

ACHIEVE Study

(A Comparison: High Intense periodic vs Every week therapy in children with cerebral palsy)

Manual of Procedures

A collaboration between the Ohio State University and
Nationwide Children's Hospital

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“The protocol will be a written document detailing how the ACHIEVE study is conducted and be included as part of an IRB amendment. The elements of a protocol, based on this revised

proposal, will include a) Study design and organization, b) Study objectives, c) Background information, d) Patient population, and e) Treatment comparisons. Requirements for patient entry, treatment and outcomes evaluation, plus procedures for data collection procedures will be clearly outlined”

A. Introduction

A1) What is the ACHIEVE Study?

The ACHIEVE study is a five-year randomized controlled trial testing the theoretical principle that frequency of rehabilitation is an important regulator of therapeutic response in pediatrics.

AIMS

The first aim of the study is to compare motor function outcomes for children participating in repeated periodic bouts of high intensity physical therapy (high intense periodic) to that of children participating in every week physical therapy. The study compares two service delivery models which provide 40 hours of therapy to children ages 2-8 years of age with cerebral palsy (CP). Short term and long-term outcomes will be assessed using a variety of outcome evaluations to determine whether therapy effects can be maintained over time. The second aim of this study is to determine individual differences in children’s response to intensity. Potential factors impacting response include age, GMFCS level, child engagement, parent engagement and parent satisfaction.

300 children, ages 2-8 years of age with CP representing all GMFCS levels (I-V) will be enrolled to determine the short term and long term effects of two dosing protocols. Both groups will receive functional and goal directed training using the principles of motor learning in a one-on-one setting. Both groups will receive a total of 40 hours of therapy. Participants will be randomized into one of two groups:

- a. 1 hour per day, 1 x per week for 40 weeks
- b. Two bouts of 2 hour per day, 5 x per week for 2 weeks with an 18 week break between bouts

The results of the ACHIEVE study will inform clinicians and families about dosing and provide much-needed recommendations for frequency of rehabilitation in order to optimize motor function and development of young children with CP. The ACHIEVE study will help clinicians make informed decisions about how and when to deliver therapy. The ACHIEVE study will help answer questions for patients with CP and their families, such as: What are my options for intensity of therapy? What are the benefits and harms of higher intensity and usual weekly programs? Given my child’s personal characteristics, conditions, and preferences, what should I expect will happen to my child if I enroll in a high intensity or usual weekly therapy program? Ultimately the answers to these questions will vastly improve patient-centered outcomes.

A2) Key Features of the ACHIEVE Study

The ACHIEVE program will include the following important features:

- **Outpatient-based delivery:** Therapy sessions are 1:1 (therapist to child). Therapists are licensed physical therapists. All therapists who treat patients in ACHIEVE will attend bi-yearly training session on treatment fidelity.

- **Motor Learning Based Therapy Techniques:** cyclical shaping with immediate positive reinforcement, massed practice, planned variation and application of motor learning principles, including:
 - repetition
 - task specificity
 - active practice
 - generalization of skills
 - allowing errors
 - allowing self-correction
 - structured practice
 - developmentally appropriate feedback
 - sufficient time to practice

- **Age Appropriate, Play Based, and Functional Activities:** Motor activities selected based on child's goals and functional level and may include: varied motor exploration in prone, supine, sitting, kneeling and standing; use of objects and toys; social interactions; cause and effect games with toys and therapist; self-help skills including feeding and taking part in dressing; facilitating spontaneous movement and exploration, functional navigation of environment with and without adaptive equipment; transfers and transitions between positions and/or equipment; balance training; locomotor skills such as jumping, hopping, climbing or pedaling a bicycle/tricycle; and any other individualized activities of interest to the child.

A3) Inclusion and Exclusion Criteria

The inclusion and exclusion criteria for the ACHIEVE program are as follows:

- **Inclusion Criteria**
 - an age of 2-8 years at the initiation of treatment
 - a diagnosis of CP in the GMFCS levels (I-V)
 - the ability to tolerate a 2 hour therapy session based on parent report and evaluating therapists

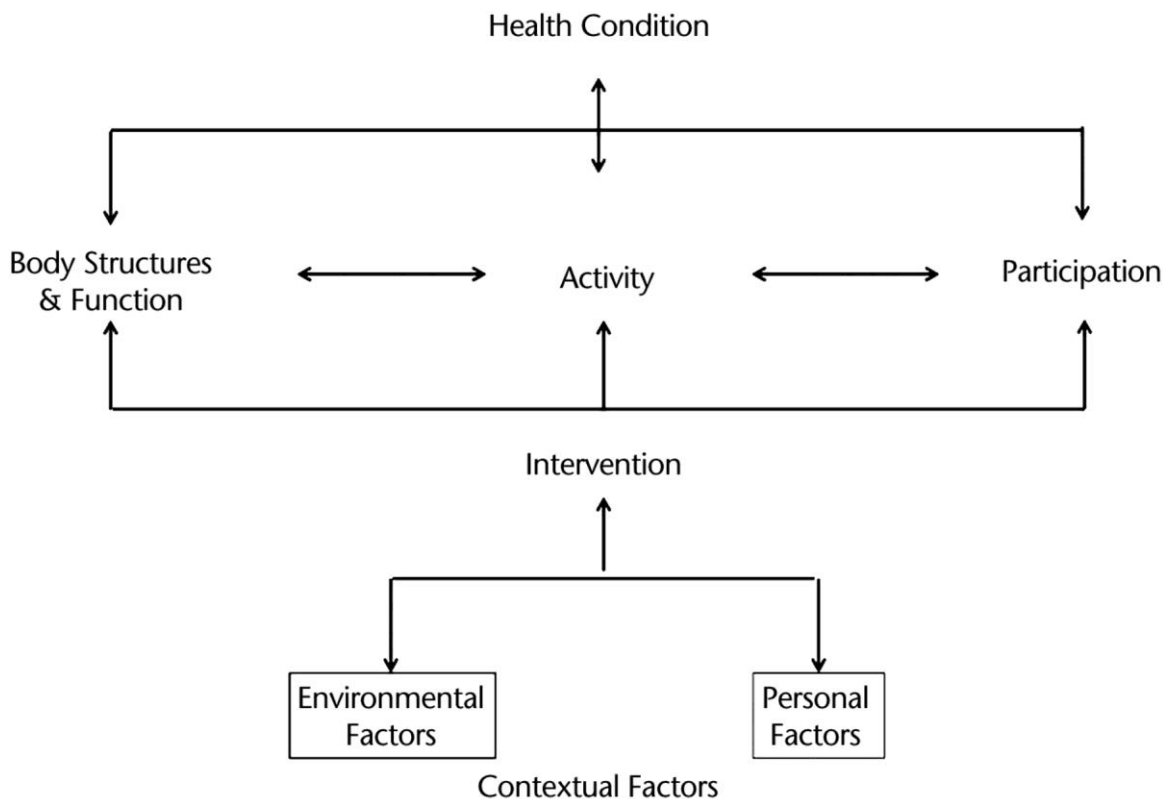
- **Exclusion Criteria**
 - uncontrollable seizures or any co-morbid condition that prevents full participation during treatment sessions.
 - participation in another daily physical therapy treatment program (5x/week) within the last 6 months
 - auditory or visual conditions that prevent full participation during treatment sessions
 - progressive neurological disorder with no potential for improvement
 - recent surgery where physical therapy is contraindicated

A4) ICF Model

The International Classification of Functioning, Disability, and Health (ICF) Model is the theoretical framework of the World Health Organization (WHO) used to conceptualize function and disability (see Figure 1 below). The ICF describes the dynamic relationship between body functions and structures, activities, participation and environmental factors and how these domains relate to a person's level of function. The Motor Learning Based intervention described here for the ACHIEVE study is targeting the activity level with the goal of increasing

functional independence and indirectly improving participation in the child's natural settings. This intervention is identical to the one described in a case series by Heathcock et al. (2015), which demonstrated positive changes at the activity level of young children with cerebral palsy, GMFCS Level V. Home exercise activities will be used to help address the child's environmental factors related to motor development. Personal factors will also be considered in treatment planning and goal setting.

Figure 1. The International Classification of Functioning, Disability, and Health (ICF) Model. Reprinted with permission from *Toward a Common Language for Functioning, Health, and Disability: The International Classification of Functioning Disability and Health*. Geneva, Switzerland: World Health Organization; 2002.



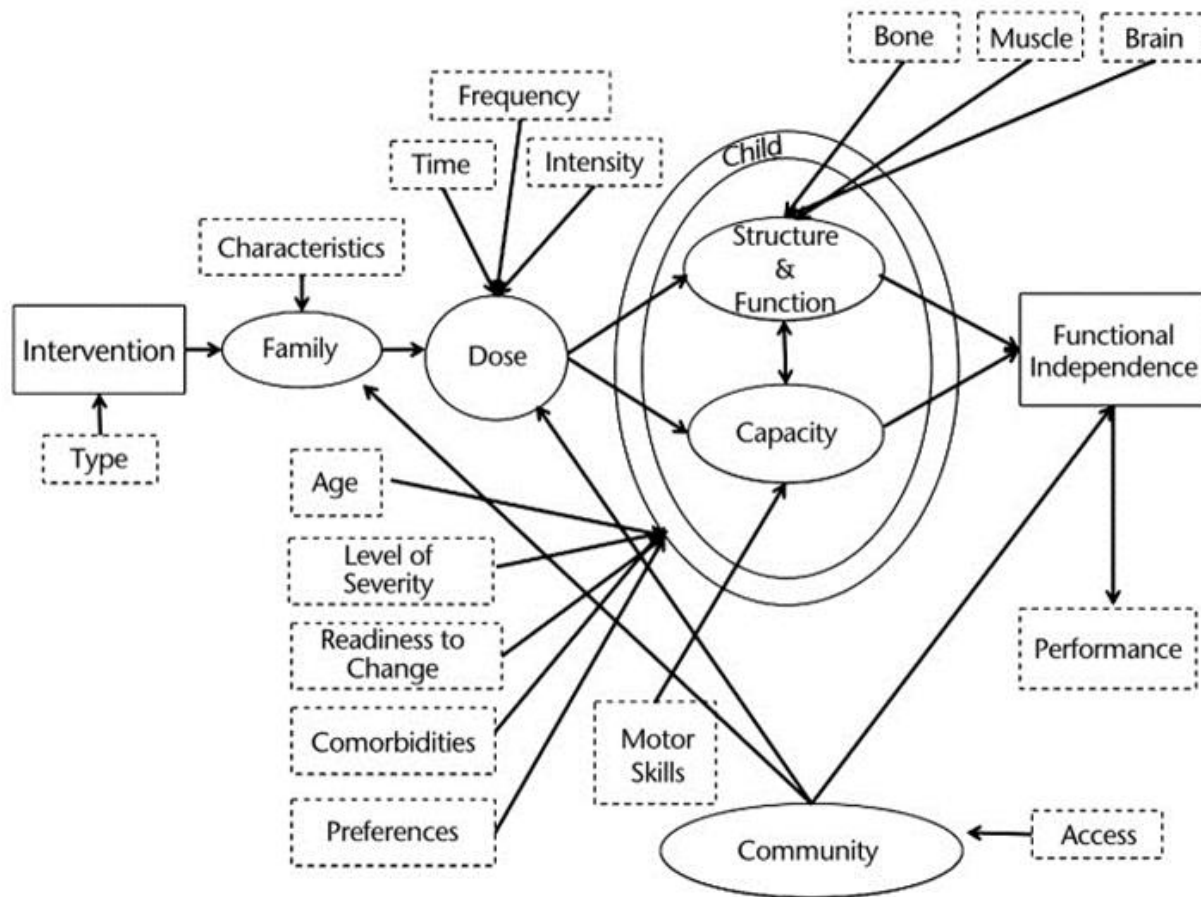
A5) Path Model for Dosing

A Path Model (see Figure 2) was created to guide researchers in studying dosing parameters for physical therapy intervention for children with cerebral palsy. Determining dosing parameters for pediatric physical therapy interventions through research is critical for informing clinical decision making, health policy, and guidelines for reimbursement (Gannotti et al 2014). The Path Model recommends researchers consider the influence of mediating and moderating variables (such as family, community and intervention dose) on the dose-response relationship and desired outcome of increasing functional independence. Gannotti et al. encourage researchers to operationalize the variable of interest for each component in the model and obtain a large enough sample size to ensure statistical power.

The ACHIEVE

study has been constructed based on this Path Model. With the focus on dosing, time and intensity has been standardized to allow a better understanding of the effect of frequency. The participant characteristics (body structure, function and capacity), which can effect dose-related responses, is also restricted in this study to allow for optimal dosing recommendations for a specific group of children with cerebral palsy. The large recruitment number will allow for statistical power.

Figure 2. Path Model for Dosing. Reprinted with permission from Gannotti ME, Christy JB, Heathcock JC, Kolobe TH. A path model for evaluating dosing parameters for children with cerebral palsy. *Physical Therapy*. Mar 2014;94(3):411-421.



B. Procedural Information

B1) Screening

B1.1 Assessment of the Child and Family

A preliminary assessment from a chart review or interaction with the child will help decide if he/she will qualify for the ACHIEVE program. If possible, this will be completed by a treating therapist. This assessment will serve to determine if the child can tolerate the two hour therapy session and establish family goals. Children who pass this initial screen and elect to participate

in the study will undergo baseline outcome evaluation and be randomized into a treatment group.

B1.2 Review of the Child's History and Treatment

A complete review of the medical history is important because of the potential intensity of the ACHIEVE program. Reviewing medical history and potential co-morbid conditions that may exist may influence eligibility and outcome for the program. This program creates an environment that may affect all areas of development, including cognitive functioning, social and emotional development. The ACHIEVE team needs to have a solid understanding of each child's medical history to make certain the child is medically stable enough to benefit from this program. A referral from a physician will also be requested as another way to ensure the child is seen as medically eligible for the program.

B1.3 Involving the Family

A detailed explanation of the ACHIEVE program will be provided to the parents and other interested family members before the child is enrolled in the program. Scheduling is an important aspect of the program to consider. We recommend the family be certain that the treatment schedule, attendance expectations and transportation requirements are manageable. Different sites will be offered in the Columbus, Ohio area for scheduling convenience. We hope to be flexible to allow each child to participate at a time of day that meets their individual needs. The scheduling options will be provided to each family prior to enrollment. The ACHIEVE program may be more intensive intervention than what the child and family are used to receiving. We make an effort to keep the atmosphere playful and engaging at all times so it is both a positive experience for the child and family. It is important to us that the families are engaged and participating in the sessions (if possible) to allow them to help reinforce and support the transition of new skills into the home environment and child's daily routine.

B2) Recruitment

Participants will be recruited from all outpatient occupational and physical therapy (OT/PT) clinics at Nationwide Children's Hospital (NCH), NCH outpatient specialty clinics, including Neonatology Clinic, Cerebral Palsy Clinic, and Stroke Clinic, and through posted advertisements. The lead therapists at NCH will review schedules of the outpatient OT/PT clinics and specialty clinics to identify possible candidates. All treatment therapist will also be encouraged to contact the lead therapists if they see a child who may qualify for the study. The study coordinator and/or research assistants will make the first contact with the family to determine whether they are interested in participating in the study. Please see Appendix C for all IRB approved advertisements.

B3) Enrollment

When a family indicates that they would like to enroll in the study, they will be contacted by the study coordinator. The study coordinator will obtain the parent or guardian's verbal consent to participate over the phone. Once verbal consent is obtain, the participant can be randomized into a treatment group. Once the participant is randomized, the study coordinator will then schedule the baseline assessment and NCH-based treatment according to the group assignment. If possible, the baseline assessment should be scheduled at the Dr. Heathcock's pediatric laboratory at OSU to allow the study coordinator to obtain informed consent at that visit while maintaining the blinding of the assessor. If the baseline assessment must be scheduled at the patient's home and informed consent is not obtained prior to the baseline assessment, the study coordinator or research assistant must attend the

baseline assessment to obtain informed consent. Every effort will be made during that visit to maintain the blinding of the assessor.

B4) Treatment Scheduling

Once a family enrolls in the study and is randomized to a treatment group, the child's treatment sessions can be scheduled at NCH. The study coordinator will notify the lead therapists at NCH that the patient is ready to schedule and provide the scheduling preferences. The lead therapists will offer open treatment times, which the study coordinator will confirm with the family. Once confirmed, the lead therapists will schedule the treatment sessions in Epic (electronic medical record) and mail a welcome packet to the family that contains a treatment calendar. Once treatment is scheduled, the study coordinator will schedule the baseline assessment to coordinate with the start of treatment.

Each patient must have an active physician referral and physical therapy evaluation on file. If the patient currently receives physical therapy at NCH, these items are already on file. However, if the patient has not received physical therapy at NCH recently, they will need to be obtained. The lead therapists will request the referral from the patient's physician. This referral can be faxed to the front desk at 614-722-6746. The lead therapists will notify the front desk that this referral is expected, and the front desk staff will be able to add it to the patient's chart. Additionally, a copy of the baseline assessment results should be faxed and added to the chart to serve as an outside evaluation. The study coordinator and lead therapists will coordinate to ensure these needs are met.

B5) Data Management

All data from the OEs, PTCs, and treatment sessions will be entered into a Research Electronic Data Capture (REDCap) database. The REDCap database will be housed at OSU. The data will be entered into the database by the study coordinator and OSU research assistants. All paper copies of the assessment forms will be kept in a locked cabinet at Dr. Heathcock's pediatric laboratory at OSU.

C. PTC and OE Procedures

C1) Scheduling

If informed consent is not obtained prior to the baseline evaluation, the baseline evaluation should be scheduled at Dr. Heathcock's pediatric laboratory at OSU if possible for the family to allow the study coordinator or research assistants to obtain informed consent. All other outcome evaluation (OE) and physical therapy consultation (PTC) visits can be scheduled at the participant's home or at the OSU laboratory depending on the family's preference. OE visits will be performed at baseline and at the following intervals after the start of treatment: 9 months, 12 month, and 18 months. Eight monthly PTC visits will be scheduled, with the first PTC scheduled 4-5 weeks after the start of treatment.

C2) Outcome Evaluations

Assessments performed at each Outcome Evaluation (OE) will be completed by a blinded physical therapist. All assessments will be videotaped by one or two video cameras. Assessments used during OE's are listed below and described in greater detail in section E.

- Hollingshead Four-Factor Index of Socioeconomic Status (baseline assessment only)

- Gross Motor Function Measure-88 – All Dimensions (with scores also reported for Gross Motor Function Measure-66)
- Bayley Scales of Infant and Toddler Development, 3rd edition: Cognitive, Receptive Communication, Expressive Communication, Fine Motor, Gross Motor, Social-Emotional, and Adaptive Behavior Questionnaire (when developmentally appropriate)
- I-PAS play time)
- Pediatric Evaluation of Disability Inventory- Computer Adaptive Test (PEDI-CAT) (0, 9 month assessments)
- Goal Attainment Scale rating (9, 12 and 18 month assessments)
- Pediatric Outcomes Data Collection Instrument (PODCI) (0, 9 month assessments)
- Manual Ability Classification Scale (MACS) or mini-MACS (0, 9, and 18 month assessments)
- Communication Function Classification Scale (CFCS) (0, 9, and 18 month assessments)
- Parent Partnership Scale (9 month assessment only)
- Therapy Record Form (0, 9, and 18 month assessments)

C3) Physical Therapy Consultation

C3.1 Assessments

Assessments performed at each physical therapy consultation (PTC) will be completed by a clinical research physical therapist. All assessments will be videotaped by one video camera. Assessments completed at each PTC are listed below and described in greater detail in section E.

- GMFM-88-all dimensions (PTC 3 and 6)
- GMFM-88 one dimension, as determined by research PT at baseline assessment (PTC 1, 2, 4, 5, 7, 8)
- GAS rating
- Patient engagement
- Parent engagement in learning HEP
- Parent engagement with child
- Therapist effectiveness during PTC
- Compliance with HEP

Additional assessments completed at PTC 3 include:

- PODCI
- MACS/mini-MACS
- CFCS
- PEDI-CAT
- Parent Partnership Scale

C3.2 Home Exercise Program Review

Each patient will have a personalized home exercise program (HEP) that will be reviewed at each PTC. During each PTC, the research physical therapist will review the program with the parent or guardian and update the patient's HEP with progressive activities as needed. The research physical therapist will print a copy out for reference and to give to the parent at each PTC. The research physical therapist will review the activities in the HEP with the parent and demonstrate each activity with the patient. The HEP review is video-recorded.

C4) Home Exercise Program

Each patient will have a personalized HEP that will be reviewed at each PTC. The lead treating therapist at each site will confer with the assessing/research physical therapist on appropriate home exercises for the patient to complete at home. The research physical therapist will design the home program. The research physical therapist will print a copy out for reference and to give to the parent at each PTC. The research physical therapist will review the activities in the HEP with the parent and demonstrate each activity with the patient. The HEP review is video-recorded. The parent will be provided with daily tracking sheets to record time spent on each activity as well as the child's participation and alertness with each activity. Parents are instructed to return the tracking sheets to the PT at the following PTC.

D. Treatment Procedures

D1) Participant Schedules

Participants will be enrolled in one of two treatment groups, usual weekly or high intensity periodic. The treatment phase is 40 weeks long. Each group receives 40 total hours of therapy. The treatment schedule for each group is as follows:

- a. 1 hour per day, 1 x per week for 40 weeks
- b. Two bouts of 2 hour per day, 5 x per week for 2 weeks with an 18 week break between bouts

D2) Goal Setting

Family and child centered care is central to our clinical research and pediatric therapy. Activity and participation goals will be established with the families during the program, reflecting what the families and clinician consider meaningful and relevant. Regardless of group assignment, the functional aspects of each goal will be a primary component of the intervention during each therapy session. Goals will be set by the treatment physical therapist at the first treatment visit. These goals will then be sent to Rachel Ferrante and/or Elizabeth Maus who will establish Goal Attainment Scaling (GAS) for each goal. The GAS will then be sent to the research physical therapist at OSU who will rate each goal at OE and PTC visits. The treatment physical therapists will comment on progress toward goals in their visit documentation at least weekly during the treatment phase. All goals and progress comments will be written using the following definition of assistance levels:

- Independent: patient contributes 100% of effort to complete task and skill begins to serve a functional purpose.
- Contact Guard Assist (CGA): patient contributes 100% of effort to complete task but requires occasional contact for safety; functional purpose of the skill is emerging, but not yet consistent.
- Minimum Assist (MIN A): patient contributes 75-99% of effort to complete task.
- Moderate Assist (MOD A): patient contributes 25-74% of effort to complete task.
- Maximum Assist (MAX A): patient contributes 1-24% of effort to complete task.
- Dependent: patient contributes 0% of effort to complete task.

D3) Treatment Methods

The procedure for treatment sessions will vary between participants due to the variable nature of each child's motor ability and goals; however, each session will address certain targeted skills that will then be personalized to meet each child's needs. These targeted skills include:

increased independence with functional gross motor positions, weight bearing transitions, mobility and locomotor skills. Each session will address mobility on a motor level, but for some children, it may be appropriate to also address mobility on a cognitive level, using assistive technology. Based on the child's baseline skills and progress throughout the program, certain variations of the targeted skills may be necessary and beneficial to treatment. Variations may include modifying the surface, available supports, and/or activity to address the child's goals. See Table 1 for details on each of the targeted skills and possible variations. Toys will be used as motivators to encourage participants to perform the treatment activities. Table 2 contains a list of common toys that will be used during treatment sessions, as well as possible skills that can be addressed using the toy and example treatment activities. Also see Figure 2 for a list of standard equipment that will be available for use at each treatment session.

Table 1. Targeted Skills

<u>Targeted Skills</u>	<u>Types</u>	<u>Variations</u>
Positions	<ul style="list-style-type: none"> • Sitting • Quadruped • Kneeling • Standing • Single limb stance 	<ul style="list-style-type: none"> • With/without upper extremity support • Address balance during reaching and dynamic activities • Modified with benches or wedges for support • Use of dynamic surface for upper or lower extremity weight bearing • Maintain balance with/without object manipulation
Weightbearing Transitions	<ul style="list-style-type: none"> • Rolling • In/Out Sitting • In/Out Quadruped • In/Out Kneeling • In/Out Standing 	<ul style="list-style-type: none"> • Modified with benches, wedges, or bolsters for support • Part practice: practice "steps" of transition separately
Mobility - Motor Level	<ul style="list-style-type: none"> • Rolling • Crawling • Creeping • Walking: with/without assistive device • Walking on unlevel surfaces and/or stairs • Curb steps • Navigating obstacles 	<ul style="list-style-type: none"> • Use a large wedge for gravity assist • Use different surface to increase or decrease difficulty. A smooth and stable surface is less difficult for mobility than an unstable surface. • Use of narrow path for narrow base of support • Varying height of steps and obstacles
Locomotor skills	<ul style="list-style-type: none"> • Jumping and hopping • Climbing • Pedaling a bicycle or tricycle 	<ul style="list-style-type: none"> • With faded physical assistance • Stable surface may be easier for climbing, springy surface may be easier for jumping/hopping • Assist with steering so patient can focus on pedaling

		<ul style="list-style-type: none"> • Combine locomotor and balance skills targeting random practice • Adapt pedals to secure foot placement
Mobility - Cognitive Level	<ul style="list-style-type: none"> • Power mobility: Go Baby Go Early Power Mobility with switch activation 	<ul style="list-style-type: none"> • Add visual cue on switch • Assist with steering so patient can focus on activating switch

Table 2. Examples of Common Toy Motivators to Address Targeted Skills

<u>Toy</u>	<u>Targeted Skill</u>	<u>Example Activity</u>
Gumball machine	<ul style="list-style-type: none"> • Sitting balance • Quadruped balance • Reaching for transitions 	Ring sitting balance: Position the child in ring sitting on the floor with the gumball machine in front. Place the ball slightly outside of her reach. Encourage her to get a ball, then return to upright sitting to place it in the toy. Repeat activity 5-10 times, placing the ball in a different place each time to encourage reaching to each side.
Star Stacker	<ul style="list-style-type: none"> • Sitting balance • Kneeling or standing at bench • Sit to stand • Pull to stand • Floor transitions 	Sidelying to sit transition: Position the child in sidelying on the floor mat with the stars near his hands. Place the star stacker near his feet. Encourage her to pick up a star, then push into a sitting position to place it on the stacker.
Snail Toy	<ul style="list-style-type: none"> • Rolling • Prone activities 	Rolling: Position the child in supine on the floor mat. Place the snail toy beside her and within reach. Draw her attention to the toy, then start to roll it around the child above her head. Encourage her to roll into prone as she looks at and reaches for the toy.
Play Food	<ul style="list-style-type: none"> • Sit to stand • Standing endurance • Squat to stand 	Squat to stand: Position the child in standing in front of a bench for upper extremity support. Place toy bowls and spoons on the bench and play food on the floor. Encourage him to squat to retrieve a piece of food from the floor, then stand up to place it in the bowl and “cook.” Repeat the activity 5-10 times.
Squigz	<ul style="list-style-type: none"> • Sit to stand at vertical, horizontal, or unstable (therapy ball) surface • Standing endurance and standing balance at vertical, horizontal, or unstable (therapy ball) surface 	Bench sit to stand at wall mirror: Position child in a sitting position on a bench with knees and hips at 90 degrees in front of the mirror. Instruct the child to stand up and pull a squig off the mirror, then sit down. Repeat the activity 5-10 times.

	<ul style="list-style-type: none"> • Squat to stand with varying levels of support 	
Musical puzzle	<ul style="list-style-type: none"> • Climbing stairs with targeted step pattern • Climbing curb steps • Navigating obstacle course • Climbing to top of rope ladder, slide or play structure • Jumping/hopping forward for distance or over obstacle 	Stair climbing: Place puzzle board at top of stair case. Place pieces at bottom of stair case. Instruct the child to pick up a puzzle piece and take it to the top of the stairs. Instruct the child in targeted step pattern/support use (reciprocal/nonreciprocal steps, hands free/handrail). Assist the child with faded tactile and verbal cues. Perform 8-10 times to complete puzzle.
Basketball hoop with weighted or unweighted balls	<ul style="list-style-type: none"> • Step ups with/without added weight • Sit to stand with/without added weight • Jumping/hopping forward for distance or over obstacle 	Curb step: Place curb step of 2-10" in front of basketball hoop. Place ball in front of curb step. Instruct child to pick up ball and climb up step to place in hoop. Instruct child to step down from curb to retrieve ball and repeat activity 10-15 times. Assist child with faded tactile and verbal cues. Vary environment with height of curb step, use of several weighted balls of different mass and size. Rearrange hoop and ball for child to hold ball atop curb step and step down in order to approach basketball hoop. Repeat 10-15 times.

Figure 3. Standard Equipment

- Floor mat
- Wall mirror for feedback
- Adjustable height benches: 7'-11", 10.5"-17", and 16"-23"
- Bolsters: 6", 8" and, 16"
- Wedges: 6" and 10"
- Therapy balls: peanut and round of various sizes
- Gait trainers: Rifton Pacer, R82 Mustang
- Reverse rolling walker
- Scooters
- Go Baby Go Early Power Mobility Devices
- Weighted Pads/Ankle Weights
- Gait belt
- Stair case with hand rail and at least 4 steps
- Curb steps: 2-10"
- Weighted balls such as medicine balls, Yuck-E-Ball balls (0.5-10 lbs)

D4) Heart Rate Monitoring, Videotaping, and Patient Engagement During Therapy Sessions

NCH treatment therapists will perform heart rate monitoring and videotaping at every 10th hour of treatment to measure intensity and fatigue and the videotaped sessions will be used to measure patient engagement via observations described at the end of this section. The 10th hour occurs every 10th visit for usual therapy group and the first hour of 5th and 10th sessions during intensive bouts. Heart rate will be measured at the start of the session, halfway through

(30 minutes for usual therapy group, 60 minutes for high intensity periodic group) session, and at the end of the session. Heart rate will be measured using a fingertip pulse oximeter which will be available at each treatment site.

The videos of therapists treating ACHIEVE patients will be used for treatment fidelity checks using a standardized treatment fidelity assessment tool developed by the NIH's Behavioral Change Consortium. Only therapists with demonstrated treatment fidelity and subsequent reliability checks will provide treatment to patients enrolled in ACHIEVE.

After completion of a videotaped session, the site lead therapist will upload the treatment video file(s) onto a secured storage drive within the NCH network. Video files should be labeled according to participant ID, treatment visit number, video file number, and date. For example: AC002's 10th hour of therapy on 05/13/16 (includes 2 videos) would be labeled as follows:
AC002_TH10_1_2016.05.13
AC002_TH10_2_2016.05.13

A blinded therapist will view the recorded therapy sessions and complete a Patient Engagement Measure. This measure includes ratings in the following categories:

1. Activity level (high, medium, low, none)
2. Behavioral state (awake and active, awake and quiet, drowsy, fussy/crying)
3. Facial expression (smiling, neutral/engaged, neutral/flat, frowning)
4. Temperament (presence or absence of: self-regulation, consolability, positive affect, persistence)

These engagement categories are also rated as part of measuring engagement during the patient's home exercise program (see E13)

Appendix D for a copy of Engagement Forms

D5) Treatment Logs

NCH treatment therapists will keep a log of ACHIEVE treatment visits to allow data to be transferred to REDCap. The treatment logs will include the following information about each visit: treatment therapist's name, session number, date, percentage of treatment time spent on each skill, heart rate if applicable, whether the session was videotaped, and a comment section for any additional information. The lead research therapist at each site will provide a treatment log for each patient that will be kept at a secured location at the site. The lead therapist should monitor the treatment log to ensure it is completed accurately.

D6) Documentation

A "smartphrase" documentation template will be available in the NCH electronic medical record, Epic, to ensure complete documentation at each treatment session. The visit documentation should include the percentage of time spent on each skill, adding up to 100%, and the patient's heart rate if it is a visit in which heart rate is recorded. Skill categories include: Supine, Sitting, Rolling, Prone, Standing, Transitions, Ambulation, Fxn'l Navigation, Balance, Jump/Hopping, Coordination. A comment regarding progress toward each goal should be made at least weekly regardless of group assignment.

E. Tests and Measures

E1) Gross Motor Function Measure (GMFM)

The GMFM is a criterion-referenced, evaluative assessment tool that measures change in gross motor skills over time or following an intervention. Five dimensions are assessed, including: "Lying & Rolling," "Sitting," "Crawling & Kneeling," "Standing," and "Walking, Running, & Jumping." The GMFM-88 is the original assessment that includes 88 items. The GMFM-66 includes 66 items that are a subset of the original 88 items. Each item uses a four-point scoring system (0-3 points). This assessment can be used for children from 5 months to 16 years of age. The GMFM-88 will administered with scores reported for both the GMFM-88 and GMFM-66.

- Administration - Administration time is approximately 45-60 minutes. The GMFM-88 has been validated for use for children with cerebral palsy and Down syndrome. A raw score and percentage score are calculated for each of the five dimensions. Total raw score and total percentage score are also calculated. Because scores will also be reported for the GMFM-66, testing is completed without aids or orthoses.
- Scoring – The GMFM-88 raw score and percentage score are calculated for each of the five dimensions. The dimension raw score is the sum of all points received in that dimension. The dimension percentage score is calculated by dividing the dimension raw score by the total possible points for that dimension and multiplying by 100. Total raw score and total percentage score are also calculated. The total raw score is the sum of the raw scores from all five dimensions. The total percentage score is the average of the percentage scores from all five dimensions (i.e. (%A + %B + %C + %D + %E) divided by 5). Calculating a GMFM-66 total score and standard error of measurement (as well as change scores) requires the use of GMAE-2 software, which is available to the research physical therapists for scoring purposes.
- Equipment: smooth/firm floor, two straight lines (8 inch apart and 20 feet long), straight line (¾ inch wide and 20 feet long), circle (24 inch diameter) marked on floor, large firm exercise mat, small toys, small bench, large bench, stopwatch, 12-24 inch long stick, other toys as motivators, a large object that must be carried with two hands, five steps with railing, a stool on castors. Additionally, a comfortable space at least 15 feet for running is required.

E2) Bayley Scales of Infant and Toddler Development - Third Edition (Bayley-III)

The Bayley-III is a norm-referenced assessment tool. There are five main subtests, including: cognitive, language (receptive communication and expressive communication), motor (fine motor and gross motor), social-emotional, and adaptive behavior. For the purposes of the current research, the cognitive, receptive communication, expressive communication, fine motor, and gross motor subtests will be used. The Bayley-III can be used for children 1 month to 42 months of age. While it is designed as a discriminative tool that is used for identifying delays, it has been used historically to chart progress over time over the course of an intervention program. For the purposes of the current research, the Bayley-III will be used as an evaluative tool to measure change over time.

- Administration and scoring: Administration time is approximately 50-90 minutes depending on the child's age. A raw score, scaled score, and age equivalent score are calculated for each of the five subtests. A composite score and percentile rank is calculated for the cognitive, language (receptive communication and expressive communication), and motor (fine motor and gross motor) sections. A scaled score of 8-12 is considered to be within the average range for the child's age.

- Since the developmental age of a child with cerebral palsy may be lower than their chronological age, the Bayley-3 will continue to be administered for children older than 42 months, unless the child is clearly functioning beyond the ceiling of the test in all five domains.
- Equipment: Bayley kit, two washcloths, 5 small coins, food pellets (like Cheerios), safety scissors, three steps (standard size), stopwatch, blank paper

E3) Goal Attainment Scaling (GAS)

GAS is a personalized tool that allows for goals to be defined at different levels of mastery and assigned numerical values for score calculation, similar to a Likert scale. The scale has 5 points representing different levels of mastery of the individual patient's goal. A score of -2 represents baseline, -1 less change than expected, 0 for the expected level of change, and +1 and +2 for achievement of more change than expected. Only a single dimension of change should be described at each level of the scale to ensure ordinality.

- Administration and scoring: The treating therapist will establish three goals at the start of treatment. Two of the goals will be written at the activity of the ICF, and one goal will be written at the participation level of the ICF. The treating therapist will then send the goals to Dr. Carey who will establish the scale. The patient's progress on the scale will be assessed at each PTC visit and at all OE visits after baseline.
- Equipment: none

E4) Pediatric Evaluation of Disability Inventory-Computer Adaptive Test (PEDI-CAT)

The Pediatric Evaluation of Disability Inventory-Computer Adaptive Test (PEDI-CAT) is a revised version of the PEDI used for children from birth to 21 years old with any physical, behavioral, and/or cognitive conditions. The PEDI-CAT measures functional abilities across four domains: Daily Activities, Mobility (including a wheelchair subdomain), Social/Cognitive, and Responsibility. Computer adaptive testing technology individualizes the assessment to the individual child, based on the user's responses to previous assessment items regarding the child's ability level. The PEDI-CAT has two versions, A Speedy CAT with 15 questions per domain and a Content-Balanced or "Comprehensive" CAT with about 30 questions per domain. Both versions have been shown to produce scores within the margin of error (determined by standard error).

- Administration and scoring: The PEDI-CAT is completed by a parent using a computer or iPad with PEDI-CAT software. It takes approximately 15 minutes to complete. Raw scores are converted to norm referenced scores and criterion-reference (scaled) scores. Scores and individual items answers can be stored and/or exported for further interpretation.
- Equipment: PEDI-CAT software for iPad or computer, PEDI-CAT manual

E5) Infant-Preschool Play Assessment Scale (I-PAS)

The I-PAS is a criterion-referenced assessment for children from birth to 5 years of age. It will be used to measure spontaneous play.

- Administration and scoring: The I-PAS will be scored from the videotaped GMFM-88 and Bayley-III assessments taken at the assessment visits.
- Equipment: none

E6) Heart Rate

Heart rate monitoring will be performed using a pediatric pulse oximeter. It will be measured every tenth hour of treatment at the beginning, middle, and end of the session. The purpose of

heart rate monitoring is to ensure the patients' heart rates are in a safe range throughout all phases of the treatment session in all three dosing parameters. The expected heart rate range based on age according to Fleming et al.⁴ is as follows:

- 2-3 years old – 76-142 beats per minute (bpm)
- 3-4 years old– 70-136 bpm
- 4-6 years old– 65-131 bpm
- 6-8 years old– 59-123 bpm

E7) Hollingshead Four-Factor Index of Socioeconomic Status

The Hollingshead Four-Factor Index collects information on demographics such as education, occupation, income, marital status, and race. This purpose of including this index is to ensure that the ACHIEVE study is recruiting from all socioeconomic levels. This is completed by the assessing physical therapist as an interview with the parent at the baseline assessment only. Answers should be recorded on the score form and calculated and compared to occupational scale scores in the testing manual.

E8) Pediatric Outcomes Data Collection Instrument (PODCI)

The Pediatric Outcomes Data Collection Instrument (PODCI)- Pediatric form is valid for children ages 2-11 years and is completed by the child's parent. Separate forms are used for adolescents and/or parents of adolescents ages 12-18 years. The PODCI assessed function in four areas: Upper Extremity Function, Transfers and Basic Mobility, Sports and Physical Function, and Comfort/Pain. Global Function score combines the four functional assessments with a Happiness with Physical Condition score. Scores from the paper survey are entered into an Excel spreadsheet to calculate mean and normative scores. The PODCI is for use in a variety of musculoskeletal conditions and is not specific to cerebral palsy.

E9) Manual Ability Classification System (MACS and mini-MACS)

The Manual Ability Classification System (MACS) for Children with Cerebral Palsy ages 4-18 of age (mini-MACS for children 1-4 years of age) classifies how children with cerebral palsy use their hands to handle objects in daily activities. It includes five levels. Per the MACS user guide, these levels are based on the child's self-initiated ability to handle objects and their need for assistance or adaptation. This is determined through parent/caregiver interview. Refer to the user guide for level description.

E10) Communication Function Classification System (CFCS)

The Communication Function Classification System (CFCS) is used to classify everyday communication performance of the child into 5 levels. Per the CFCS user guide, the overall effectiveness of the communication performance should be based on how the child usually takes part in everyday situations requiring communication, rather than their best capacity. The level can be determined through observation during the assessment and by parent/caregiver interview. Refer to the user guide and decision tree for specific level descriptions.

E11) Parent Partnership Scale (PPS)

The Parent Partnership Survey is a survey completed by the patient's parent or guardian. It assesses parents' satisfaction with the physical therapy services they receive as a part of the ACHIEVE study.

E12) Therapy Record Form

The therapy record is a means to track all physical, occupational, and speech therapy services as well as recreational physical activities (such as swimming, gymnastics, hippotherapy, etc) before and during the study enrollment period. Therapy record forms are filled out by the parent/guardian at baseline, 9 month and 18 month outcome evaluations.

E13) Engagement During Home Exercise Program

Engagement during the home exercise program will be measured in several ways.

- a. Therapists' effectiveness at training parent participation in home program instruction (completely ineffective, more ineffective than effective, more effective than ineffective, completely effective) scored by blinded therapist from video
- b. Parental engagement in learning and demonstrating home program activities during the all PTCs (low, occasional, moderate, high) scored by therapist during PTC
- a. Parent comfort in completing the home program activities (completely uncomfortable, somewhat uncomfortable, somewhat comfortable, completely comfortable) reported by parent during PTC
- b. Parent engagement with the child during home program activities during demonstration (low, occasional, moderate, high) scored by blinded therapist from video
- c. Patient Engagement scored by blinded therapists from video
 - a. Activity level (high, medium, low, none)
 - b. Behavioral state (awake and active, awake and quiet, drowsy, fussy/crying)
 - c. Facial expression (smiling, neutral/engaged, neutral/flat, frowning)
 - d. Temperament (presence or absence of: self-regulation, consolability, positive affect, persistence)

E14) HEP Tracking

Tracking and compliance of the home exercise program (delivered by parents) will be measured with a "tracking sheet." A home program tracking sheet is a very important tool to help measure the amount of time spent at home completing structured gross motor activities. Parents fill out the tracking sheet. A home program is provided to each family to help carryover what is being done in each treatment session to the home environment. The tracking sheet will give us insight into how often the home exercise plan is being completed at home. It will also allow us to track the level of participation and alertness of the participant each time they complete the home program at home. An example of a home program and tracking sheet is presented below for a 2.5 year old child with more severe CP. A subset of participants will use a fitbit during their home exercise program. This will allow us an alternative way to measure activity and participation in a home program. Dr. Heathcock (PI) has recent experience using a fitbit to measure activity during a parent-delivered intervention for children with CP and will continue to develop this tool for ACHIEVE. In brief, this device is time-synced to a research-only encrypted cell phone allowing for accurate and private tracking of activity. This may give an objective measure of time the child was active and can be correlated to the tracking sheet.

Table 3. Schedule of Tests and Measures

Visit Type	Tests and Measures
Baseline	GMFM-88 (full), Bayley-III, PEDI-CAT, I-PAS, Hollingshead Index, PODCI, MACS/mini-MACS, CFCS, Therapy Record Form

PTC 1	GMFM-88 (two dimensions), GAS, Parental engagement in HEP, Parent comfort HEP, HEP tracking sheet
PTC 2	GMFM-88 (two dimensions), GAS, Parental engagement in HEP, Parent comfort HEP, HEP tracking sheet
PTC 3	GMFM-88 (full), GAS, Parental engagement in HEP, Parent comfort HEP, HEP tracking sheet, PEDI-CAT, PODCI, mini-MACS, CFCS, Parent Partnership Scale
PTC 4	GMFM-88 (two dimensions), GAS, Parental engagement in HEP, Parent comfort HEP, HEP tracking sheet
PTC 5	GMFM-88 (two dimensions), GAS, Parental engagement in HEP, Parent comfort HEP, HEP tracking sheet
PTC 6	GMFM-88 (full), GAS, Parental engagement in HEP, Parent comfort HEP, HEP tracking sheet
PTC 7	GMFM-88 (two dimensions), GAS, Parental engagement in HEP, Parent comfort HEP, HEP tracking sheet
PTC 8	GMFM-88 (two dimensions) GAS, Parental engagement in HEP, Parent comfort HEP, HEP tracking sheet
9 month OE	GMFM-88 (full), Bayley-III, PEDI-CAT, I-PAS, PODCI, GAS, MACS/mini-MACS, CFCS, Parent Partnership Scale, Therapy Record Form
12 month OE	GMFM-88 (full), Bayley-III, PEDI-CAT, I-PAS, GAS
18 month OE	GMFM-88 (full), Bayley-III, PEDI-CAT, I-PAS, GAS, MACS/mini-MACS, CFCS, Therapy Record Form

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