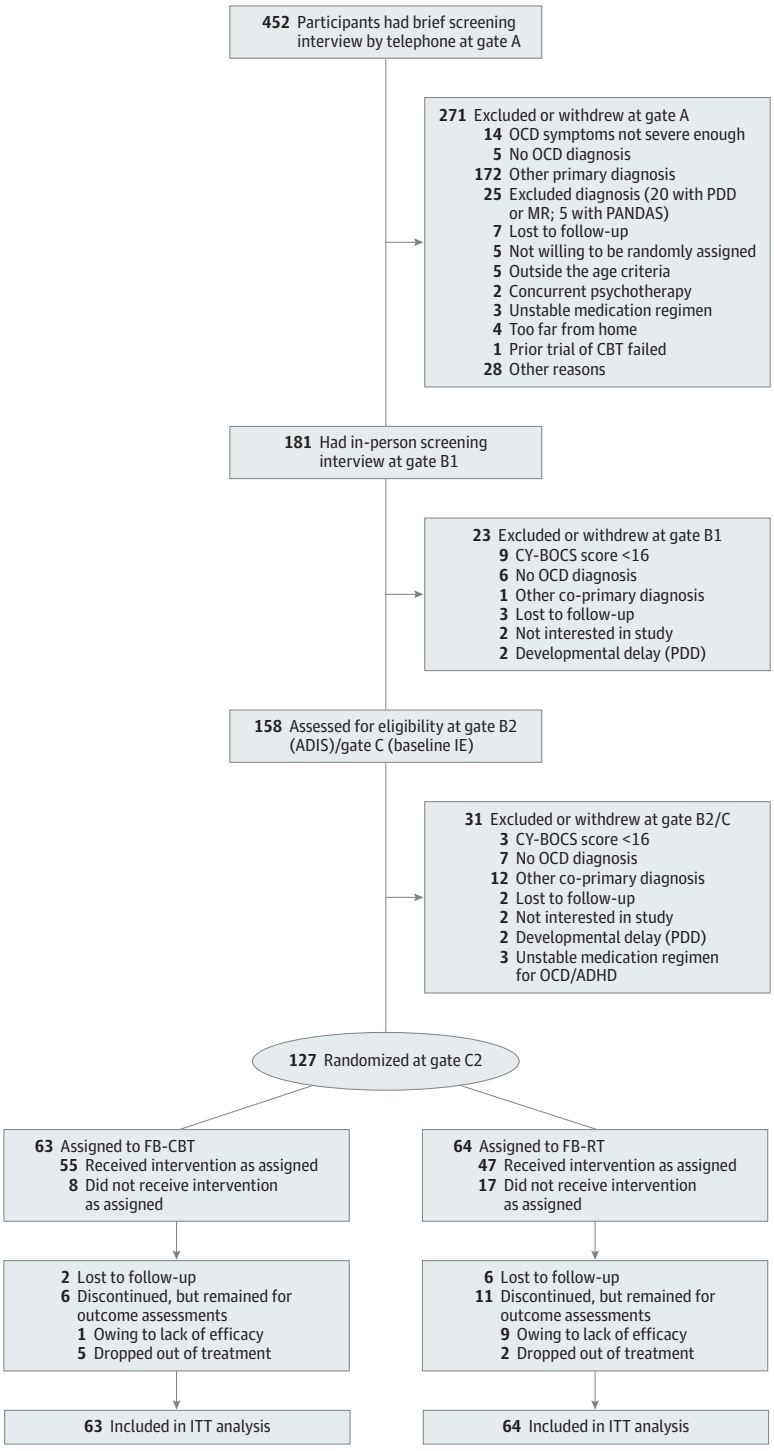
Inclusion criteria were as follows: (1) 5 to 8 years of age, (2) primary OCD according to the *DSM-IV-TR*,[22] (3) clinically relevant OCD symptoms defined as a score of 16 or higher on the Children's Yale-Brown Obsessive Compulsive Scale (CY-BOCS),[23] (4) stable symptoms for 3 months or longer, and (5) outpatient. Exclusion criteria were as follows: (1) pervasive developmental disorder(s), (2) pediatric autoimmune neuropsychiatric disorders associated with strep infection (PANDAS), (3) concurrent psychotherapy, (4) acute suicidality, (5) concomitant medications that have not been stable for 8 weeks, or (6) enrolled in prior adequate (≥10 EX/RP sessions) trial of CBT for OCD. The "primary" diagnosis was defined as the disorder causing the most significant functional impairment and clinical distress based on the Kiddie Schedule for Affective Disorders and Schizophrenia diagnostic interview with the parent(s) and child; participants could have received other co-primary diagnoses and still be included in our study.

Given that children with streptococcal-precipitated OCD may have a more episodic symptom course, we carefully considered the implications of including children with PANDAS in the present study. Our decisions were guided by the state of the PANDAS literature in 2006 when our study was funded. Participants whose parents reported during the initial telephone screening that their child had previously received a diagnosis of PANDAS or who, upon in-person assessment, met the published research diagnostic criteria for PANDAS[24] and/or were taking antibiotics for treatment/prophylaxis of OCD, tic symptoms, or rheumatic fever were excluded.

Determination of Eligibility

Eligibility was determined via a 3-gate procedure that was used in our prior randomized clinical trials.[9,10] Gate A included a brief screening interview with a parent/guardian (telephone and/or in-person). The gate B intake interview included (1) obtaining informed consent, (2) assessing OCD symptomology, and (3) assessing comorbidity. Gate C included the week 0 baseline assessment, followed by a separate meeting that revealed the randomization outcome to the family. Progression from gate A to gate C was typically completed within 3 weeks and is summarized in the CONSORT (Consolidated Standards of Reporting Trials) diagram (**Figure 1**).

Figure 1. Trial Flow Diagram



ADHD indicates attention-deficit/hyperactivity disorder; ADIS, Anxiety Disorder Interview Schedule; CY-BOCS, Children's Yale-Brown Obsessive Compulsive Scale; FB-CBT, family-based cognitive behavior therapy; FB-RT, family-based relaxation treatment; IE, independent evaluator; ITT, intention-to-treat; MR, mental retardation; OCD, obsessive-compulsive disorder; PANDAS, pediatric autoimmune neuropsychiatric disorders associated with strep infection; and PDD, pervasive developmental disorder.

Randomization

Patients were randomly assigned to 1 of the 2 treatment conditions between 2006 and 2011 using a computer-generated permuted blocking procedure, stratified by site, SRI medication status, and presence of comorbid tics at baseline.

Treatments

Treatment Delivery Schedule

Twelve sessions were delivered over 14 weeks for both treatment conditions. The first 2 sessions (with a duration of 90 minutes) were conducted with parents only, whereas the remain-

ing sessions (with a duration of 60 minutes each) were conducted jointly with parents and children. The last 2 sessions were held over 4 weeks.

Outline of Treatment Components

Family-based CBT focused on providing the child and parent with the "tools" to understand, manage, and reduce OCD symptoms. The primary components included (1) psychoeducation, (2) behavior management skills training (parent tools), (3) externalizing OCD and EX/RP (child tools), and (4) family process components. Psychoeducation topics included OCD's neurobiology, correction of OCD misattributions, identifying OCD behaviors, and rationale for treatment. Parenting tools included behavioral management of the child's OCD symptoms with differential attention (eg, ignoring and rewards), modeling, and scaffolding (ie, parent guides child's use of tools, gradually giving the child less help to promote self-regulation). All parenting tools were rehearsed in session and practiced at home as part of weekly homework assignments. Exposure plus response prevention emphasized active collaboration with the family by assisting in hierarchy development and implementing gradual exposure to triggers. Parents were actively involved during in-session and home-based EX/RP.

Key adaptations to CBT to better fit this young age group included modified psychoeducation, increased focus on parent-based skills, and simplification of CBT skills. The following adaptations to standard individual OCD treatment models were most crucial: (1) involving parents in all phases of treatment; (2) tailoring psychoeducation, exposures, and homework to meet the child's unique developmental level; and (3) focusing on the family context (eg, lack of familiarity with the mental health system, patterns of family accommodation, and parental psychopathology) and, in particular, the parent's response to their child's anxieties.

Family-based RT focused on implementing relaxation strategies aimed at lowering the child's anxiety. Family-based RT components included (1) psychoeducation, (2) affective education, and (3) relaxation training. Psychoeducation content included the relationship between stress management and anxiety, the rationale for treatment, and the implementation of a reward system devoid of other differential attention principles. Affective education emphasized teaching the child how to identify negative and positive feelings, with special emphasis on recognizing anxiety. Relaxation training consisted of developmentally adapted instruction in progressive muscle relaxation and guided imagery.

Pharmacotherapists provided oversight for patients on a stable SRI medication regimen at each independent evaluator visit. Adverse effects, with a special focus on suicidal ideation and/or behavior, were closely monitored, but families did not transfer medication management to the study physician.

Supervision and Oversight

Each site had major supervisory responsibilities: Brown University provided clinical supervision of FB-CBT and FB-RT; Duke University organized data management and statistical analyses; and the University of Pennsylvania provided supervision of diagnostic interviews and independent evaluator assessments. Treatment providers for FB-CBT and FB-RT were clinical psychologists and clinical psychology trainees already familiar with CBT. Therapists completed in-person training at study initiation and extensive on-site and cross-site supervision in both treatment arms. To assess treatment fidelity, approximately 12% of intervention sessions (92 sessions per treatment) were coded independently by trained raters. Sessions were rated on adherence to session-specific content, and each had prescribed and proscribed targets. Each target was rated based on the following scores: 1 (none to low adherence), 2 (acceptable adherence), and 3 (excellent adherence). Ratings indicated high adherence to both prescribed elements (with mean [SD] scores of 2.86 [0.09] and 2.94 [0.07] for FB-CBT and FB-RT, respectively) and proscribed elements (with mean [SD] scores of 2.96 [0.07] and 3.00 [0.01] for FB-CBT and FB-RT, respectively). An independent data and safety monitoring board provided regular oversight and met biannually during the trial.

Assessment

Independent evaluators were all doctoral-level psychologists and blind to treatment condition; they were trained to reliability on the CY-BOCS and Clinical Global Impression–Severity (CGI-S) and –Improvement (CGI-I) scales[25] through joint interviews, videotape reviews, and participation in monthly cross-site supervision conferences. Reliability was checked regularly on randomly selected videotapes, and independent evaluators were retrained if they fell below 80% agreement. Independent evaluators were trained to adapt their interview procedures as needed to maximize developmental sensitivity of assessments.[4]

Independent evaluator evaluations were completed at weeks 0, 5, 9, and 14; all parent- and therapist-reported measures were completed on scheduled visit days. Consistent with an intent-to-treat approach, all patients were assessed at each time point.

Measures

The symptoms of OCD, the severity of symptoms, and the improvement of symptoms were measured using the CY-BOCS, CGI-S, and CGI-I. The CY-BOCS is a clinician interview that merges data from clinical observation and parent and child reports. For the purposes of our study, all CY-BOCS interviews were completed with parent and child together. The literature supports the use of the measure for children as young as 6 years,[26] and it was used successfully in our prior work[11,20] with 5-year-old children. The CGI-S is a 7-point scale measuring clinician-rated illness severity. The CGI-I is a 7-point scale measuring clinician-rated improvement in treatment.[27,28] Those who were rated as "much improved" or "very much improved" on the CGI-I scale were considered as meeting the criterion for response (ie, the primary dichotomous outcome measure). The CGI has been used in multiple child psychiatric studies for children as young as 40 months[29] and for children with OCD as young as 3 years.[11,20,30,31]

Functioning was measured using the Children's Obsessive-Compulsive Disorder Impact Scale–Revised (COIS-R),[32] which provides a standardized format for assessing OCD's impact on

social, school, and home functioning in children and adolescents 5 to 17 years of age.[33] The parent report form of the measure was used for our study.

Quality of life was measured using the Pediatric Quality of Life Enjoyment and Satisfaction Questionnaire (PQ-LES-Q). The PQ-LES-Q is a 15-item parent-reported scale measuring quality of life. The scale has solid psychometric properties, with excellent internal consistency and adequate concurrent validity, and was developed for children and adolescents 6 to 17 years of age.[34] We used a parent report of this measure, based on the finding that close relatives are able to give accurate proxy ratings about quality of life.[35]

Demographics were measured using the caregiver-reported Conners-March Developmental Questionnaire,[36] including age, grade level, sex, race, and socioeconomic status.

Comorbid psychiatric disorders were assessed with the Kiddie Schedule for Affective Disorders and Schizophrenia for School Age Children–Present and Lifetime Version,[37,38] which is a semistructured, clinician interview that yields *DSM-IV-TR* diagnoses across Axis I domains. Interviews were administered to the primary caregiver(s) regarding the child and to children (although children 5-6 years of age varied in their ability to participate actively). This interview is routinely used to assess psychiatric diagnoses in children as young as 5 years.[39,40]

Sample Size and Power

The determination of the sample size was based on response rates of 45% for FB-CBT and 20% for FB-RT taken from our prior pilot study.[20] For the simplest versions of the primary aims, 2 independent binomial proportions were compared using the Pearson χ^2 statistic with a χ^2 approximation with a 2-sided significance level of .05; group sample sizes of 62 and 62 (N = 124) have an approximate power of 0.853 when the proportions are 0.45 and 0.20, respectively.

Missing Data

As part of the study design, efforts were made to collect outcome data on all randomly assigned participants, even when treatment was prematurely stopped.[41] Prior to analysis, we used multiple imputation to replace missing values.[42] A sequential regression multivariate imputation algorithm[43] was implemented in the IVEware package for SAS.[44] The imputation model included all longitudinal outcome measures, time since randomization, treatment indicators, putative moderators and mediators,[41] and the baseline stratification variables. Five data sets were generated. The results reported were calculated using Rubin's rules[43] for combining the results of identical analyses performed on each of the 5 imputed data sets.

Statistical Analysis

All randomly assigned participants were included in the analyses, in accordance with intention-to-treat principles. A multivariate χ^2 test was used to test for between-group differences in response rates at week 14. Separate longitudinal regression models were used to examine mean differences in the 2 continuous outcomes (CY-BOCS and COIS-R scores) between conditions at each assessment visit. Each regression model included indicators of time (assessment visit), group assignment, and all time-by-group interaction terms. Baseline stratification variables used in the randomization procedure (site, SRI medication status, and tics) were also included in each model a priori. Residual error terms were assumed to follow a mean-zero, normal distribution with an unstructured covariance structure used to capture the within-person correlation over time. The fitted models report mean scores at each assessment visit and make inferences about between-groups comparisons at the final assessment visit. Tests were 2-sided, and a P value of less than .05 was considered to be statistically significant. The sequential Dunnett test was used to control the overall (familywise) error rate.[45] Longitudinal models were fit using PROC MIXED in SAS statistical software, version 9.2 level 2M2 (SAS Institute). Throughout the analyses, adjusted degrees of freedom were implemented and presented using the EDF option in SAS. In our case, we used the SAS macro COMBCHI.[46]

To enhance interpretation of the results for response, we calculated the number needed to treat (NNT)[42] with FB-CBT relative to FB-RT. For the continuous outcomes, we calculated standardized between-group mean differences[47] at the week 14 visit.

Results

Recruitment and Retention

The CONSORT diagram is depicted in Figure 1. Recruitment took place between 2006 and 2011 and ended once the recruitment goal was met. The final follow-up visit was completed in 2013. Participants were recruited from (1) study site clinics, (2) schools, (3) primary care physicians, and (4) mental health providers, and via (5) paid and public service advertisements in local media. Of the 127 participants who were randomly assigned, 126 (99.2%) completed at least 1 postbaseline assessment. The mean number of completed sessions of FB-CBT was 11.2 (95% CI, 10.6-11.8) out of a possible 12 sessions. The mean number of completed sessions of FB-RT was 10.1 (95% CI, 9.31-10.9). A total of 102 participants (80.3%) completed acute treatment: 8 dropped out of the study and were lost to follow-up (6 FB-RT patients and 2 FB-CBT patients), 7 dropped out of treatment but remained in the study for outcome assessments (2 FB-RT patients and 5 FB-CBT patients), and 10 prematurely stopped the assigned treatment owing to lack of efficacy, received out-of-protocol treatment, but remained in the study for outcome assessments (9 FB-RT patients and 1 FB-CBT patient). Investigation of postrandomization activity indicated that FB-RT patients were more likely to prematurely stop and receive out-of-protocol treatment ($\chi^2_1 = 4.75$, $P < .03$). There was 1 adverse event reported in our study. This patient was prematurely removed from FB-CBT in order to seek additional psychopharmacological treatment for aggressive, impulsive behavior.

Sample Characteristics

Table 1 summarizes the baseline and clinical characteristics presented by treatment condition. No significant between-groups differences emerged. Few participants (7.1%) were

Table 1. Baseline Characteristics of Participants

Characteristic	FB-RT (n = 64)	FB-CBT (n = 63)	All (n = 127)
Study center, No. (%)			
Brown University	25 (39.1)	23 (36.5)	48 (37.8)
Duke University Medical Center	17 (26.6)	18 (28.6)	35 (27.6)
University of Pennsylvania	22 (34.4)	22 (34.9)	44 (34.6)
Demographics			
Age, mean (SD), y	7.0 (1.2)	7.4 (1.2)	7.2 (1.2)
Age at onset, mean (SD), y	4.7 (1.6)	5.4 (1.7)	5.1 (1.7)
5-6 y, No. (%)	31 (48.4)	23 (36.5)	54 (42.5)
Female sex, No. (%)	28 (43.8)	39 (61.9)	67 (52.8)
Race, No. (%)			
White	55 (85.9)	59 (93.7)	114 (89.8)
Black	1 (1.6)	1 (1.6)	2 (1.6)
Asian	3 (4.7)	0 (0.0)	3 (2.4)
Mixed	3 (4.7)	1 (1.6)	4 (3.1)
Not reported	2 (3.1)	2 (3.2)	4 (3.1)
Ethnicity			
Not Hispanic or Latino	61 (95.3)	60 (95.2)	121 (95.3)
Hispanic or Latino	3 (4.7)	3 (4.8)	6 (4.7)
Family income, range, $	70 000-80 000	80 000-90 000	70 000-80 000
OCD baseline severity, mean (SD)			
CY-BOCS score	25.97 (3.98)	25.13 (4.46)	25.55 (4.23)
COIS-R score	23.46 (12.68)	23.97 (16.43)	23.72 (14.62)
CGI-S scale score	4.67 (0.76)	4.71 (0.89)	4.69 (0.82)
<5, No. (%)	30 (46.9)	26 (41.3)	56 (44.1)
≥5, No. (%)	34 (53.1)	37 (58.7)	71 (55.9)
Baseline comorbidities, No. (%)			
Any	35 (54.7)	40 (63.5)	75 (59.1)
Anxiety	29 (45.3)	30 (47.6)	59 (46.5)
Separation anxiety	8 (12.5)	8 (12.7)	16 (12.6)
Specific phobia	11 (17.2)	16 (25.4)	27 (21.2)
Social phobia	10 (15.6)	4 (6.3)	14 (11.0)
Generalized anxiety disorder	13 (20.3)	12 (19.0)	25 (19.7)
Mood	1 (1.6)	1 (1.6)	2 (1.6)
Dysthymia	1 (1.6)	0 (0.0)	1 (0.8)
Depressive disorder NOS	0 (0.0)	1 (1.6)	1 (0.8)
Tic disorder, No. (%)	16 (25.0)	13 (20.6)	29 (22.8)
Tourette syndrome	7 (10.9)	7 (11.1)	14 (11.0)
Chronic motor tic disorder	3 (4.7)	2 (3.2)	5 (3.9)
Chronic vocal tic disorder	3 (4.7)	1 (1.6)	4 (3.1)
Transient tic disorder	3 (4.7)	3 (4.8)	6 (4.7)
Past tics reported	1 (1.6)	2 (3.2)	3 (2.4)
Externalizing	13 (20.3)	18 (28.6)	31 (24.4)
ADHD	10 (15.6)	8 (12.7)	18 (14.2)
Oppositional defiant disorder	6 (9.4)	12 (19.0)	18 (14.2)
Elimination disorder	5 (7.8)	2 (3.2)	7 (5.5)
Enuresis	5 (7.8)	2 (3.2)	7 (5.5)
Encopresis	1 (1.6)	0 (0.0)	1 (0.8)
Psychotropic medication, No. (%)			
For ADHD	4 (6.3)	3 (4.8)	7 (5.5)
SRI	0 (0.0)	2 (3.2)	2 (1.6)
Other	0 (0.0)	1 (1.6)	1 (0.8)
Observed participants, No. (%)			
Baseline	64 (100.0)	63 (100.0)	127 (100.0)
Week 5	62 (96.9)	62 (98.4)	124 (97.6)
Week 9	57 (89.1)	58 (92.1)	115 (90.6)
Week 14	57 (89.1)	59 (93.7)	116 (91.3)

Abbreviations: ADHD, attention-deficit/hyperactivity disorder; CGI-S, Clinical Global Impression–Severity; COIS-R, Child Obsessive-Compulsive Impact Scale–Revised; CY-BOCS, Children's Yale-Brown Obsessive Compulsive Scale; FB-CBT, family-based cognitive behavior therapy; FB-RT, family-based relaxation treatment; NOS, not otherwise observed; OCD, obsessive-compulsive disorder; SRI, serotonin reuptake inhibitor.

taking psychotropic medication during the trial, and these patients were equitably distributed between treatment arms.

Clinical Response and Effect Sizes of Clinical Significance

Using site, sex, baseline age (in months), baseline CGI-S scale score, medication status (coded as taking SRI medication at baseline or not), and tic disorder as covariate(s), the percentages of children who were rated 1 (very much improved) or 2 (much improved) on the CGI-I scale at 14 weeks were 72% for FB-CBT and 41% for FB-RT. The NNT with FB-CBT vs FB-RT to see 1 additional responder at week 14, on average, was estimated as 3.2 (95% CI, 2.2-5.8). On continuous measures with the same covariates included in the model, FB-CBT was superior to FB-RT at week 14 on the CY-BOCS (**Figure 2**) and COIS-R. Treatment effect sizes between groups for week 14 CY-BOCS and COIS-R scores were 0.84 (95% CI, 0.62-1.06) and 0.42 (95% CI, 0.06-0.77), respectively, which correspond to a large and medium standardized effect size, respectively.

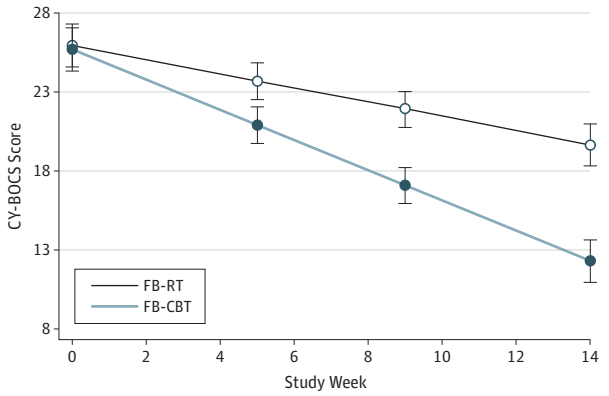
Random regression analyses identified a statistically significant linear trend with time ($t = -18.14$, $P < .001$) and a time-by-treatment interaction ($t = -6.54$, $P < .001$). There were no significant differences between conditions at week 14 on the PQ-LES, as the 95% CI included zero (0.23; 95% CI, −0.09 to 0.55). **Table 2** provides point estimates, planned comparisons, and the respective effect sizes on each continuous variable.[24]

A multivariate χ^2 test found no statistically significant site-by-treatment interactions for response at the week 14 visit ($P = .48$). Similarly, no time-by-site-by-treatment interactions were found at the week 14 visit for the continuous outcomes: CY-BOCS ($P = .58$), COIS-R ($P = .31$), or PQ-LES ($P = .82$).

Discussion

Our developmentally sensitive FB-CBT program that included EX/RP was found more efficacious in reducing OCD symptoms and functional impairment in young children (5-8 years of age) than a similarly structured relaxation program. Considering the general absence of knowledge about the efficacy of treatments for young children with early emerging OCD, these findings have significant public health implications. These findings are also consistent with those of an ear-

Figure 2. Results of Fitting Longitudinal Model



The circles indicate mean values, and the error bars indicate SD. CY-BOCS indicates Children's Yale-Brown Obsessive Compulsive Scale; FB-CBT, family-based cognitive behavior therapy; and FB-RT, family-based relaxation treatment.

Table 2. Group-Specific Response Rates, Mean Scores, and Between-Group Effect Sizes at Week 14

	Mean Score or Effect Size (95% CI)			
Week 12 Variable	Responder Status[a]	CY-BOCS[b]	COIS-R[c]	PQ-LES-Q[d]
Estimated mean scores[e]				
FB-CBT	0.72 (0.58-0.83)	12.30 (10.95-13.65)	11.68 (8.80-14.56)	4.16 (4.02-4.29)
FB-RT	0.41 (0.28-0.55)	19.67 (18.31-21.03)	16.52 (13.62 to 19.41)	4.02 (3.88-4.15)
Effect sizes[f]				
FB-CBT vs FB-RT	0.31 (0.17-0.45)	0.84 (0.62-1.06)	0.42 (0.06-0.77)	0.23 (−0.09 to 0.55)
FB-CBT NNT	3.2 (2.2-5.8)			

Abbreviations: CGI-I and -S, Clinical Global Impression–Improvement and –Severity; COIS-R, Child Obsessive-Compulsive Impact Scale–Revised; CY-BOCS, Children's Yale-Brown Obsessive Compulsive Scale; FB-CBT, family-based cognitive behavior therapy; FB-RT, family-based relaxation treatment; NNT, number needed to treat; PQ-LES-Q, Pediatric Quality of Life Enjoyment and Satisfaction Questionnaire.

[a] Responder status scores range from 0.00 to 1.00, reflecting the percentage of responders who were rated as "much improved" or "very much improved" on the CGI-I scale.

[b] The CY-BOCS scores range from 0.00 to 40.00, with larger scores reflecting more symptoms of obsessive-compulsive disorder.

[c] The COIS-R is a 33-item rating scale with items scored from 0 (not at all) to 3 (very much), with higher scores indicating greater functional impairment caused by symptoms of obsessive-compulsive disorder.

[d] The PQ-LES-Q is a 15-item rating scale with items scored from 1 (very poor) to 5 (very good); the first 14 items are summed, with higher scores reflecting greater enjoyment and satisfaction.

[e] Modeled using site, sex, baseline age (in months), baseline severity (CGI-S), medication status (taking medication at baseline or not), and presence of tic disorder at baseline as covariates.

[f] Between-group differences in estimated response rate at week 14 were given for responder status. Between-group differences in estimated mean score at week 14 divided by the pooled standard deviation of the outcome at week 14, otherwise known as the Cohen *d* (95% CI), was given for CY-BOCS, COIS-R, and PQ-LES-Q. The magnitudes associated with *d* have recognized conventions (ie, small effect, 0.20-0.49; medium effect, 0.50-0.79; large effect, ≥0.80).[47] All effect size estimates are reported such that positive scores indicate that the first treatment group was superior to the comparison group in functioning.

lier randomized pilot study,[20] as well as several studies of FB treatment for older children with OCD.[5,8]

Given the use of an active control treatment in this trial, the magnitude of the difference in response rates between groups is particularly notable.[48] Family-based RT was equivalent to FB-CBT in the amount of therapist contact time and included the cogent and face-valid rationale of using anxiety management strategies while also controlling for treatment expectancy, acceptability, credibility, and the effects of repeated assessment. Response rates on the CGI-I in this trial (72% for FB-CBT) were even better than in our group's pilot study[20] with the same population (50% for FB-CBT) and similar to or better than our other multisite OCD studies: POTS I (75% for combined CBT and selective SRI; 64% for CBT alone) and POTS II (65.7% for combined CBT and medication management), which may reflect the importance of providing treatment closer in time to when the OCD first emerges. A particular strength of these comparisons is the use of a common metric to define response. Naturally, this is only one definition of treatment response, yet we see a larger effect size difference in the continuous measure of outcome.

Family-based CBT was not only superior in reducing OCD symptoms on both categorical and continuous measures of outcome, but also reduced OCD-related dysfunction more than did the control condition. The effect on OCD-related dysfunction, though significant, was not as robust as the effect on OCD symptoms. Some previous trials found that pediatric OCD symptom improvements preceded improvement in functioning,[49] while others did not.[7] At this early age, perhaps the impact on functioning may take longer to observe, because a young child gradually approaches situations more appropriately only after significant symptom reduction.

Contrary to our hypotheses, FB-CBT was not significantly superior to FB-RT on quality-of-life ratings. Quality of life has been shown to significantly improve for adolescents undergoing CBT for OCD,[7] but perhaps for younger children, improvements in the broader construct of quality of life may lag behind improvements in symptoms and functioning. It is possible that assessment of the construct of quality of life for these very young children may require different questions and different content. The absence of change in quality of life in our study also may have been due to the fact that the PQ-LES-Q, the measure we used, was not exclusively designed for younger children (despite being validated for 6-17 years of age) and that scores were not in the clinical range at baseline.

The clinical implications of our results highlight the use of this FB-CBT model as the first-line choice for young children with OCD. Despite little psychotropic medication use in this sample, children with early-onset OCD are indeed able to benefit from a treatment approach that is uniquely tailored to their developmental needs and family context. Family-based EX/RP treatment is effective, tolerable, and acceptable to young children and their families, and appears to be feasible to implement without concomitant pharmacotherapy. The sample included children with a range of OCD severity (although, as a group, they were in the moderate-to-severe range) and co-occurring psychiatric conditions, which enhances generalizability of findings to clinical samples of young children with OCD.

Our study has several limitations that warrant mention. First, despite following strategies recommended by expert consultants involved in our study, recruitment paralleled the broader OCD treatment literature with regard to limited enrollment of racial and ethnic minority families and youth. This is a significant weakness and leaves the applicability of our findings to a broader range of ethnic and racial groups in question. Similarly, the lack of socioeconomic diversity in the sample is a concern. Another potential threat to generalizability lies in the high level of expertise at these respective sites and, by extension, of the clinicians who provided the treatments. Preliminary evidence from open studies[50,51] of CBT supports its effectiveness for pediatric OCD in community clinics with patients 7 years of age or older, but whether these findings are applicable to these community contexts with younger children remains to be discovered. Questions remain as well about the generalizability of outcomes achieved with families willing to accept randomization, although a large CBT effectiveness study of adult OCD found outcomes with nonrandomized patients with OCD slightly better than those reported from randomized trials.[52] Finally, albeit beyond the scope of our study, the durability of gains in primary outcomes and in longer-term functioning will be examined separately, as will moderators and mediators of treatment outcome.

Conclusions

In summary, our findings add to the evidence base supporting CBT for pediatric OCD by extending downward the age range that can benefit from CBT protocols emphasizing EX/RP. With appropriate parental support, young children with OCD who undergo FB-CBT can make significant gains beyond what can be expected from having parents attempt to teach relaxation strategies to their children with OCD. Hopefully, these gains in overall development can decrease the chronicity and morbidity of this debilitating illness. Our findings lend further support that these young children have "real" and impairing OCD that warrants more than a "watch and wait" approach. Finally, our study underscores the need to disseminate these treatment models beyond academic medical centers to clinical settings (eg, pediatric offices and community clinics) where young children first present for treatment.

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