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Protocol of the CHILD-BRIGHT READYorNot™ Brain-Based Disabilities Trial: An RCT investigating the effectiveness of a patient-facing e-health intervention designed to enhance health care transition readiness in youth.

Journal:	<i>BMJ Open</i>
Manuscript ID	bmjopen-2021-048756
Article Type:	Protocol
Date Submitted by the Author:	06-Jan-2021
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Keywords:	Clinical trials < THERAPEUTICS, Developmental neurology & neurodisability < PAEDIATRICS, REHABILITATION MEDICINE

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Title: Protocol of the CHILD-BRIGHT READYorNot™ Brain-Based Disabilities Trial: An RCT investigating the effectiveness of a patient-facing e-health intervention designed to enhance health care transition readiness in youth.

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Strengths and limitations of this study

- This study takes a user-centred approach, using a patient-facing e-health intervention to engage with youth and improve transition readiness for individuals with brain-based disabilities (BBD) transitioning to adult health care.
- The embedded mixed method randomized controlled trial (RCT) design is ideal to answer complex research questions and provides stronger and richer evidence than a single method alone.
- The generalizability of the study findings may be limited to youth with BBD who have started to take charge of their own health.
- Prior to study recruitment, we proactively adapted our study methods in response to the COVID-19 pandemic.
- Strong patient and family involvement at all stages of the study focuses on improving the lived experiences of youth with BBD and their families.

INTRODUCTION

With health care innovations, more and more people with pediatric-onset disabilities are surviving into adulthood, thus increasing the need for proactive care strategies for this growing cohort [1]. The process of transition to adulthood can be challenging, as youth with brain-based disabilities (BBD) and their families move from familiar pediatric health care services and learn how to navigate new adult services. In many jurisdictions in Canada, as elsewhere, the transfer from pediatric to adult health care services is set by policy and occurs regardless of whether the youth is ready for transition of care. Poor health care transition can have negative health outcomes and result in poor quality of life for youth with BBD, such as autism spectrum disorder cerebral palsy, epilepsy, fetal alcohol spectrum disorder and spina bifida. Lack of access to health care services can result in the increased use of high-cost health care services, increased emergency department visits, family burden, and exacerbated health issues [2–5]. In Canada, this transfer of care typically occurs at age 18. Since the policy-driven age of transfer cannot be changed, we designed the READYorNot™ (READiness in Youth fOR traNsition Out of pediaTric care) Brain-Based Disabilities Trial to evaluate a patient-facing e-health intervention aimed at fostering self-management and self-advocacy skills in youth with BBD to improve their readiness for adult health care.

Youth with BBD are expected to be prepared for health care transfer by developing the knowledge and skills to manage their health condition. Transition is defined as “a purposeful, planned process that addresses the medical, psychosocial and educational/vocational needs of adolescents and young adults with chronic physical and medical conditions as they move from child-centered to adult-oriented health care systems” [6]. The field of transition of care has grown over the past few decades, with several calls-to-action to improve processes of transition and to develop interventions and resources with a vision to maximize lifelong functioning through uninterrupted health care services as individuals move from adolescence to adulthood [7–9].

The use of information technology, such as eHealth interventions and applications “Apps”, is an appealing, accessible and flexible way to engage youth with BBD and their families. Patient engagement via information technology has been shown to directly impact patient behaviour in a way that promotes positive health outcomes, patient satisfaction, care delivery efficiency, reduces costs and improves quality of care and patient safety [10,11]. We developed the MyREADY Transition™ BBD App to improve transition readiness of youth with BBD. This App was co-created with researchers, health care professionals, technology designers, youth and families working in partnership.

Objectives

The aim of this study is to determine whether the MyREADY Transition™ BBD App intervention will result in greater transition readiness compared to usual care for youth with BBD between 15 and 17 years of age. We hypothesize that youth who receive their usual care and use the MyREADY Transition™ BBD App intervention will have higher self-management change scores over a 6-month period compared to youth who receive their usual care. The secondary aims of the study include exploring the contextual experiences of youth using the App, as well as the interactive processes of youth, their parents/caregivers and health care providers around use of the intervention. We also aim to explore health economic outcomes by comparing the

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3 incremental cost of the intervention compared to current standard of care per unit of
4 effectiveness.
5

6 ***Design***

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8 This protocol paper describes a randomized controlled trial (RCT) using a mixed methods study
9 design. The RCT will use a pragmatic approach to test whether the intervention works under
10 usual conditions. Specifically, we will use an embedded experimental model design [12], which
11 will involve embedding a qualitative component within the RCT. See Figure 1. Our selection of
12 outcome metrics and the comprehensive design to evaluate this intervention was guided by the
13 Institute for Health Care Improvement (IHI)'s Triple Aim framework (better health, better
14 experience, at lower cost).
15

16 ***Patient and public involvement***

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18 The Canadian Institutes of Health Research's Strategy for Patient-Oriented Research (SPOR)
19 endorses that patients and parents/caregivers with "lived experiences," together with health
20 professionals and decision makers, join researchers as members of the research team. Since the
21 very beginning, we have engaged a core group of youth, parents/caregivers and health care
22 stakeholders, including a Patient and Family Advisory Council (PFAC), composed of
23 representative youth (adolescents and young adults) and parents/caregivers. We designed our
24 study in collaboration with patients and families and health care professionals. Our partnership
25 with the PFAC will continue throughout the execution of our RCT, thus enhancing participant
26 recruitment, data collection, engagement planning, and Knowledge Translation (KT). In the KT
27 stage of the project we will work together on novel and meaningful ways to share the study
28 findings about potential benefits of the App.
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31 **METHODS**

32 ***Study setting***

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35 READYorNot™ Brain-Based Disabilities Trial is a patient-oriented project of CHILD-BRIGHT
36 (Child Health Initiatives Limiting Disability-Brain Research Improving Growth and Health
37 Trajectories), which is a pan-Canadian research program. A large cohort of youth with BBD
38 living in Alberta, Ontario, Quebec, and the Maritimes in Canada will be recruited.
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41 ***Eligibility Criteria***

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43 The following are study inclusion criteria: 1) age of 15 to 17; 2) a diagnosis of autism spectrum
44 disorder, cerebral palsy, epilepsy, fetal alcohol spectrum disorder, or spina bifida; 3) in pediatric
45 care in one of the four study regions and for whom a discharge from pediatric care is planned but
46 not for at least 6 months; 4) cognitive ability to provide informed consent and the ability to read
47 and understand English or French; 5) access to internet and a smartphone, iPad/tablet or desktop
48 computer; and 6) TRANSITION-Q [13] score >40 (as a screen to define a minimum threshold
49 for transition readiness based on our earlier work). In a validation sample of Ontario youth aged
50 12 -25 years with chronic health conditions, including our target population, the average
51 TRANSITION-Q score for 13 year olds was 40; for 15 year olds was 53; and for 17 year olds
52 was 59 [14]. The decision to set the threshold at > 40 aligns with clinical judgement and is
53 conservative given that the youngest age group in the validation sample demonstrated this
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3 minimum level of readiness. The TRANSITION-Q screen will inherently not include youth who
4 have severe intellectual disability, and/or those who rely significantly on parents/caregivers in
5 most areas of daily functioning, self-care and/or communication.

6
7 Individuals will be excluded if they are: 1) in “acute crisis” with unstable physical or
8 mental health that would interfere with the ability to participate in the study; 2) have sensory
9 impairments, such as uncorrected vision or hearing loss, which would interfere with the use of
10 the App; or 3) are enrolled in a potentially confounding trial (e.g., a different transition
11 intervention study).
12

13 ***Intervention***

14 The MyREADY Transition™ BBD App is designed to educate and empower youth with BBD as
15 they prepare for transitioning from pediatric to adult care. We collaborated with the PFAC to
16 inform the design and help tailor the App to incorporate features that were meaningful to them.
17 The App is constructed as a “Journey in the City” with a mentor (virtual coach) that helps the
18 user navigate the buildings and sequentially introduces the educational sections. The content of
19 the App is composed of messages, texts, quizzes, videos, and skill-based-achievement
20 challenges. The App uses pop-up features to manage reminders aimed at keeping engagement
21 and ensuring adherence to the App. The App has 19 visits organized into 5 ordered chapters. In
22 terms of exposure time, there is ‘planned’ flexibility to allow participants to proceed through the
23 MyREADY Transition™ BBD App intervention at their own pace. There is a timer in the App to
24 inform participants when the next visit is unlocked. Participants will receive instructions to wait
25 at least one day between visits. This waiting period will allow participants to process and reflect
26 on the take-away message(s) in each visit and/or engage in one of the suggested between-session
27 practice activities. The waiting period will also moderate the pace and aligns with how young
28 people learn and digest information. There are approximately 5-7 hours of content within the
29 App. For the RCT, the recommended exposure to the App intervention is between one visit per
30 day (19 days) and one visit per week (19 weeks). Games and fun activities are incorporated to
31 encourage youth to explore the App between visits.
32

33 For participants randomized into the intervention group, the research assistant (RA) will
34 help them download the App on their device. To ensure that the participant understands how to
35 access the App’s features, they will watch an introduction video demonstrating the first visit of
36 the App and they will be given a reference handout with tips and strategies for using the App.
37 Participants will also receive a website link for App support, including support for download,
38 access to the introduction video, a list of Frequently Asked Questions, as well as a series of short
39 how-to tutorial videos about different features in the App. The RCT Research Coordinator and
40 Research IT team will further support the use of the App and troubleshoot issues as they arise.
41 This will be done using a designated email to capture and respond to queries and will include an
42 automated response, indicating receipt and approximate response time. During study
43 participation, participants will receive reminders to promote the use of the MyREADY
44 Transition™ BBD App. Parents of youth in the intervention group and health care providers in
45 recruiting clinics will receive guidelines on how to support youth in the intervention group,
46 including an explanation that we want to know how youth are using the App independently,
47 suggestions about ways they can help youth use the App without influencing their use of the
48 App, and information about how youth can access App support.
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Usual care (intervention and control group)

Participants randomized into the control group will continue to get the same care they have been getting (their usual care). Youth participants in both the control and intervention groups, along with their parent/caregiver will receive a standard reference handout (Supplementary File 3) that will provide a basic overview of what they might expect as they get ready for transition. This handout ensures that all study participants have a minimum standard of preparation beyond the usual care they are receiving. Any support the youth (and parents/caregivers) receive as part of any ongoing transition programs in usual care will be documented by youth, parents/caregivers and health care providers. Documentation will include an inventory checklist with a section to add specific information relevant to the transition process. Participants and their parents/caregivers will also be asked what they perceive as supports and if they received these supports.

Outcomes

In this RCT, both quantitative and qualitative data types will be collected. All data collection forms will be available in both English and French. Detailed demographic information from parents/caregivers will be collected to understand and describe the different kinds of families participating in CHILD-BRIGHT studies and to compare this study sample to youth with BBD across Canada.

Measures.

The primary outcome is transition readiness which will be measured with the 29-item version of the Transition Readiness Assessment Questionnaire (TRAQ). The TRAQ has a Self-management domain (16 items) and a Self-advocacy domain (13 items) [15]. It is a validated, patient-centred questionnaire used in previous studies to measure transition readiness [16] and is designed to be self-administered at baseline and six months. Details about the TRAQ and other secondary outcome measures are provided in Supplementary File 4.

Secondary outcomes will evaluate whether the intervention has an effect on population health. All youth will be asked to complete the Canadian Occupational Performance Measure (COPM) [17], TRANSITION-Q [13], and PedsQL™ Pediatric Quality of Life Instrument, Generic Core, and Teen Report (13-18 years) [18]. To assess the impact of the App on the families' health care experience, parents/caregivers will complete the Measure of Process of Care (MPOC) (family-centred care) [19], and youth will be asked to complete the Newest Vital Sign [20] as a measure of their health literacy at baseline. Cost utility and cost-effectiveness will be measured using Health Utilities Index® (Hui2/3) Proxy-Assessed (health-related quality of life) [21] and Resource Use Questionnaire (RUQ) [22]. In addition, consent will be requested to obtain youth participants' Health Card Numbers to link information provided in the study with provincial health administrative data about use of health services, such as physician office visits, visits to emergency rooms and hospitalizations during the study period. Participants may choose to participate without sharing their Health Card information.

The MyREADY Transition™ BBD App will log user metrics which will be useful during the intervention (to monitor App use and provide support) and during the evaluation phases of the project (to quantify App use). The App will collect data related to end user login to the

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3 system (times and dates); visit/session completion and average time spent; number of clicks by
4 challenge and event type; access to games at the arcade; time spent on a visit/session and trends
5 over time. Within the App, users are asked to provide feedback at the end of each visit. They will
6 rate their experience on a three-point scale using emojis for happy, neutral and unhappy/sad.
7 Questions ask about experiences with the videos, quizzes, and challenges; the usefulness of the
8 content in the visit/session and will ask whether they completed the visit/session alone or with
9 help. The App will collect demographic data provided during the registration process and device
10 information, such as the type of device and its operating system. Participants in the intervention
11 group will also be asked to complete the System Usability Scale [23] at the 6-month visit. The
12 measure will focus on users' utilization of the App and its features, the perceived value,
13 experience, and satisfaction with the intervention.
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16 *Interviews.*

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18 As part of the embedded qualitative study, interviews will be conducted with a subset of 30
19 youth in the intervention group following their completion of the RCT outcome measures. The
20 purpose will be to understand youth perceptions of their transition readiness skills and awareness
21 after using the App, to understand how they may have used the App in their everyday lives and
22 in interactions with health care providers, and to understand how the App might have influenced
23 their care. Interviews will also be completed with approximately 10 parents and 10 health care
24 providers, who can share their perspectives on the potential of the App to improve transition
25 readiness.
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27 To complement quantitative findings related to App use, and for opportunities to improve
28 the App, a subset of approximately 20 youth will also be interviewed. They will be asked how
29 the App was used initially and over time, barriers and facilitators to its use, and participants'
30 experience and satisfaction with the App and its features. To capture process and user experience
31 we will conduct interviews at the end of intervention exposure or after 6 months, whichever
32 timepoint comes first. All interviews will be one-on-one, semi-structured, conducted over the
33 phone or by video-conference (e.g. Zoom), and will be audio-recorded.
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36 *Sample size*

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38 The primary sample will be comprised of 264 youth with BBD, aged 15 to 17 years, who are
39 recruited in one of the four study regions (Alberta, Ontario, Quebec, and Maritimes) and who are
40 at least 6 months pre-transfer to the adult health care system. The parent or caregiver of the youth
41 will also be recruited to the study. The study aims to have an equal number of participants in the
42 control and intervention groups. Randomization will be stratified by region with a 1:1 allocation
43 ratio for patients: 132 in the control group (continuing with usual care) and 132 in the
44 intervention group (continuing with usual care and receiving App intervention). The primary
45 outcome measure (Transition Readiness Assessment Questionnaire, TRAQ) has been validated
46 on a sample of Canadian youth with congenital heart disease [24] and, in the absence of literature
47 specific to BBD, our sample size calculation was based on these findings. We anticipate a mean
48 TRAQ self-management baseline score of 3.01 (SD 1.02) (out of a possible 5.0) as reported for
49 youth (with congenital heart disease) without a transition intervention and an anticipated mean
50 score of 4.0 at 6 months post-intervention resulting in a change score of 1 (i.e., 1 SD), with
51 $\alpha=0.05$ and 90% power. Therefore, in each region, 23 youth are required in each of the 2 arms x
52 4 regions = 184. With an estimated attrition rate of 30%, we will enroll a total of 264 participants
53 across the 4 regions.
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3 From the sample of 132 youth participants in the intervention group, we will purposefully
4 sample two subsets of participants for different study aims. A subset of approximately 30 youth
5 participants, as well as 10 parents/caregivers and 10 health care providers will be interviewed to
6 describe and understand the primary outcomes after the 6-month quantitative data collection
7 point. To address our secondary aim, a subset of approximately 20 youth participants will be
8 interviewed to capture process and user experience of the App at the end of exposure or after 6
9 months, whichever timepoint comes first.
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12 **Recruitment**

13 Before the onset of the study, we expanded our recruitment approach (Supplementary File 1) to
14 include strategies to facilitate the study's operations within the context of physical distancing
15 measures related to the COVID-19 pandemic [25,26]. First, clinicians in the patient's circle of
16 care at each clinic site in each of the four regions will approach eligible participants to ascertain
17 interest and obtain permission for the RA to contact them. They may obtain this permission in
18 person after physical distancing measures are lifted, by telephone or by mail. Recruitment
19 materials will also be shared on websites and social media. As a result, some participants may
20 wish to self-refer and contact the RA directly. The RAs will contact potential participants by
21 phone to complete a screening checklist and confirm eligibility. If there is a clinic appointment
22 scheduled, contact will be made in advance so that they have an opportunity to learn about the
23 study and ask questions. The consent and assent form will be sent to potential participants in
24 advance of the scheduled visit. Due to physical distancing measures, individuals can choose to
25 have the forms sent to them by email or by mail. Prior to collecting any data, informed consent
26 will be obtained. Youth will provide assent and a parent/caregiver will also provide consent both
27 for their child and for themselves. Again, to accommodate physical distancing measures, we
28 have added a telephone verbal consent procedure. Ideally, participants will complete the baseline
29 study visit at the same appointment time. If the latter is not possible, the RA will arrange another
30 study appointment convenient for both the youth and parent/caregiver.
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35 **RANDOMIZATION**

36 Randomization will be stratified by region with a 1:1 allocation ratio for participants: control
37 group who will continue with usual care, or intervention group who will continue with usual care
38 and receive the MyREADY Transition™ BBD App. The unit of randomization is the patient.
39 Variable block randomization will be used with block sizes of 2, 4, 6, and 8. Allocation will be
40 done via REDCap (Research Electronic Data Capture) [27]. Individuals who meet the eligibility
41 requirements at the point of screening, and who give consent to participate will be randomized
42 after the baseline questionnaires are completed. Participants allocated to the control or usual care
43 group will not know the specific details of the electronic intervention being offered to the
44 intervention group. Due to the nature of the intervention, participants cannot be blinded to group
45 allocation, however, outcome assessment and data analysis will be blinded.
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49 **PROCEDURES**

50 Prior to the start of the study, each study RA will complete e-learning module training provided
51 by the research team (Supplementary File 2). Procedural fidelity will be monitored.
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54 ***Data collection***

To track participants according to the CONSORT guidelines [28,29], a de-identified log of screened youth patients at all participating sites will be kept, recording inclusion/exclusion criteria and reasons for eligible youth patients not being recruited or randomized.

Participants will have the option to complete the questionnaire package electronically or in printed form. In case the baseline visit is not done in-person due to physical distancing measures, we have added the option to conduct the baseline visit via telephone or “Zoom” meeting (Zoom Video Communications, Inc.). To better establish rapport with participants, the RA may conduct the Zoom meeting with their own camera on [30,31]. Participants will have the option to turn on their camera or keep it off. Zoom meetings will not be recorded.

Internal pilot phase

The British Medical Research Council explicitly recommends the use of feasibility studies prior to Phase III clinical trials [32]. To guide the planning and to enhance the likelihood of success of our full scale RCT, the first three months of recruitment will comprise an internal pilot phase where study procedures will be observed and considerations will be taken into account about key implementation aspects, such as recruitment (including refusal rates and screening process), multi-site coordination/collaboration (including communication, documentation and provision of support) and intervention uptake and adherence (including technical support needs among App users). The results will be used to refine and enhance the research design. The RCT will proceed with procedural modifications based on the findings of the internal pilot study and final study analyses will incorporate all data. As long as the alpha-level is controlled, internal pilot designs have, at most, a small adverse effect on the significance level and may greatly improve the power [33].

ANALYSIS

A mixed methods approach will be used that combines both quantitative and qualitative research methods and techniques. This methodology is commonly used in patient-centred care research as both qualitative and quantitative methods combined can serve to answer complex research questions and allow for stronger and richer evidence than could be accomplished by a single method alone. Contextual qualitative data is necessary where the complexity of different sites throughout Canada might create challenges for evaluating the effectiveness of the intervention. This mixed methods approach will allow us to shed light on any potential variations in effects emerging from the RCT.

Quantitative analysis

Patient demographics and baseline outcome variables will be summarized using descriptive summary measures. Analyses will be performed using SAS 9.4 (Cary, NC). Intention to treat analysis will be used. We will use multiple imputation to handle missing data. Analysis of covariance (ANCOVA) will be used to adjust for baseline function as a sensitivity analysis to address any residual imbalance from the randomization. Since participants will be recruited from only four regions, we will model the effect of region as a fixed effect rather than a random effect [34]. A fixed effects model will allow region-specific intervention effects to be modelled (i.e. region by intervention interaction effects). If no region by intervention interactions are found, the interaction terms will be dropped from the model and we will estimate an overall intervention effect. Detailed information about the statistical analysis addressing each objective is provided in Supplementary File 5.

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3 Cost-effectiveness analyses will be conducted from the patient and system (provincial)
4 perspective. Costing relates to the cost to develop and resources to support the intervention as
5 well as resources used for treatment/management of participants' health conditions over the
6 study. We will use an exploratory health economic evaluation to assess youth engagement prior
7 to transition. A decision tree that models the intervention and control groups will be conducted.
8 A 3% discount rate will be applied to outcomes and costs extending beyond one year. All
9 measurement and analytic assumptions made for the base case analysis will be clearly stated.
10 The mean cost per child and the mean effectiveness result per child for each group will be
11 represented in an incremental cost-effectiveness ratio, the ratio of the difference between groups
12 in mean cost per patient to the difference in mean effectiveness. Subgroups of patients based on
13 baseline demographic factors may be analyzed separately, if appropriate. Extensive sensitivity
14 analysis including probabilistic sensitivity analysis will be undertaken to test the robustness of
15 the results to variations in underlying assumptions.
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19 *Qualitative data analysis*

20 Individual interviews will be audio-recorded and transcribed verbatim. Data will be stored and
21 managed electronically using NVivo® Version 11. Conventional content analysis [35] will be
22 used to code, categorize and synthesize the data to contextualize the analysis of the primary aim
23 of the RCT. In addition, data related to usability of the App will be monitored and analyzed to
24 inform ongoing App development.
25
26

27 **ETHICS AND DISSEMINATION**

28 The study has been approved by the Research Ethics Board of each participating site, and the
29 study will be conducted according to the principles of the Declaration of Helsinki. Findings of
30 the RCT will be published in open access, peer-reviewed scientific journals and presented at
31 national and international conferences. Knowledge translation activities directed at the
32 stakeholder community will also include presentations at meetings, and dissemination of
33 teaching and training tools through patient associations, and patient and family advocacy groups.
34 All participants will receive a plain language report at the end of the study after the RCT results
35 have been analyzed. After the completion of this RCT, our team will explore the potential to
36 make the App more widely available.
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40 **CONCLUSION**

41 Readiness for health care transition means that youth with BBD need to develop the necessary
42 skills to manage their health condition. There are real gaps in empowerment and education for
43 this population at this crucial stage in their life. The CHILD-BRIGHT READYorNot™ Brain-
44 Based Disabilities Trial is a mixed-methods RCT to test a novel patient-faced e-Health
45 intervention. While the App is an educational tool for youth with BBD to take charge of their
46 health, there is an animated “mentor” character in the App who serves as a guide to support
47 youth as they learn the necessary skills for their journey through health care transition. Youth
48 with BBD from four regions in Canada are participating in a study designed in collaboration with
49 patients and families to ensure that findings are relevant, meaningful and applicable to the lives
50 of people receiving transition care. Our recruitment strategy includes remote and virtual options
51 in response to the current requirements for physical distancing due to the COVID-19 pandemic.
52 We expect that these novel strategies will continue to be beneficial even after the physical
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3 distancing measures are relaxed, and that the societal trend toward telehealth solutions in health
4 care may enhance future uptake of the intervention into clinical practice [36].
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7 **Registration Details**

8 This RCT has been registered with ClinicalTrials.gov (NCT03852550).
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26 Authors' contributions

27 JWG, AM are the guarantors; JWG, AM, KA, RR, AHK, LT contributed in the study conception
28 and design; JWG, BG, LN contributed in the drafting of the manuscript; JWG, KA, AHK, RR,
29 LT, BG, LN, SS, NM, AVDL, AM were involved in the critical revision; All the authors, PFAC
30 members and RCT Investigators reviewed the manuscript, gave their input and final approval.

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33 Funding statement

34 We gratefully acknowledge funding from the CHILD-BRIGHT network, funded under the
35 Canadian Institutes of Health Research (CIHR) Strategy for Patient-Oriented Research (SPOR)
36 initiative, and with partner support from Montreal Children's Hospital Foundation, McMaster
37 University Faculty of Health Sciences, New Brunswick Health Research Foundation, McMaster
38 Children's Hospital Foundation and Hamilton Health Sciences.

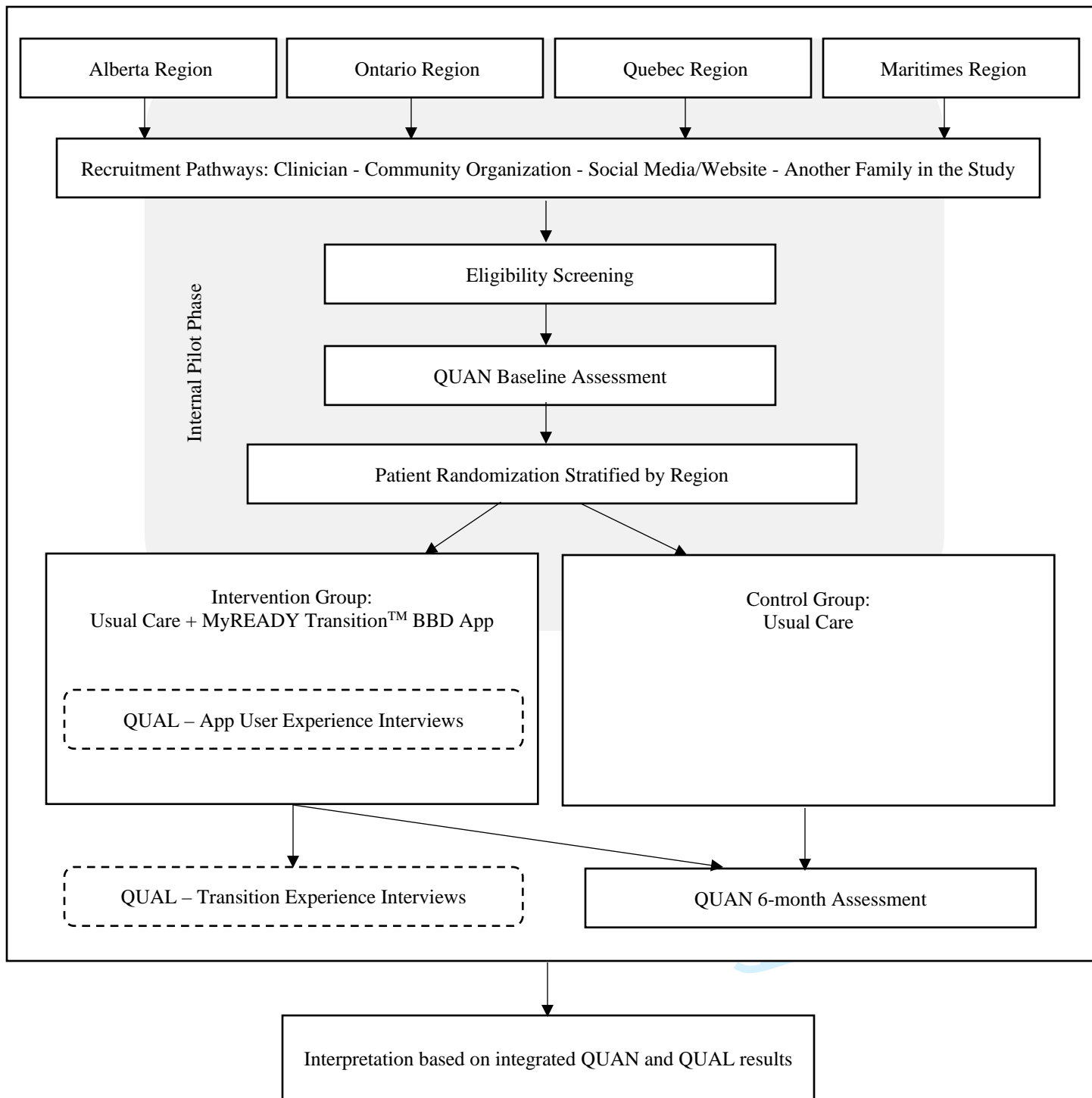
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41 Competing interests statement

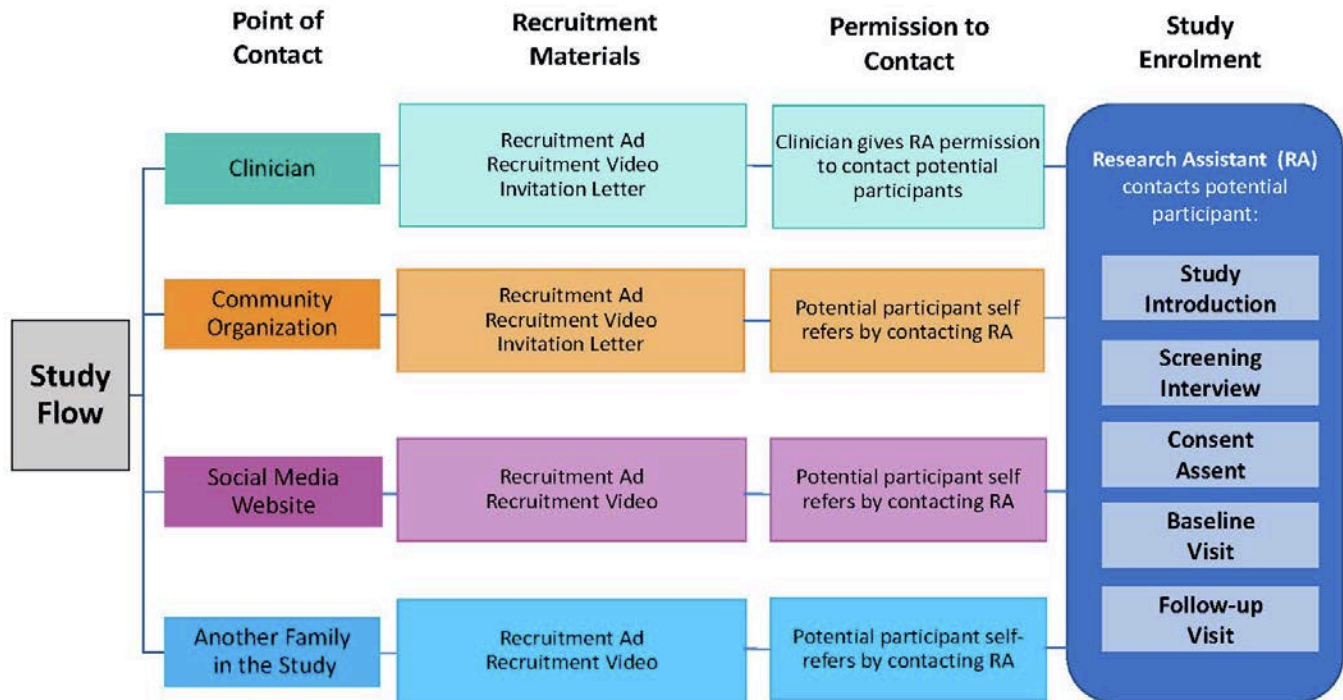
42 JWG and AM have received research grants from the Canadian Institutes of Health Research
43 (CIHR) Strategy for Patient-Oriented Research (SPOR). JWG holds the Scotiabank Chair in
44 Child Health Research. AHK and RR were paid in part for their work as consultants. BG, LN,
45 SS, NM, AVDL were paid for their work as project staff members. LT was paid in part for his
46 work as statistical consultant.

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Figure 1. READYorNot™ Brain-Based Disabilities Trial Design



Supplementary File 1: Recruitment Approach



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Supplementary File 2: Outline of E-Learning Training Modules for Research Staff

Name of Module	Summary of Content in the Module
Introduction and Team	Highlights the core team, core staff, patient and family advisory council (PFAC), recruitment sites, and funding.
Study Summary	A high-level overview of the study including purpose, objectives, study design, and expected outcomes.
Patient Engagement	Explains patient-oriented research, how patient and family engagement is incorporated into the study and refers research staff to the Patient-Oriented Research Curriculum in Child Health (PORCCH).
RA Responsibilities and Training	Reviews responsibilities of the RA regarding training, communication, administration and maintaining confidentiality.
Study Assessment Procedures	Explains the various ways that study assessments can be completed including how to set up a zoom meeting call.
Screening Interview	Reviews recruitment and all the steps required to complete the screening interview to determine if a potential participant is eligible for the study. Includes how to access and use instruments in REDCap.
Consent, Assent and Preparing for Baseline Visit	Reviews the verbal consenting and assenting process in REDCap and the Eligibility and Consent/Assent Status form. Information on completion methods for study measures and how to send them to the study participants is also included.
Study Measures	Reviews all the study measures for parent/caregiver, youth and those to be completed by the RA with the youth, for both baseline and follow-up visits. Videos on how to administer the COPM and NVS are included.
Randomization	Step by step information on how to run randomization in REDCap and information and videos of what it means to be in the control group or intervention group.
Concluding the Baseline Visit	Reviews participant appreciation, handout materials for youth and parent/caregiver, and information on the follow-up visit.
MyREADY Transition™ BBD App	Detailed information on the creation of the App, who it is designed for, how to access it, and an overview of the app itself. Information is also provided about the technical support website.
Follow-Up Visit	Reviews participant appreciation, and procedure for the follow-up visit after 6 months.
Information and Resources	Shows where to access other study documents and resources (e.g. in the File Repository in REDCap)
Contact Information	Contact information for RCT coordinators for ongoing support, to answer questions or if more information is required.

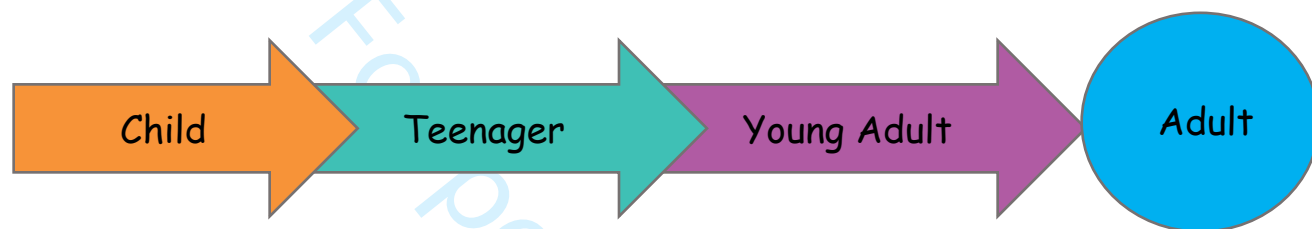
Supplementary File 3: Standard Reference Handouts (Youth and Parent/Caregiver versions)

Helping You Get Ready for Adult Health Care

Information for Teens

As a teenager, you are starting to learn how to take care of yourself. Over the next few years, you will gradually take on more responsibility for your health. This process, called, transition, is part of growing up.

Health care transition is when you make the change from getting pediatric services (e.g., from the children's health care team, the children's hospital or the children's treatment centre) to looking after your own health in adult services.



Typically, as a child, your parents and the health care team took care of you.

During your teenage years, your parents and the health care team will help you learn what you need to know and do to take care of your health.

You gradually take on more responsibility for your care.

As a young adult, you will transfer from pediatric to adult health care.

When you become an adult, you generally will be more responsible for your own health care, with help from others as needed.

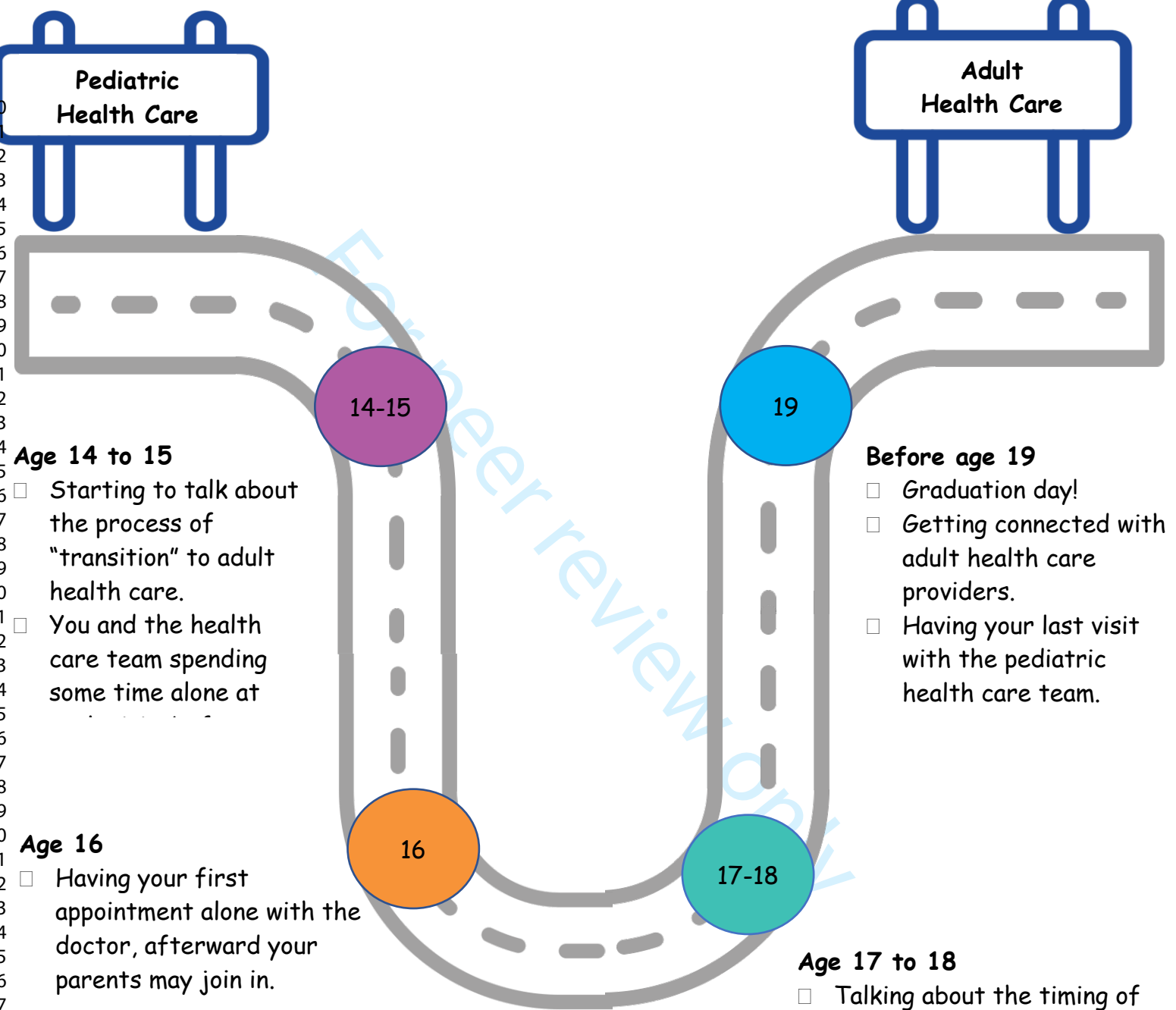
At around age 18, most teens are expected to say goodbye to their pediatric health care team. They transfer to adult care where they begin taking care of their health care. The exact time of transfer varies from person to person.

There is a map on the next page that shows how health care teams usually work closely with families to make 'graduation' to adult health care go as smoothly as possible, by:

- telling you what to expect
- giving you lots of information about your health
- helping you make plans, set goals and learn what you need to do
- making the change gradual, not sudden
- supporting you along the way

Some of the Usual Steps in the Journey from Pediatric to Adult Health Care

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Pediatric Health Care

Adult Health Care

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Age 14 to 15

- Starting to talk about the process of "transition" to adult health care.
- You and the health care team spending some time alone at

Age 16

- Having your first appointment alone with the doctor, afterward your parents may join in.
- Sharing information about your health: how you are feeling and how you have been doing.
- Getting comfortable asking questions of health care providers.

Before age 19

- Graduation day!
- Getting connected with adult health care providers.
- Having your last visit with the pediatric health care team.

Age 17 to 18

- Talking about the timing of your "transfer" to adult health care.
- Reviewing health services and resources.

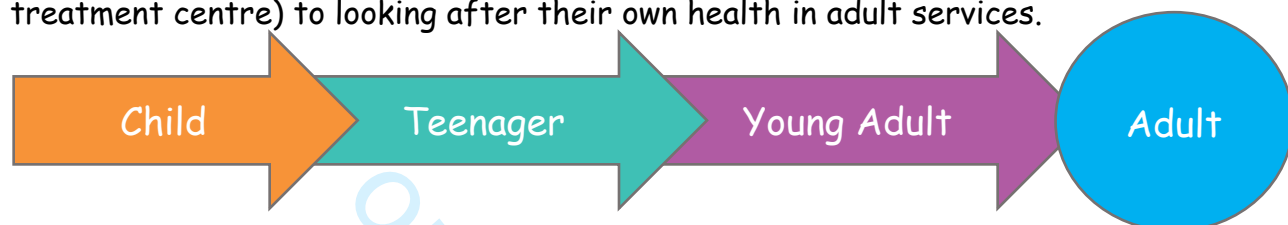
Information adapted from: Hamilton Health Sciences, 2010 PD 7140 – 08/2010. WPC\PtEd\CH\SpasticityClinicAdultCareTeens-lw.doc. dt/August 11, 2010. Getting On TRAC For Adult Care <http://www.bcchildrens.ca/transition-to-adult-care/Documents/ONTRAC-timeline-brochureBCCH.pdf>
 Images: Road Sign by Rinrin from the Noun Project, Curved Road by Ben Davis from the Noun Project

Helping Your Child Get Ready for Adult Health Care

Information for Parents

Over the next few years, your teen will gradually take on more responsibility for their health. This process, called transition, is part of growing up.

Health care transition is when youth make the change from getting pediatric services (e.g., from the children's health care team, the children's hospital or the children's treatment centre) to looking after their own health in adult services.



Typically, throughout childhood, you took care of your child along with the health care team.

During the teenage years, you and the health care team will help your teen learn how to take care of their health.

To the extent that they are able, your teen will gradually take on more responsibility for their own care.

As a young adult, your teen will transfer from pediatric to adult care.

When your teen becomes an adult, they generally will be more responsible for their own health care, with help from others as needed.

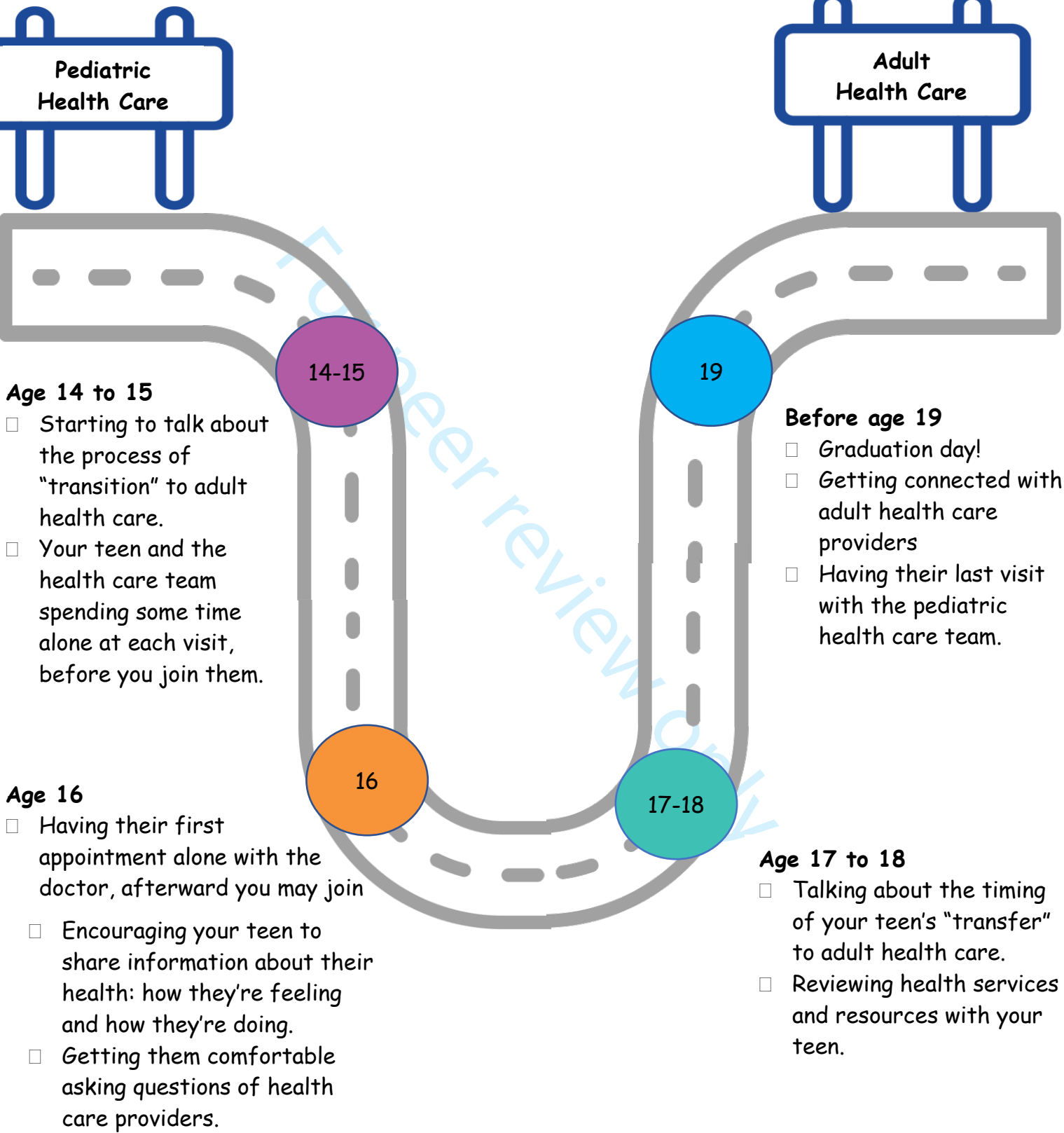
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- supporting you and your teen along the way

Some of the Usual Steps in the Journey from Pediatric to Adult Health Care

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Information adapted from: Hamilton Health Sciences, 2010 PD 7140 – 08/2010. WPC\PtEd\CH\SpasticityClinicAdultCareTeens-lw.doc. dt/August 11, 2010. Getting On TRAC For Adult Care <http://www.bcchildrens.ca/transition-to-adult-care/Documents/ONTRAC-timeline-brochureBCCH.pdf>
 Images: Road Sign by Rinrin from the Noun Project, Curved Road by Ben Davis from the Noun Project

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Supplementary File 4: Study Measures

Data Collection Forms	Completed by	Timing		
		Screen Prior to Baseline	Baseline Visit	6 Month Visit
Primary Outcome Measure				
<p><i>Transition Readiness Assessment Questionnaire (TRAQ)</i> [1]</p> <p>The TRAQ has often been used in previous studies to measure transition readiness [2]. While TRAQ measure refinement is ongoing, and other versions are now available, our sample size calculation is based on findings from an intervention trial [3] where the 29-item version of the TRAQ was used. The 29-item version has a Self-management domain (16 items) and a Self-advocacy domain (13 items). Each item is scored 1-5, where 1 = “No, I do not know how” and 5 = “Yes, I always do this when I need to.” The TRAQ will be completed by youth participants in both groups at Baseline and at 6-Months.</p>	Youth		X	X
Secondary Outcome Measures				
<p><i>TRANSITION-Q</i> [4]</p> <p>The TRANSITION-Q is a 14-item transition readiness/self-management ability scale [4,5]. This short, clinically meaningful and psychometrically sound scale can be used in research and in pediatric and adolescent clinics to help evaluate readiness for transition [4]. Item responses (“never” = 0, “sometimes” = 1, and “always” = 2) are summed to create a raw score, with a possible range from 0 to 28. Raw scores are transformed using a table provided by the developers and the transformed scores range from 0-100. A higher score indicates greater transition readiness; exhibiting more self-management skills with higher frequency [4,6].</p>	Research Assistant with youth	X		X
<p><i>Canadian Occupational Performance Measure (COPM)</i> [7]</p> <p>The COPM is an evidence-based, generic, and individualized outcome measure used to capture a client’s self-perception of performance and satisfaction in everyday living, over time [7]. The measure can be used to identify problems in performing activities of daily living, and the participant is encouraged to think about things that they want to do, need to do or are expected to do but</p>	Research Assistant with youth		X	X

<p>can't do, don't do or aren't satisfied with the way they do. The participant will be asked to rate the current performance of each using a 10-point scale from 'not able to do it' to 'able to do it very well'. The patient is also asked to rate satisfaction with performance on a 10-point scale from 'not satisfied at all' to 'extremely satisfied' with higher scores reflecting better performance and satisfaction with performance as perceived by the participant. The performance and satisfaction can be re-assessed following a period of treatment [8].</p>				
<p><i>Newest Vital Sign</i> [9] The NVS is a health literacy measure that can be easily administered in three minutes. The NVS will help provide a description about participants at baseline and explore determinants of change in self-management, as well as tailoring the intervention in the knowledge translation phase.</p>	<p>Research Assistant with youth</p>		<p>X</p>	
<p><i>PedsQL™ Pediatric Quality of Life Instrument, Generic Core, Teen Report (13-18 years)</i> [10] The PedsQL™ Pediatric Quality of Life Instrument, Generic Core, Teen Report (13-18 years) will be completed by youth participants at Baseline and at 6 months. The form is brief (23 items), practical (less than 4 minutes to complete), multidimensional (physical, emotional, social, school functioning), reliable (child self-report; 0.90) and valid (distinguishes between healthy children and children with acute and chronic health conditions; distinguishes disease severity within a chronic health condition), and responsive to clinical change over time.</p>	<p>Youth</p>		<p>X</p>	<p>X</p>
<p><i>System Usability Scale (SUS)</i> [11] is a self-reported survey focusing on users' utilization of the application and its features, the perceived value, experience and satisfaction with the intervention. It will provide additional information about the users' adherence, behavior, motivation and experience with the IT platform, as well as the main reasons for using or not using it.</p>	<p>Youth (intervention group)</p>			<p>X</p>
<p><i>Demographic Information Form</i> was developed by the CHILD-BRIGHT Network. Studies involving humans collect information on gender, race and ethnicity as well as other characteristics of individuals that may influence how people respond. These questions will help us understand and describe the participants in CHILD-BRIGHT studies.</p>	<p>Parent</p>		<p>X</p>	
<p><i>Profile Information Form</i> was developed by the CHILD-BRIGHT Network and includes questions</p>	<p>Parent</p>		<p>X</p>	

<p>about the child's functionalities and how certain factors might impact their quality of life. These questions will help us understand and describe the participants in CHILD-BRIGHT studies.</p>				
<p><i>Measure of Process of Care (MPOC)</i> [12] The Measure of Processes of Care is a well-validated and reliable self-report measure of parents' perceptions of the extent to which the health services they and their child(ren) receive are family-centred. The original version of MPOC is a 56-item questionnaire; as of 1999 there is a shorter, 20-item version. MPOC has been used internationally in many evaluations of family-centred service. Parents/caregivers will complete the (modified with permission) MPOC-20 at Baseline and 6-Months.</p>	Parent		X	X
<p><i>Health Utilities Index® (Hui2/3) Proxy-Assessed (health-related quality of life)</i> [13] The HUI is a generic health status instrument developed in Canada for use with children and has been incorporated in numerous clinical studies as well as the Canadian Community Health Survey, allowing the generation of norms for most age groups.</p>	Parent		X	X
<p><i>Resource Use Questionnaire (RUQ)</i> [14] The RUQ is typically an interviewer-administered questionnaire for parents of children aged 11 to 18 years. The original RUQ measures the family resource use of condition-related treatments, services and programs, as well as parent time losses and family out-of-pocket costs. It also documents condition-related government subsidies and funding that families receive. Resources measured include those delivered by a parent, by other providers (e.g. behavioural specialist) or a combination of both. In this RCT, a modified subset of RUQ questions will be administered and completed by the parent/caregiver.</p>	Parent		X	X

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Supplementary File 5: Study Objectives, Outcomes, Hypotheses and Analysis Methods

Objective	Outcome	Hypothesis	Method of Analysis
Primary: To determine whether the MyREADY Transition™ BBD App intervention will result in improved transition readiness	Primary: <ul style="list-style-type: none"> • Change in TRAQ self-management score from Baseline to 6 months. 	Intervention > Control	ANCOVA
	Secondary: <ul style="list-style-type: none"> • Change in TRAQ self-advocacy score from Baseline to 6 months. 	Intervention > Control	ANCOVA
	Secondary: <ul style="list-style-type: none"> • Health care transition experience 	Individual semi-structured interviews	Qualitative Methods
Secondary: What is the effect of the MyREADY Transition™ BBD App intervention for improving health and use of health systems?	Primary: Population Health <ul style="list-style-type: none"> • Serious illness (hospitalizations, ICU admission questions from Resource Use Questionnaire • PedsQL™ Pediatric Quality of Life • TRANSITION-Q 	Intervention > Control	ANCOVA for continuous outcomes Logistic regression for hospitalization
	Secondary: <ul style="list-style-type: none"> • Utilization MyREADY Transition™ BBD App 	User metrics built into MyREADY Transition™ BBD App intervention to assess the extent to which various components of the intervention are accessed	Descriptive
	Secondary: Cost utility/cost-effectiveness <ul style="list-style-type: none"> • Health Utilities Index® (Hui2/3) • Resource Use Questionnaire 	Evaluate changes in patients' health in relation to changes in cost to assess if the intervention represents an efficient allocation of health care resources	Descriptive. Cost-effectiveness analysis with support from Child-Bright health economics network team
	Secondary: <ul style="list-style-type: none"> • Achievement of health/life goals • COPM 	Intervention > Control	Paired Student t-tests to compare mean ratings for performance and satisfaction on the COPM scoring system (10-point scale) with > 2 points difference as clinically meaningful difference

1 ANCOVA: Analysis of covariance

2 COPM: Canadian Occupational Performance Measure

3 HUI: Health Utilities Index®

4 ICU: Intensive Care Unit

5 TRAQ: Transition Readiness Assessment Questionnaire

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For peer review only



STANDARD PROTOCOL ITEMS: RECOMMENDATIONS FOR INTERVENTIONAL TRIALS

SPIRIT 2013 Checklist: Recommended items to address in a clinical trial protocol and related documents*

Section/item	Item No	Description	Addressed on Page No
Administrative information			
Title	1	Descriptive title identifying the study design, population, interventions, and, if applicable, trial acronym	1
Trial registration	2a	Trial identifier and registry name. If not yet registered, name of intended registry	12
	2b	All items from the World Health Organization Trial Registration Data Set	N/A
Protocol version	3	Date and version identifier	11
Funding	4	Sources and types of financial, material, and other support	14
Roles and responsibilities	5a	Names, affiliations, and roles of protocol contributors	14
	5b	Name and contact information for the trial sponsor	14
	5c	Role of study sponsor and funders, if any, in study design; collection, management, analysis, and interpretation of data; writing of the report; and the decision to submit the report for publication, including whether they will have ultimate authority over any of these activities	N/A
	5d	Composition, roles, and responsibilities of the coordinating centre, steering committee, endpoint adjudication committee, data management team, and other individuals or groups overseeing the trial, if applicable (see Item 21a for data monitoring committee)	N/A
Introduction			
Background and rationale	6a	Description of research question and justification for undertaking the trial, including summary of relevant studies (published and unpublished) examining benefits and harms for each intervention	4-5
	6b	Explanation for choice of comparators	7
Objectives	7	Specific objectives or hypotheses	4-5

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2	Trial design	8	Description of trial design including type of trial (eg, parallel group, crossover, factorial, single group), allocation ratio, and framework (eg, superiority, equivalence, noninferiority, exploratory)	5
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8	Methods: Participants, interventions, and outcomes			
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10	Study setting	9	Description of study settings (eg, community clinic, academic hospital) and list of countries where data will be collected. Reference to where list of study sites can be obtained	5
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15	Eligibility criteria	10	Inclusion and exclusion criteria for participants. If applicable, eligibility criteria for study centres and individuals who will perform the interventions (eg, surgeons, psychotherapists)	5-6
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21	Interventions	11a	Interventions for each group with sufficient detail to allow replication, including how and when they will be administered	6-7
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26		11b	Criteria for discontinuing or modifying allocated interventions for a given trial participant (eg, drug dose change in response to harms, participant request, or improving/worsening disease)	N/A
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31		11c	Strategies to improve adherence to intervention protocols, and any procedures for monitoring adherence (eg, drug tablet return, laboratory tests)	9
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36		11d	Relevant concomitant care and interventions that are permitted or prohibited during the trial	7
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39	Outcomes	12	Primary, secondary, and other outcomes, including the specific measurement variable (eg, systolic blood pressure), analysis metric (eg, change from baseline, final value, time to event), method of aggregation (eg, median, proportion), and time point for each outcome. Explanation of the clinical relevance of chosen efficacy and harm outcomes is strongly recommended	7-8
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48	Participant timeline	13	Time schedule of enrolment, interventions (including any run-ins and washouts), assessments, and visits for participants. A schematic diagram is highly recommended (see Figure)	Figure 1
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54	Sample size	14	Estimated number of participants needed to achieve study objectives and how it was determined, including clinical and statistical assumptions supporting any sample size calculations	8-9
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2 Recruitment 15 Strategies for achieving adequate participant enrolment to 9
3 reach target sample size
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5 **Methods: Assignment of interventions (for controlled trials)**
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7 Allocation:

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9 Sequence generation 16a Method of generating the allocation sequence (eg, 9
10 computer-generated random numbers), and list of any
11 factors for stratification. To reduce predictability of a
12 random sequence, details of any planned restriction (eg,
13 blocking) should be provided in a separate document that
14 is unavailable to those who enrol participants or assign
15 interventions
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18 Allocation concealment mechanism 16b Mechanism of implementing the allocation sequence (eg, 9
19 central telephone; sequentially numbered, opaque, sealed
20 envelopes), describing any steps to conceal the sequence
21 until interventions are assigned
22
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24 Implementation 16c Who will generate the allocation sequence, who will enrol N/A
25 participants, and who will assign participants to
26 interventions
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28 Blinding (masking) 17a Who will be blinded after assignment to interventions (eg, N/A
29 trial participants, care providers, outcome assessors, data
30 analysts), and how
31
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33 17b If blinded, circumstances under which unblinding is N/A
34 permissible, and procedure for revealing a participant's
35 allocated intervention during the trial
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37 **Methods: Data collection, management, and analysis**
38

39 Data collection methods 18a Plans for assessment and collection of outcome, baseline, 9-11
40 and other trial data, including any related processes to
41 promote data quality (eg, duplicate measurements,
42 training of assessors) and a description of study
43 instruments (eg, questionnaires, laboratory tests) along
44 with their reliability and validity, if known. Reference to
45 where data collection forms can be found, if not in the
46 protocol
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50 18b Plans to promote participant retention and complete 6
51 follow-up, including list of any outcome data to be
52 collected for participants who discontinue or deviate from
53 intervention protocols
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2	Data	19	Plans for data entry, coding, security, and storage,	protocol
3	management		including any related processes to promote data quality	
4			(eg, double data entry; range checks for data values).	
5			Reference to where details of data management	
6			procedures can be found, if not in the protocol	
7				
8	Statistical	20a	Statistical methods for analysing primary and secondary	10-11
9	methods		outcomes. Reference to where other details of the	
10			statistical analysis plan can be found, if not in the protocol	
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13		20b	Methods for any additional analyses (eg, subgroup and	11
14			adjusted analyses)	
15				
16		20c	Definition of analysis population relating to protocol non-	10
17			adherence (eg, as randomised analysis), and any	
18			statistical methods to handle missing data (eg, multiple	
19			imputation)	
20				
21				
22	Methods: Monitoring			
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24	Data monitoring	21a	Composition of data monitoring committee (DMC);	DMC not
25			summary of its role and reporting structure; statement of	needed; RCT
26			whether it is independent from the sponsor and competing	independent
27			interests; and reference to where further details about its	from sponsor
28			charter can be found, if not in the protocol. Alternatively,	
29			an explanation of why a DMC is not needed	
30				
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32		21b	Description of any interim analyses and stopping	N/A
33			guidelines, including who will have access to these interim	
34			results and make the final decision to terminate the trial	
35				
36	Harms	22	Plans for collecting, assessing, reporting, and managing	N/A
37			solicited and spontaneously reported adverse events and	
38			other unintended effects of trial interventions or trial	
39			conduct	
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42	Auditing	23	Frequency and procedures for auditing trial conduct, if	9
43			any, and whether the process will be independent from	
44			investigators and the sponsor	
45				
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47	Ethics and dissemination			
48				
49	Research ethics	24	Plans for seeking research ethics committee/institutional	11
50	approval		review board (REC/IRB) approval	
51				
52	Protocol	25	Plans for communicating important protocol modifications	11,12
53	amendments		(eg, changes to eligibility criteria, outcomes, analyses) to	
54			relevant parties (eg, investigators, REC/IRBs, trial	
55			participants, trial registries, journals, regulators)	
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2	Consent or assent	26a	Who will obtain informed consent or assent from potential trial participants or authorised surrogates, and how (see Item 32)	9-10
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6		26b	Additional consent provisions for collection and use of participant data and biological specimens in ancillary studies, if applicable	N/A
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10	Confidentiality	27	How personal information about potential and enrolled participants will be collected, shared, and maintained in order to protect confidentiality before, during, and after the trial	In protocol
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16	Declaration of interests	28	Financial and other competing interests for principal investigators for the overall trial and each study site	14
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19	Access to data	29	Statement of who will have access to the final trial dataset, and disclosure of contractual agreements that limit such access for investigators	N/A
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24	Ancillary and post-trial care	30	Provisions, if any, for ancillary and post-trial care, and for compensation to those who suffer harm from trial participation	N/A
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28	Dissemination policy	31a	Plans for investigators and sponsor to communicate trial results to participants, healthcare professionals, the public, and other relevant groups (eg, via publication, reporting in results databases, or other data sharing arrangements), including any publication restrictions	11
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35		31b	Authorship eligibility guidelines and any intended use of professional writers	
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39		31c	Plans, if any, for granting public access to the full protocol, participant-level dataset, and statistical code	12
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42	Appendices			
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44	Informed consent materials	32	Model consent form and other related documentation given to participants and authorised surrogates	N/A
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47	Biological specimens	33	Plans for collection, laboratory evaluation, and storage of biological specimens for genetic or molecular analysis in the current trial and for future use in ancillary studies, if applicable	N/A
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*It is strongly recommended that this checklist be read in conjunction with the SPIRIT 2013 Explanation & Elaboration for important clarification on the items. Amendments to the protocol should be tracked and dated. The SPIRIT checklist is copyrighted by the SPIRIT Group under the Creative Commons "[Attribution-NonCommercial-NoDerivs 3.0 Unported](https://creativecommons.org/licenses/by-nc-nd/3.0/)" license.

BMJ Open

Protocol of the CHILD-BRIGHT READYorNot™ Brain-Based Disabilities Trial: A Randomized Controlled Trial (RCT) investigating the effectiveness of a patient-facing e-health intervention designed to enhance health care transition readiness in youth.

Journal:	<i>BMJ Open</i>
Manuscript ID	bmjopen-2021-048756.R1
Article Type:	Protocol
Date Submitted by the Author:	12-Feb-2021
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Primary Subject Heading:	Paediatrics
Secondary Subject Heading:	Rehabilitation medicine
Keywords:	Clinical trials < THERAPEUTICS, Developmental neurology & neurodisability < PAEDIATRICS, REHABILITATION MEDICINE

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3 **Title: Protocol of the CHILD-BRIGHT READYorNot™ Brain-Based Disabilities Trial: A**
4 **Randomized Controlled Trial (RCT) investigating the effectiveness of a patient-facing e-**
5 **health intervention designed to enhance health care transition readiness in youth.**
6

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Strengths and limitations of this study

- This study takes a user-centred approach, using a patient-facing e-health intervention to engage with youth and improve transition readiness for individuals with brain-based disabilities (BBD) transitioning to adult health care.
- The embedded mixed method randomized controlled trial (RCT) design is ideal to answer complex research questions and provides stronger and richer evidence than a single method alone.
- The generalizability of the study findings may be limited to youth with BBD who have started to take charge of their own health.
- Prior to study recruitment, we proactively adapted our study methods in response to the COVID-19 pandemic.
- Strong patient and family involvement at all stages of the study focuses on improving the lived experiences of youth with BBD and their families.

Abstract

Introduction

Youth with brain-based disabilities (BBD), as well as their parents/caregivers, often feel ill-prepared for the transfer from pediatric to adult health care services. To address this pressing issue, we developed the MyREADY Transition™ BBD App, a patient-facing e-health intervention. The primary aim of this randomized controlled trial (RCT) is to determine whether the App will result in greater transition readiness compared to usual care for youth with BBD. Secondary aims include exploring the contextual experiences of youth using the App, as well as the interactive processes of youth, their parents/caregivers and health care providers around use of the intervention.

Methods and analysis

We aim to randomize 264 youth with BBD between 15 and 17 years of age, to receive existing services/usual care (control group) or to receive usual care along with the App (intervention group). Our recruitment strategy includes remote and virtual options in response to the current requirements for physical distancing due to the COVID-19 pandemic. We will use an embedded experimental model design, which involves embedding a qualitative study within a randomized controlled trial. The Transition Readiness Assessment Questionnaire (TRAQ) will be administered as the primary outcome measure. Analysis of covariance will be used to compare change in the two groups on the primary outcome measure; analysis will be intention-to-treat. Interviews will be conducted with subsets of youth in the intervention group, as well as parents/caregivers and health care providers.

Ethics and dissemination

The study has been approved by the Research Ethics Board of each participating site in four different regions in Canada. We will leverage our patient and family partnerships to find novel dissemination strategies. Study findings will be shared with the academic and stakeholder

community, including dissemination of teaching and training tools through patient associations, and patient and family advocacy groups.

INTRODUCTION

With health care innovations, more and more people with pediatric-onset disabilities are surviving into adulthood, thus increasing the need for proactive care strategies for this growing cohort [1]. The process of transition to adulthood can be challenging, as youth with disabilities and their families move from familiar pediatric health care services and learn how to navigate new adult services. In many jurisdictions in Canada, as elsewhere, the transfer from pediatric to adult health care services is set by policy and occurs regardless of whether the youth is ready for transition of care. Poor health care transition can have negative health outcomes and result in poor quality of life for youth with brain-based disabilities (BBD), such as autism spectrum disorder cerebral palsy, epilepsy, fetal alcohol spectrum disorder and spina bifida. Lack of access to health care services can result in the increased use of high-cost health care services, increased emergency department visits, family burden, and exacerbated health issues [2–5].

In Canada, this transfer of care typically occurs at age 18. Since the policy-driven age of transfer cannot be changed, we designed the READYorNot™ (READiness in Youth fOR traNsition Out of pediaTric care) Brain-Based Disabilities Trial to evaluate a patient-facing e-health intervention (MyREADY Transition™ BBD App) aimed at fostering self-management and self-advocacy skills in youth with BBD to improve their readiness for adult health care.

Youth with BBD are expected to be prepared for health care transfer by developing the knowledge and skills to manage their health condition. Transition is defined as “a purposeful, planned process that addresses the medical, psychosocial and educational/vocational needs of adolescents and young adults with chronic physical and medical conditions as they move from child-centered to adult-oriented health care systems” [6]. The field of transition of care has grown over the past few decades, with several calls-to-action to improve processes of transition and to develop interventions and resources with a vision to maximize lifelong functioning through uninterrupted health care services as individuals move from adolescence to adulthood [7–9].

The use of information technology, such as eHealth interventions and applications “Apps”, is an appealing, accessible and flexible way to engage youth with BBD and their families. Patient engagement via information technology has been shown to directly impact patient behaviour in a way that promotes positive health outcomes, patient satisfaction, care delivery efficiency, reduces costs and improves quality of care and patient safety [10,11]. We developed the MyREADY Transition™ BBD App to improve transition readiness of youth with BBD. This App was co-created with researchers, health care professionals, technology designers, youth and families working in partnership.

Objectives

The aim of this study is to determine whether the MyREADY Transition™ BBD App intervention will result in greater transition readiness compared to usual care for youth with BBD between 15 and 17 years of age. We hypothesize that youth who receive their usual care and use the MyREADY Transition™ BBD App intervention will have higher self-management change scores over a 6-month period compared to youth who receive their usual care. The secondary aims of the study include exploring the contextual experiences of youth using the App, as well as

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3 the interactive processes of youth, their parents/caregivers and health care providers around use
4 of the intervention. We also aim to explore health economic outcomes by comparing the
5 incremental cost of the intervention compared to current standard of care per unit of
6 effectiveness.
7

8 9 ***Design***

10 This protocol paper describes a randomized controlled trial (RCT) using a mixed methods study
11 design. The RCT will use a pragmatic approach to test whether the intervention works under
12 usual conditions. Specifically, we will use an embedded experimental model design [12], which
13 will involve embedding a qualitative component within the RCT. See Figure 1. Our selection of
14 outcome metrics and the comprehensive design to evaluate this intervention was guided by the
15 Institute for Health Care Improvement (IHI)'s Triple Aim framework (better health, better
16 experience, at lower cost).
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19 ***Patient and public involvement***

20 The Canadian Institutes of Health Research's Strategy for Patient-Oriented Research (SPOR)
21 endorses that patients and parents/caregivers with "lived experiences," together with health
22 professionals and decision makers, join researchers as members of the research team. Since the
23 very beginning, we have engaged a core group of youth, parents/caregivers and health care
24 stakeholders, including a Patient and Family Advisory Council (PFAC), composed of
25 representative youth (adolescents and young adults) and parents/caregivers. We designed our
26 study in collaboration with patients and families and health care professionals. Our partnership
27 with the PFAC will continue throughout the execution of our RCT, thus enhancing participant
28 recruitment, data collection, engagement planning, and Knowledge Translation (KT). In the KT
29 stage of the project we will work together on novel and meaningful ways to share the study
30 findings about potential benefits of the App.
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34 **METHODS**

35 ***Study setting***

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37 READYorNot™ Brain-Based Disabilities Trial is a patient-oriented project of CHILD-BRIGHT
38 (Child Health Initiatives Limiting Disability-Brain Research Improving Growth and Health
39 Trajectories), which is a pan-Canadian research program. A large cohort of youth with BBD
40 living in Alberta, Ontario, Quebec, and the Maritimes in Canada will be recruited.
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42

43 ***Eligibility Criteria***

44 The following are study inclusion criteria: 1) age of 15 to 17; 2) a diagnosis of autism spectrum
45 disorder, cerebral palsy, epilepsy, fetal alcohol spectrum disorder, or spina bifida; 3) in pediatric
46 care in one of the four study regions and for whom a discharge from pediatric care is planned but
47 not for at least 6 months; 4) cognitive ability to provide informed assent and the ability to read
48 and understand English or French; 5) access to internet and a smartphone, iPad/tablet or desktop
49 computer; and 6) TRANSITION-Q [13] score >40 (as a screen to define a minimum threshold
50 for transition readiness based on our earlier work). In a validation sample of Ontario youth aged
51 12 -25 years with chronic health conditions, including our target population, the average
52 TRANSITION-Q score for 13 year olds was 40; for 15 year olds was 53; and for 17 year olds
53 was 59 [14]. The decision to set the threshold at > 40 aligns with clinical judgement and is
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3 conservative given that the youngest age group in the validation sample demonstrated this
4 minimum level of readiness. The TRANSITION-Q screen will inherently not include youth who
5 have severe intellectual disability, and/or those who rely significantly on parents/caregivers in
6 most areas of daily functioning, self-care and/or communication.
7

8 Individuals will be excluded if they are: 1) in “acute crisis” with unstable physical or
9 mental health that would interfere with the ability to participate in the study; 2) have sensory
10 impairments, such as uncorrected vision or hearing loss, which would interfere with the use of
11 the App; or 3) are enrolled in a potentially confounding trial (e.g., a different transition
12 intervention study).
13

14 ***Intervention***

15 The MyREADY Transition™ BBD App is designed to educate and empower youth with BBD as
16 they prepare for transitioning from pediatric to adult care. We collaborated with the PFAC to
17 inform the design and help tailor the App to incorporate features that were meaningful to them.
18 The App is constructed as a “Journey in the City” with a mentor (virtual coach) that helps the
19 user navigate the buildings and sequentially introduces the educational sections. The content of
20 the App is composed of messages, texts, quizzes, videos, and skill-based-achievement
21 challenges. The App uses pop-up features to manage reminders aimed at keeping engagement
22 and ensuring adherence to the App. The App has 19 visits organized into 5 ordered chapters. In
23 terms of exposure time, there is ‘planned’ flexibility to allow participants to proceed through the
24 MyREADY Transition™ BBD App intervention at their own pace. There is a timer in the App to
25 inform participants when the next visit is unlocked. Participants will receive instructions to wait
26 at least one day between visits. This waiting period will allow participants to process and reflect
27 on the take-away message(s) in each visit and/or engage in one of the suggested between-session
28 practice activities. The waiting period will also moderate the pace and aligns with how young
29 people learn and digest information. There are approximately 5-7 hours of content within the
30 App. For the RCT, the recommended exposure to the App intervention is between one visit per
31 day (19 days) and one visit per week (19 weeks). Games and fun activities are incorporated to
32 encourage youth to explore the App between visits.
33

34 For participants randomized into the intervention group, the research assistant (RA) will
35 help them download the App on their device. To ensure that the participant understands how to
36 access the App’s features, they will watch an introduction video demonstrating the first visit of
37 the App and they will be given a reference handout with tips and strategies for using the App.
38 Participants will also receive a website link for App support, including support for download,
39 access to the introduction video, a list of Frequently Asked Questions, as well as a series of short
40 how-to tutorial videos about different features in the App. The RCT Research Coordinator and
41 Research IT team will further support the use of the App and troubleshoot issues as they arise.
42 This will be done using a designated email to capture and respond to queries and will include an
43 automated response, indicating receipt and approximate response time. During study
44 participation, participants will receive reminders to promote the use of the MyREADY
45 Transition™ BBD App. Parents of youth in the intervention group and health care providers in
46 recruiting clinics will receive guidelines on how to support youth in the intervention group,
47 including an explanation that we want to know how youth are using the App independently,
48 suggestions about ways they can help youth use the App without influencing their use of the
49 App, and information about how youth can access App support.
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Usual care (intervention and control group)

Participants randomized into the control group will continue to get the same care they have been getting (their usual care). Youth participants in both the control and intervention groups, along with their parent/caregiver will receive a standard reference handout (Supplementary File 1) that will provide a basic overview of what they might expect as they get ready for transition. This handout ensures that all study participants have a minimum standard of preparation beyond the usual care they are receiving. Any support the youth (and parents/caregivers) receive as part of any ongoing transition programs in usual care will be documented by youth, parents/caregivers and health care providers. Documentation will include an inventory checklist with a section to add specific information relevant to the transition process. Participants and their parents/caregivers will also be asked what they perceive as supports and if they received these supports.

Outcomes

In this RCT, both quantitative and qualitative data types will be collected. All data collection forms will be available in both English and French. Detailed demographic information from parents/caregivers will be collected to understand and describe the different kinds of families participating in CHILD-BRIGHT studies and to compare this study sample to youth with BBD across Canada.

Measures.

The primary outcome is transition readiness which will be measured with the 29-item version of the Transition Readiness Assessment Questionnaire (TRAQ) [15]. While TRAQ measure refinement is ongoing, and other versions are now available, our sample size calculation is based on findings from an intervention trial [16] where the 29-item version of the TRAQ was used. The 29-item version has a Self-management domain (16 items) and a Self-advocacy domain (13 items). Each item is scored from 1-5, where 1 = “I do not need to do this”, 2 = “I do not know how but I want to learn”, 3 = “I am learning to do this”, 4 = “I have started doing this”, and 5 = “I always do this when I need to”. It is a validated, patient-centred questionnaire used in previous studies to measure transition readiness [17] and is designed to be self-administered at baseline and six months. Details about the TRAQ and other secondary outcome measures are provided in Supplementary File 2.

Secondary outcomes will evaluate whether the intervention has an effect on population health. All youth will be asked to complete the Canadian Occupational Performance Measure (COPM) [18], TRANSITION-Q [13], and PedsQL™ Pediatric Quality of Life Instrument, Generic Core, and Teen Report (13-18 years) [19]. To assess the impact of the App on the families' health care experience, parents/caregivers will complete the Measure of Process of Care (MPOC) (family-centred care) [20], and youth will be asked to complete the Newest Vital Sign [21] as a measure of their health literacy at baseline. Cost utility and cost-effectiveness will be measured using Health Utilities Index® (Hui2/3) Proxy-Assessed (health-related quality of life) [22] and Resource Use Questionnaire (RUQ) [23]. In addition, consent will be requested to obtain youth participants' Health Card Numbers to link information provided in the study with provincial health administrative data about use of health services, such as physician office visits,

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3 visits to emergency rooms and hospitalizations during the study period. Participants may choose
4 to participate without sharing their Health Card information.

5 The MyREADY Transition™ BBD App will log user metrics which will be useful during
6 the intervention (to monitor App use and provide support) and during the evaluation phases of
7 the project (to quantify App use). The App will collect data related to end user login to the
8 system (times and dates); visit/session completion and average time spent; number of clicks by
9 challenge and event type; access to games at the arcade; time spent on a visit/session and trends
10 over time. Within the App, users are asked to provide feedback at the end of each visit. They will
11 rate their experience on a three-point scale using emojis for happy, neutral and unhappy/sad.
12 Questions ask about experiences with the videos, quizzes, and challenges; the usefulness of the
13 content in the visit/session and will ask whether they completed the visit/session alone or with
14 help. The App will collect demographic data provided during the registration process and device
15 information, such as the type of device and its operating system. Participants in the intervention
16 group will also be asked to complete the System Usability Scale [24] at the 6-month visit. The
17 measure will focus on users' utilization of the App and its features, the perceived value,
18 experience, and satisfaction with the intervention.
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23 *Interviews.*

24 As part of the embedded qualitative study (Figure 1), we will purposefully sample two subsets of
25 participants for different study aims from the sample of 132 youth participants in the intervention
26 group. All interviews will be one-on-one, semi-structured, conducted over the phone or by video-
27 conference (e.g. Zoom), and will be audio-recorded.
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29 A subset of approximately 30 youth participants, as well as 10 parents/caregivers and 10
30 health care providers will be interviewed to describe and understand the primary outcomes after
31 the 6-month quantitative data collection point. These interviews will be conducted following
32 their completion of the RCT outcome measures. The purpose will be to understand youth
33 perceptions of their transition readiness skills and awareness after using the App, to understand
34 how they may have used the App in their everyday lives and in interactions with health care
35 providers, and to understand how the App might have influenced their care. Parent and health
36 care providers will be invited to share their perspectives on the potential of the App to improve
37 transition readiness.
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39 To address our secondary aim, a subset of approximately 20 youth participants will be
40 interviewed to capture process and user experience of the App at the end of exposure or after 6
41 months, whichever timepoint comes first. These interviews will complement quantitative
42 findings related to App use, and for opportunities to improve the App. Youth will be asked how
43 the App was used initially and over time, barriers and facilitators to its use, and participants'
44 experience and satisfaction with the App and its features.
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48 *Sample size*

49 The primary sample will be comprised of 264 youth with BBD, aged 15 to 17 years, who are
50 recruited in one of the four study regions (Alberta, Ontario, Quebec, and Maritimes) and who are
51 at least 6 months pre-transfer to the adult health care system. The parent or caregiver of the youth
52 will also be recruited to the study. The study aims to have an equal number of participants in the
53 control and intervention groups. Randomization will be stratified by region with a 1:1 allocation
54 ratio for patients: 132 in the control group (continuing with usual care) and 132 in the
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3 intervention group (continuing with usual care and receiving App intervention). The primary
4 outcome measure (Transition Readiness Assessment Questionnaire, TRAQ) has been validated
5 on a sample of Canadian youth with congenital heart disease [16] and, in the absence of literature
6 specific to BBD, our sample size calculation was based on these findings: We anticipate a mean
7 TRAQ self-management baseline score of 3.01 (SD 1.02) (out of a possible 5.0) as reported for
8 youth (with congenital heart disease) [16] without a transition intervention and an anticipated
9 mean score of 4.0 at 6 months follow-up resulting in a change score of 1 (i.e., 1 SD), with $\alpha=0.05$
10 and 90% power. Therefore, in each region, 23 youth are required in each of the 2 arms x 4
11 regions = 184. With an estimated attrition rate of 30%, we will enroll a total of 264 participants
12 across the 4 regions.
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16 **Recruitment**

17 Before the onset of the study, we expanded our recruitment approach (Supplementary File 3) to
18 include strategies to facilitate the study's operations within the context of physical distancing
19 measures related to the COVID-19 pandemic [25,26]. First, clinicians in the patient's circle of
20 care at each clinic site in each of the four regions will approach eligible participants to ascertain
21 interest and obtain permission for the RA to contact them. They may obtain this permission in
22 person after physical distancing measures are lifted, by telephone or by mail. Recruitment
23 materials will also be shared on websites and social media. As a result, some participants may
24 wish to self-refer and contact the RA directly. The RAs will contact potential participants by
25 phone to complete a screening checklist and confirm eligibility. If there is a clinic appointment
26 scheduled, contact will be made in advance so that they have an opportunity to learn about the
27 study and ask questions. The consent and assent form will be sent to potential participants in
28 advance of the scheduled visit. Due to physical distancing measures, individuals can choose to
29 have the forms sent to them by email or by mail. Prior to collecting any data, informed
30 consent/assent will be obtained. All youth will provide assent and a parent/caregiver will also
31 provide consent both for their child and for themselves. Again, to accommodate physical
32 distancing measures, we have added a telephone verbal consent procedure. Ideally, participants
33 will complete the baseline study visit at the same appointment time. If the latter is not possible,
34 the RA will arrange another study appointment convenient for both the youth and
35 parent/caregiver.
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40 **RANDOMIZATION**

41 Randomization will be stratified by region with a 1:1 allocation ratio for participants: control
42 group who will continue with usual care, or intervention group who will continue with usual care
43 and receive the MyREADY Transition™ BBD App. The unit of randomization is the patient.
44 Variable block randomization will be used with block sizes of 2, 4, 6, and 8. Allocation will be
45 done via REDCap (Research Electronic Data Capture) [27]. Individuals who meet the eligibility
46 requirements at the point of screening, and who give consent to participate will be randomized
47 after the baseline questionnaires are completed. Participants allocated to the control or usual care
48 group will not know the specific details of the electronic intervention being offered to the
49 intervention group. Due to the nature of the intervention, participants cannot be blinded to group
50 allocation, however, outcome assessment and data analysis will be blinded.
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54 **PROCEDURES**

Prior to the start of the study, each study RA will complete e-learning module training provided by the research team (Supplementary File 4). Procedural fidelity will be monitored.

Data collection

To track participants according to the CONSORT guidelines [28,29], a de-identified log of screened youth patients at all participating sites will be kept, recording inclusion/exclusion criteria and reasons for eligible youth patients not being recruited or randomized.

In case the baseline visit is not done in-person due to physical distancing measures, we have added the option to conduct the baseline visit via telephone or “Zoom” meeting (Zoom Video Communications, Inc.). To better establish rapport with participants, the RA may conduct the Zoom meeting with their own camera on [30,31]. Participants will have the option to turn on their camera or keep it off. Zoom meetings will not be recorded.

A Data Transfer Agreement (DTA) will be in place with each participating centre to ensure secure transfer and storage of the study data. The RCT will be centrally managed by the RCT Coordinator at McMaster University’s CanChild. Research files will be stored on the CanChild Active Directory at McMaster, on a secure network that is in a tier 3 data facility. The CanChild Active Directory is a firewall protected server to which only the PIs and Research Coordinators will have access. Remote access to the CanChild directory is via VPN. Any personal information collected will be entered into password-protected SPSS or excel files and stored on the CanChild Active Directory, separate from other study data. Qualitative data will be stored on the CanChild Active Directory and managed electronically using NVivo, a qualitative data analysis software system. Research staff will password protect their electronic and audio digital files from the interview sessions and can transmit these into the secure cloud storage provided through McMaster's MacDrop (<https://drop.mcmaster.ca/login>), with final storage on the CanChild Active Directory. There will be a code linking identifiers to the study participant. The Study Doctor(s), Regional Coordinator and Study Research Assistant(s) at each local recruiting site will have access to the key linking study identification number with participant identity for the participants at that site.

REDCap [27] is a secure web application for building and managing online surveys and databases (www.project-redcap.org). REDCap questionnaire data for this project will be hosted by the Department of Pediatrics at McMaster University. De-identified data is stored on a secure, firewall protected server with regular backup in the Faculty of Health Sciences Computer Services Unit with only the https port available to the internet. Data can be entered into REDCap [27] by designated users or survey respondents from any computer with an internet connection. Surveys will be completed on paper by a study participant and entered into the REDCap form by the RA at the recruitment site or completed online by a study participant directly into the REDCap form. Since no identifying information is stored in REDCap, the link to electronic survey forms will be sent to the RA and the RA will email it to the participant.

Internal pilot phase

The British Medical Research Council explicitly recommends the use of feasibility studies prior to Phase III clinical trials [32]. To guide the planning and to enhance the likelihood of success of our full scale RCT, the first three months of recruitment will comprise an internal pilot phase where study procedures will be observed and considerations will be taken into account about key implementation aspects, such as recruitment (including refusal rates and screening process), multi-site coordination/collaboration (including communication, documentation and provision of

support) and intervention uptake and adherence (including technical support needs among App users). The results will be used to refine and enhance the research design. The RCT will proceed with procedural modifications based on the findings of the internal pilot study and final study analyses will incorporate all data. As long as the alpha-level is controlled, internal pilot designs have, at most, a small adverse effect on the significance level and may greatly improve the power [33].

ANALYSIS

A mixed methods approach will be used that combines both quantitative and qualitative research methods and techniques. This methodology is commonly used in patient-centred care research as both qualitative and quantitative methods combined can serve to answer complex research questions and allow for stronger and richer evidence than could be accomplished by a single method alone. Contextual qualitative data is necessary where the complexity of different sites throughout Canada might create challenges for evaluating the effectiveness of the intervention. This mixed methods approach will allow us to shed light on any potential variations in effects emerging from the RCT.

Quantitative analysis

Patient demographics and baseline outcome variables will be summarized using descriptive summary measures. Analyses will be performed using SAS 9.4 (Cary, NC). Intention to treat analysis will be used. We will use multiple imputation to handle missing data. Analysis of covariance (ANCOVA) will be used to adjust for baseline function as a sensitivity analysis to address any residual imbalance from the randomization. Since participants will be recruited from only four regions, we will model the effect of region as a fixed effect rather than a random effect [34]. A fixed effects model will allow region-specific intervention effects to be modelled (i.e. region by intervention interaction effects). If no region by intervention interactions are found, the interaction terms will be dropped from the model and we will estimate an overall intervention effect. Detailed information about the statistical analysis addressing each objective is provided in Supplementary File 5.

Cost-effectiveness analyses will be conducted from the patient and system (provincial) perspective. Costing relates to the cost to develop and resources to support the intervention as well as resources used for treatment/management of participants' health conditions over the study. We will use an exploratory health economic evaluation to assess youth engagement prior to transition. A decision tree that models the intervention and control groups will be conducted. A 3% discount rate will be applied to outcomes and costs extending beyond one year. All measurement and analytic assumptions made for the base case analysis will be clearly stated. The mean cost per child and the mean effectiveness result per child for each group will be represented in an incremental cost-effectiveness ratio, the ratio of the difference between groups in mean cost per patient to the difference in mean effectiveness. Subgroups of patients based on baseline demographic factors may be analyzed separately, if appropriate. Extensive sensitivity analysis including probabilistic sensitivity analysis will be undertaken to test the robustness of the results to variations in underlying assumptions.

Qualitative data analysis

Individual interviews will be audio-recorded and transcribed verbatim. Data will be stored and managed electronically using NVivo® Version 11. Conventional content analysis [35] will be

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3 used to code, categorize and synthesize the data to contextualize the analysis of the primary aim
4 of the RCT. In addition, data related to usability of the App will be monitored and analyzed to
5 inform ongoing App development.
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7 In summary, readiness for health care transition means that youth with BBD need to
8 develop the necessary skills to manage their health condition. There are real gaps in
9 empowerment and education for this population at this crucial stage in their life. The CHILD-
10 BRIGHT READYorNot™ Brain-Based Disabilities Trial is a mixed-methods RCT to test a
11 novel patient-faced e-Health intervention. While the App is an educational tool for youth with
12 BBD to take charge of their health, there is an animated “mentor” character in the App who
13 serves as a guide to support youth as they learn the necessary skills for their journey through
14 health care transition. Youth with BBD from four regions in Canada are participating in a study
15 designed in collaboration with patients and families to ensure that findings are relevant,
16 meaningful and applicable to the lives of people receiving transition care. Our recruitment
17 strategy includes remote and virtual options in response to the current requirements for physical
18 distancing due to the COVID-19 pandemic. We expect that these novel strategies will continue
19 to be beneficial even after the physical distancing measures are relaxed, and that the societal
20 trend toward telehealth solutions in health care may enhance future uptake of the intervention
21 into clinical practice [36].
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26 **ETHICS AND DISSEMINATION**

27 The study has been approved by the Research Ethics Board of each participating site
28 (Supplementary File 6): Hamilton Integrated Research Ethics Board (HiREB) - Clinical Trials
29 Ontario (CT0) #1666; [Alberta] Health Research Ethics Board - Health Panel
30 #MS2_Pro00086027; Horizon Health Network Research Ethics Board #2018-2689; Research
31 Ethics Board at the University of New Brunswick (Saint John) #037-2019; Mount Allison
32 University Research Ethics Board #102606; IWK Research Ethics Board # 1025247. The study
33 will be conducted according to the principles of the Declaration of Helsinki. Findings of the RCT
34 will be published in open access, peer-reviewed scientific journals and presented at national and
35 international conferences. Knowledge translation activities directed at the stakeholder
36 community will also include presentations at meetings, and dissemination of teaching and
37 training tools through patient associations, and patient and family advocacy groups. All
38 participants will receive a plain language report at the end of the study after the RCT results have
39 been analyzed. After the completion of this RCT, our team will explore the potential to make the
40 App more widely available.
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46 **Registration Details**

47 This RCT has been registered with ClinicalTrials.gov (NCT03852550).
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49 **Contributorship statement**

50 JWG, AM are the guarantors; JWG, AM, KA, RR, AHK, LT contributed in the study conception
51 and design; JWG, BG, LN contributed in the drafting of the manuscript; JWG, KA, AHK, RR,
52 LT, BG, LN, SS, NM, AVDL, AM were involved in the critical revision; All the authors, PFAC
53 members and RCT Investigators reviewed the manuscript, gave their input and final approval.
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Competing interests statement

JWG and AM have received research grants from the Canadian Institutes of Health Research (CIHR) Strategy for Patient-Oriented Research (SPOR). JWG holds the Scotiabank Chair in Child Health Research. AHK and RR were paid in part for their work as consultants. BG, LN, SS, NM, AVDL were paid for their work as project staff members. LT was paid in part for his work as statistical consultant.

Funding statement

We gratefully acknowledge funding from the CHILD-BRIGHT (Child Health Initiatives Limiting Disability - Brain Research Improving Growth and Health Trajectories) network, funded under the Canadian Institutes of Health Research (CIHR-SCA-145104) Strategy for Patient-Oriented Research (SPOR) initiative, and with funding partner support from Montreal Children's Hospital Foundation, McMaster University Faculty of Health Sciences, New Brunswick Health Research Foundation, McMaster Children's Hospital Foundation and Hamilton Health Sciences.

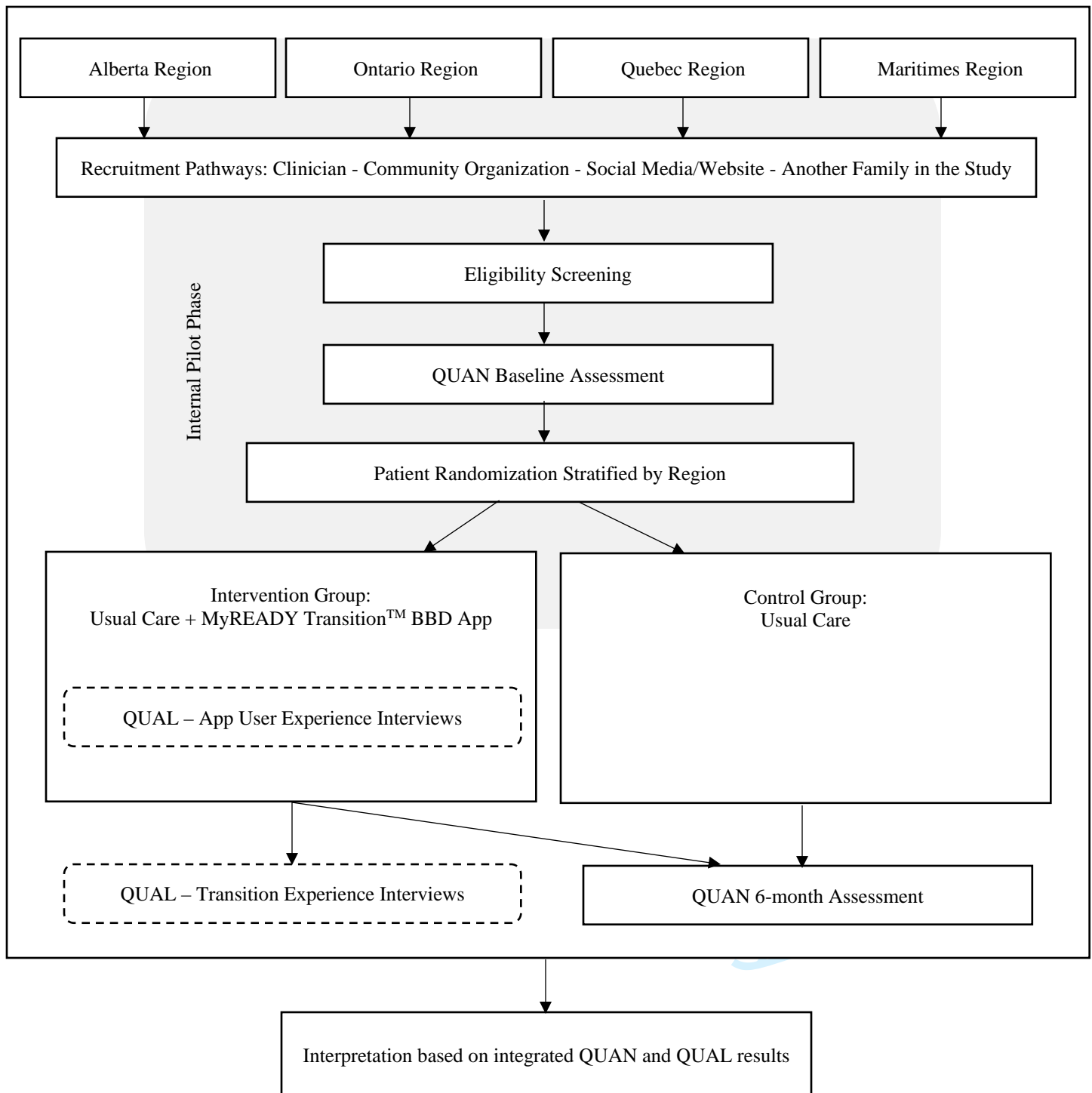
Figure 1: Figure on study design

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Figure 1. READYorNot™ Brain-Based Disabilities Trial Design

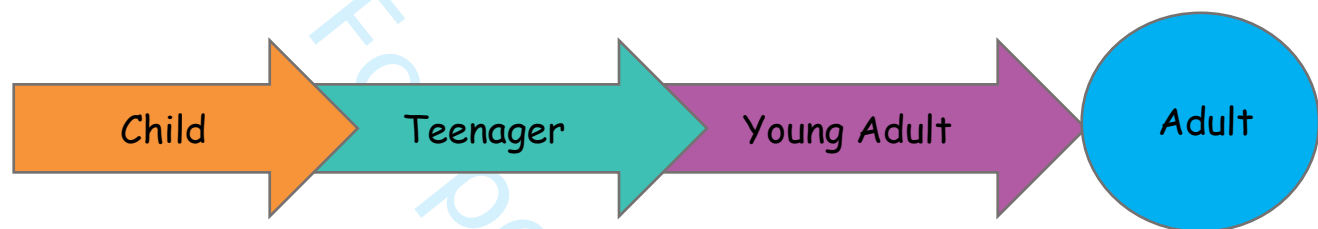
Supplementary File 1: Standard Reference Handouts (Youth and Parent/Caregiver versions)

Helping You Get Ready for Adult Health Care

Information for Teens

As a teenager, you are starting to learn how to take care of yourself. Over the next few years, you will gradually take on more responsibility for your health. This process, called, transition, is part of growing up.

Health care transition is when you make the change from getting pediatric services (e.g., from the children's health care team, the children's hospital or the children's treatment centre) to looking after your own health in adult services.



Typically, as a child, your parents and the health care team took care of you.

During your teenage years, your parents and the health care team will help you learn what you need to know and do to take care of your health.

You gradually take on more responsibility for your care.

As a young adult, you will transfer from pediatric to adult health care.

When you become an adult, you generally will be more responsible for your own health care, with help from others as needed.

At around age 18, most teens are expected to say goodbye to their pediatric health care team. They transfer to adult care where they begin taking care of their health care. The exact time of transfer varies from person to person.

There is a map on the next page that shows how health care teams usually work closely with families to make 'graduation' to adult health care go as smoothly as possible, by:

- telling you what to expect
- giving you lots of information about your health
- helping you make plans, set goals and learn what you need to do
- making the change gradual, not sudden
- supporting you along the way

Some of the Usual Steps in the Journey from Pediatric to Adult Health Care

**Pediatric
Health Care**

**Adult
Health Care**

14-15

19

16

17-18

Age 14 to 15

- Starting to talk about the process of "transition" to adult health care.
- You and the health care team spending some time alone at each visit, before your parents join you.

Age 16

- Having your first appointment alone with the doctor, afterward your parents may join in.
- Sharing information about your health: how you are feeling and how you have been doing.
- Getting comfortable asking questions of health care providers.

Before age 19

- Graduation day!
- Getting connected with adult health care providers.
- Having your last visit with the pediatric health care team.

Age 17 to 18

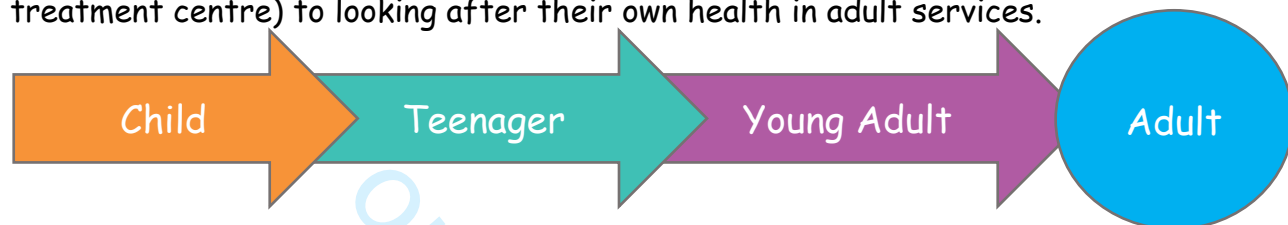
- Talking about the timing of your "transfer" to adult health care.
- Reviewing health services and resources.

Helping Your Child Get Ready for Adult Health Care

Information for Parents

Over the next few years, your teen will gradually take on more responsibility for their health. This process, called transition, is part of growing up.

Health care transition is when youth make the change from getting pediatric services (e.g., from the children's health care team, the children's hospital or the children's treatment centre) to looking after their own health in adult services.



Typically, throughout childhood, you took care of your child along with the health care team.

During the teenage years, you and the health care team will help your teen learn how to take care of their health.

To the extent that they are able, your teen will gradually take on more responsibility for their own care.

As a young adult, your teen will transfer from pediatric to adult care.

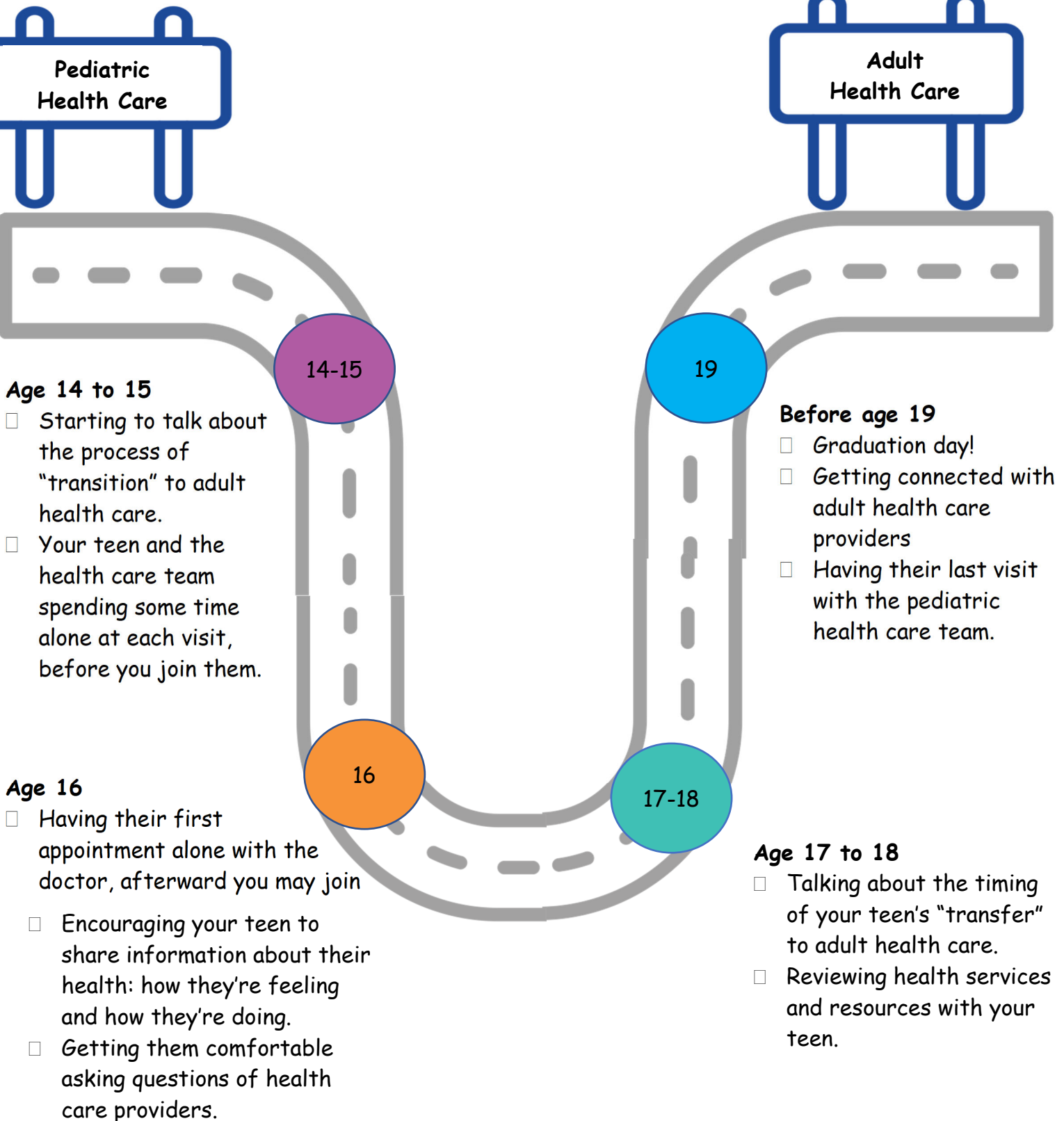
When your teen becomes an adult, they generally will be more responsible for their own health care, with help from others as needed.

At around age 18, most teens are expected to say goodbye to their pediatric health care team. They transfer to adult care where they begin taking care of their health care. The exact time of transfer varies from person to person.

There is a map on the next page that shows how health care teams usually work closely with families to make 'graduation' to adult health care go as smoothly as possible, by:

- telling your teen what to expect
- giving your teen lots of information about their health
- helping your teen make plans, set goals and learn what they need to do
- making the change gradual, not sudden
- supporting you and your teen along the way

Some of the Usual Steps in the Journey from Pediatric to Adult Health Care



Supplementary File 2: Study Measures

Data Collection Forms	Completed by	Timing		
		Screen Prior to Baseline	Baseline Visit	6 Month Visit
Primary Outcome Measure				
<p><i>Transition Readiness Assessment Questionnaire (TRAQ)</i> [1]</p> <p>The TRAQ has often been used in previous studies to measure transition readiness [2]. While TRAQ measure refinement is ongoing, and other versions are now available, our sample size calculation is based on findings from an intervention trial [3] where the 29-item version of the TRAQ was used. The 29-item version has a Self-management domain (16 items) and a Self-advocacy domain (13 items). Each item is scored from 1-5, where 1 = “I do not need to do this”, 2 = “I do not know how but I want to learn”, 3 = “I am learning to do this”, 4 = “I have started doing this”, and 5 = “I always do this when I need to” The TRAQ will be completed by youth participants in both groups at Baseline and at 6-Months.</p>	Youth		X	X
Secondary Outcome Measures				
<p><i>TRANSITION-Q</i> [4]</p> <p>The TRANSITION-Q is a 14-item transition readiness/self-management ability scale [4,5]. This short, clinically meaningful and psychometrically sound scale can be used in research and in pediatric and adolescent clinics to help evaluate readiness for transition [4]. Item responses (“never” = 0, “sometimes” = 1, and “always” = 2) are summed to create a raw score, with a possible range from 0 to 28. Raw scores are transformed using a table provided by the developers and the transformed scores range from 0-100. A higher score indicates greater transition readiness; exhibiting more self-management skills with higher frequency [4,6].</p>	Research Assistant with youth	X		X
<p><i>Canadian Occupational Performance Measure (COPM)</i> [7]</p> <p>The COPM is an evidence-based, generic, and individualized outcome measure used to capture a client’s self-perception of performance and satisfaction in everyday living, over time [7]. The measure can be used to identify problems in</p>	Research Assistant with youth		X	X

<p>performing activities of daily living, and the participant is encouraged to think about things that they want to do, need to do or are expected to do but can't do, don't do or aren't satisfied with the way they do. The participant will be asked to rate the current performance of each using a 10-point scale from 'not able to do it' to 'able to do it very well'. The patient is also asked to rate satisfaction with performance on a 10-point scale from 'not satisfied at all' to 'extremely satisfied' with higher scores reflecting better performance and satisfaction with performance as perceived by the participant. The performance and satisfaction can be re-assessed following a period of treatment [8].</p>				
<p><i>Newest Vital Sign</i> [9] The NVS is a health literacy measure that can be easily administered in three minutes. The NVS will help provide a description about participants at baseline and explore determinants of change in self-management, as well as tailoring the intervention in the knowledge translation phase.</p>	<p>Research Assistant with youth</p>		<p>X</p>	
<p><i>PedsQL™ Pediatric Quality of Life Instrument, Generic Core, Teen Report (13-18 years)</i> [10] The PedsQL™ Pediatric Quality of Life Instrument, Generic Core, Teen Report (13-18 years) will be completed by youth participants at Baseline and at 6 months. The form is brief (23 items), practical (less than 4 minutes to complete), multidimensional (physical, emotional, social, school functioning), reliable (child self-report; 0.90) and valid (distinguishes between healthy children and children with acute and chronic health conditions; distinguishes disease severity within a chronic health condition), and responsive to clinical change over time.</p>	<p>Youth</p>		<p>X</p>	<p>X</p>
<p><i>System Usability Scale (SUS)</i> [11] is a self-reported survey focusing on users' utilization of the application and its features, the perceived value, experience and satisfaction with the intervention. It will provide additional information about the users' adherence, behavior, motivation and experience with the IT platform, as well as the main reasons for using or not using it.</p>	<p>Youth (intervention group)</p>			<p>X</p>
<p><i>Demographic Information Form</i> was developed by the CHILD-BRIGHT Network. Studies involving humans collect information on gender, race and ethnicity as well as other characteristics of individuals that may influence how people respond.</p>	<p>Parent</p>		<p>X</p>	

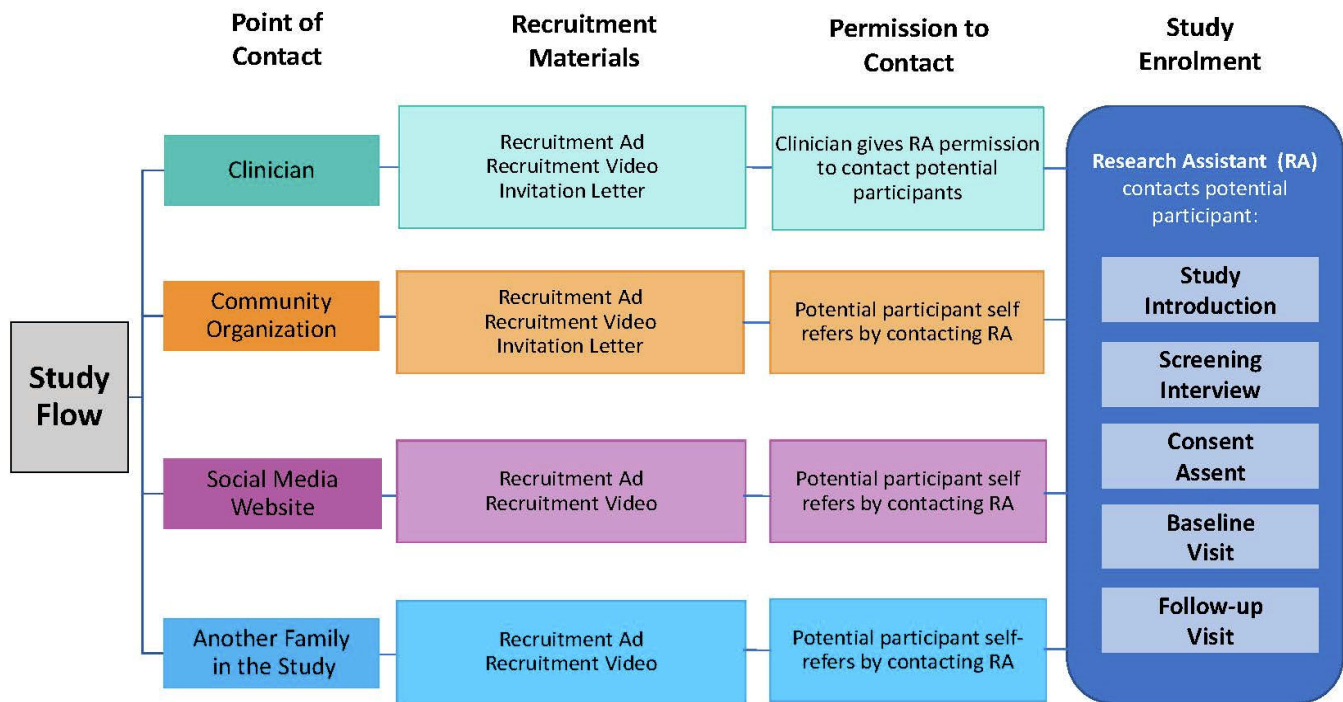
1 2 3	These questions will help us understand and describe the participants in CHILD-BRIGHT studies.				
4 5 6 7 8 9	<i>Profile Information Form</i> was developed by the CHILD-BRIGHT Network and includes questions about the child's functionalities and how certain factors might impact their quality of life. These questions will help us understand and describe the participants in CHILD-BRIGHT studies.	Parent		X	
10 11 12 13 14 15 16 17 18 19 20 21 22 23	<i>Measure of Process of Care (MPOC)</i> [12] The Measure of Processes of Care is a well-validated and reliable self-report measure of parents' perceptions of the extent to which the health services they and their child(ren) receive are family-centred. The original version of MPOC is a 56-item questionnaire; as of 1999 there is a shorter, 20-item version. MPOC has been used internationally in many evaluations of family-centred service. Parents/caregivers will complete the (modified with permission) MPOC-20 at Baseline and 6-Months.	Parent		X	X
24 25 26 27 28 29 30 31 32	<i>Health Utilities Index® (Hui2/3) Proxy-Assessed (health-related quality of life)</i> [13] The HUI is a generic health status instrument developed in Canada for use with children and has been incorporated in numerous clinical studies as well as the Canadian Community Health Survey, allowing the generation of norms for most age groups.	Parent		X	X
33 34 35 36 37 38 39 40 41 42 43 44 45 46 47 48	<i>Resource Use Questionnaire (RUQ)</i> [14] The RUQ is typically an interviewer-administered questionnaire for parents of children aged 11 to 18 years. The original RUQ measures the family resource use of condition-related treatments, services and programs, as well as parent time losses and family out-of-pocket costs. It also documents condition-related government subsidies and funding that families receive. Resources measured include those delivered by a parent, by other providers (e.g. behavioural specialist) or a combination of both. In this RCT, a modified subset of RUQ questions will be administered and completed by the parent/caregiver.	Parent		X	X

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Supplementary File 3: Recruitment Approach



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Supplementary File 4: Outline of E-Learning Training Modules for Research Staff

Name of Module	Summary of Content in the Module
Introduction and Team	Highlights the core team, core staff, patient and family advisory council (PFAC), recruitment sites, and funding.
Study Summary	A high-level overview of the study including purpose, objectives, study design, and expected outcomes.
Patient Engagement	Explains patient-oriented research, how patient and family engagement is incorporated into the study and refers research staff to the Patient-Oriented Research Curriculum in Child Health (PORCCH).
RA Responsibilities and Training	Reviews responsibilities of the RA regarding training, communication, administration and maintaining confidentiality.
Study Assessment Procedures	Explains the various ways that study assessments can be completed including how to set up a zoom meeting call.
Screening Interview	Reviews recruitment and all the steps required to complete the screening interview to determine if a potential participant is eligible for the study. Includes how to access and use instruments in REDCap.
Consent, Assent and Preparing for Baseline Visit	Reviews the verbal consenting and assenting process in REDCap and the Eligibility and Consent/Assent Status form. Information on completion methods for study measures and how to send them to the study participants is also included.
Study Measures	Reviews all the study measures for parent/caregiver, youth and those to be completed by the RA with the youth, for both baseline and follow-up visits. Videos on how to administer the COPM and NVS are included.
Randomization	Step by step information on how to run randomization in REDCap and information and videos of what it means to be in the control group or intervention group.
Concluding the Baseline Visit	Reviews participant appreciation, handout materials for youth and parent/caregiver, and information on the follow-up visit.
MyREADY Transition™ BBD App	Detailed information on the creation of the App, who it is designed for, how to access it, and an overview of the app itself. Information is also provided about the technical support website.
Follow-Up Visit	Reviews participant appreciation, and procedure for the follow-up visit after 6 months.
Information and Resources	Shows where to access other study documents and resources (e.g. in the File Repository in REDCap)
Contact Information	Contact information for RCT coordinators for ongoing support, to answer questions or if more information is required.

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Supplementary File 5: Study Objectives, Outcomes, Hypotheses and Analysis Methods

Objective	Outcome	Hypothesis	Method of Analysis
Primary: To determine whether the MyREADY Transition™ BBD App intervention will result in improved transition readiness	Primary: <ul style="list-style-type: none"> Change in TRAQ self-management score from Baseline to 6 months. 	Intervention > Control	ANCOVA
	Secondary: <ul style="list-style-type: none"> Change in TRAQ self-advocacy score from Baseline to 6 months. 	Intervention > Control	ANCOVA
	Secondary: <ul style="list-style-type: none"> Health care transition experience 	Individual semi-structured interviews	Qualitative Methods
Secondary: What is the effect of the MyREADY Transition™ BBD App intervention for improving health and use of health systems?	Primary: Population Health <ul style="list-style-type: none"> Serious illness (hospitalizations, ICU admission questions from Resource Use Questionnaire PedsQL™ Pediatric Quality of Life TRANSITION-Q 	Intervention > Control	ANCOVA for continuous outcomes Logistic regression for hospitalization
	Secondary: <ul style="list-style-type: none"> Utilization MyREADY Transition™ BBD App 	User metrics built into MyREADY Transition™ BBD App intervention to assess the extent to which various components of the intervention are accessed	Descriptive
	Secondary: Cost utility/cost-effectiveness <ul style="list-style-type: none"> Health Utilities Index® (Hui2/3) Resource Use Questionnaire 	Evaluate changes in patients' health in relation to changes in cost to assess if the intervention represents an efficient allocation of health care resources	Descriptive. Cost-effectiveness analysis with support from Child-Bright health economics network team
	Secondary: <ul style="list-style-type: none"> Achievement of health/life goals COPM 	Intervention > Control	Paired Student t-tests to compare mean ratings for performance and satisfaction on the COPM scoring system (10-point scale) with > 2 points difference as clinically meaningful difference

- 1 ANCOVA: Analysis of covariance
- 2 COPM: Canadian Occupational Performance Measure
- 3 HUI: Health Utilities Index®
- 4 ICU: Intensive Care Unit
- 5 TRAQ: Transition Readiness Assessment Questionnaire
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For peer review only

1 **Supplementary File 6: List of Recruitment Sites in Alberta, Ontario, Quebec, and the Maritimes.**
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4 Centre Hospitalier Universitaire (CHU) Sainte-Justine, Montreal, Quebec
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6 Children's Hospital of Eastern Ontario (CHEO), Ottawa, Ontario
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8 Glenrose Rehabilitation Hospital/Stollery Children's Hospital, Edmonton, Alberta
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10 Holland Bloorview Kids Rehabilitation Hospital, Toronto, Ontario
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13 IWK Health Centre, Halifax, Nova Scotia
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15 McMaster Children's Hospital, Hamilton, Ontario
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17 Montreal Children's Hospital, Montreal, Quebec
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20 Saint John Regional Hospital Department of Pediatrics, Saint John, New Brunswick
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22 St. Joseph's Health Care London/Children's Hospital London Health Sciences Centre, London, Ontario
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SPIRIT 2013 Checklist: Recommended items to address in a clinical trial protocol and related documents*

Section/item	Item No	Description	Addressed on Page No
Administrative information			
Title	1	Descriptive title identifying the study design, population, interventions, and, if applicable, trial acronym	1
Trial registration	2a	Trial identifier and registry name. If not yet registered, name of intended registry	12
	2b	All items from the World Health Organization Trial Registration Data Set	N/A
Protocol version	3	Date and version identifier	11
Funding	4	Sources and types of financial, material, and other support	15
Roles and responsibilities	5a	Names, affiliations, and roles of protocol contributors	1-2,14
	5b	Name and contact information for the trial sponsor	2
	5c	Role of study sponsor and funders, if any, in study design; collection, management, analysis, and interpretation of data; writing of the report; and the decision to submit the report for publication, including whether they will have ultimate authority over any of these activities	N/A
	5d	Composition, roles, and responsibilities of the coordinating centre, steering committee, endpoint adjudication committee, data management team, and other individuals or groups overseeing the trial, if applicable (see Item 21a for data monitoring committee)	N/A
Introduction			
Background and rationale	6a	Description of research question and justification for undertaking the trial, including summary of relevant studies (published and unpublished) examining benefits and harms for each intervention	4
	6b	Explanation for choice of comparators	7
Objectives	7	Specific objectives or hypotheses	4-5

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2	Trial design	8	Description of trial design including type of trial (eg, parallel group, crossover, factorial, single group), allocation ratio, and framework (eg, superiority, equivalence, noninferiority, exploratory)	5
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8	Methods: Participants, interventions, and outcomes			
9				
10	Study setting	9	Description of study settings (eg, community clinic, academic hospital) and list of countries where data will be collected. Reference to where list of study sites can be obtained	5 12, Supplementar y File 6
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17	Eligibility criteria	10	Inclusion and exclusion criteria for participants. If applicable, eligibility criteria for study centres and individuals who will perform the interventions (eg, surgeons, psychotherapists)	5-6
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23	Interventions	11a	Interventions for each group with sufficient detail to allow replication, including how and when they will be administered	6-7
24				
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27		11b	Criteria for discontinuing or modifying allocated interventions for a given trial participant (eg, drug dose change in response to harms, participant request, or improving/worsening disease)	N/A
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33		11c	Strategies to improve adherence to intervention protocols, and any procedures for monitoring adherence (eg, drug tablet return, laboratory tests)	6
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37		11d	Relevant concomitant care and interventions that are permitted or prohibited during the trial	7
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40	Outcomes	12	Primary, secondary, and other outcomes, including the specific measurement variable (eg, systolic blood pressure), analysis metric (eg, change from baseline, final value, time to event), method of aggregation (eg, median, proportion), and time point for each outcome. Explanation of the clinical relevance of chosen efficacy and harm outcomes is strongly recommended	7-8
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50	Participant timeline	13	Time schedule of enrolment, interventions (including any run-ins and washouts), assessments, and visits for participants. A schematic diagram is highly recommended (see Figure)	Figure 1
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55	Sample size	14	Estimated number of participants needed to achieve study objectives and how it was determined, including clinical and statistical assumptions supporting any sample size calculations	8-9
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2 Recruitment 15 Strategies for achieving adequate participant enrolment to 9,
3 reach target sample size Supplementar
4 y File 3
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6 **Methods: Assignment of interventions (for controlled trials)**
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8 Allocation:

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10 Sequence 16a Method of generating the allocation sequence (eg, 9
11 generation computer-generated random numbers), and list of any
12 factors for stratification. To reduce predictability of a
13 random sequence, details of any planned restriction (eg,
14 blocking) should be provided in a separate document that
15 is unavailable to those who enrol participants or assign
16 interventions
17
18
19 Allocation 16b Mechanism of implementing the allocation sequence (eg, 9
20 concealment central telephone; sequentially numbered, opaque, sealed
21 mechanism envelopes), describing any steps to conceal the sequence
22 until interventions are assigned
23
24
25 Implementation 16c Who will generate the allocation sequence, who will enrol 9
26 participants, and who will assign participants to
27 interventions
28
29
30 Blinding 17a Who will be blinded after assignment to interventions (eg, N/A
31 (masking) trial participants, care providers, outcome assessors, data
32 analysts), and how
33
34 17b If blinded, circumstances under which unblinding is N/A
35 permissible, and procedure for revealing a participant's
36 allocated intervention during the trial
37
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39 **Methods: Data collection, management, and analysis**

40 Data collection 18a Plans for assessment and collection of outcome, baseline, 7-11,
41 methods and other trial data, including any related processes to Supplementar
42 promote data quality (eg, duplicate measurements, y File 2
43 training of assessors) and a description of study
44 instruments (eg, questionnaires, laboratory tests) along
45 with their reliability and validity, if known. Reference to
46 where data collection forms can be found, if not in the
47 protocol
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51 18b Plans to promote participant retention and complete 6
52 follow-up, including list of any outcome data to be
53 collected for participants who discontinue or deviate from
54 intervention protocols
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2	Data	19	Plans for data entry, coding, security, and storage,	10
3	management		including any related processes to promote data quality	
4			(eg, double data entry; range checks for data values).	
5			Reference to where details of data management	
6			procedures can be found, if not in the protocol	
7				
8	Statistical	20a	Statistical methods for analysing primary and secondary	11,
9	methods		outcomes. Reference to where other details of the	Supplementar
10			statistical analysis plan can be found, if not in the protocol	y File 5
11				
12				
13		20b	Methods for any additional analyses (eg, subgroup and	11
14			adjusted analyses)	
15				
16		20c	Definition of analysis population relating to protocol non-	11
17			adherence (eg, as randomised analysis), and any	
18			statistical methods to handle missing data (eg, multiple	
19			imputation)	
20				
21				
22	Methods: Monitoring			
23				
24	Data monitoring	21a	Composition of data monitoring committee (DMC);	DMC not
25			summary of its role and reporting structure; statement of	needed; RCT
26			whether it is independent from the sponsor and competing	independent
27			interests; and reference to where further details about its	from sponsor
28			charter can be found, if not in the protocol. Alternatively,	
29			an explanation of why a DMC is not needed	
30				
31				
32		21b	Description of any interim analyses and stopping	N/A
33			guidelines, including who will have access to these interim	
34			results and make the final decision to terminate the trial	
35				
36	Harms	22	Plans for collecting, assessing, reporting, and managing	N/A
37			solicited and spontaneously reported adverse events and	
38			other unintended effects of trial interventions or trial	
39			conduct	
40				
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42	Auditing	23	Frequency and procedures for auditing trial conduct, if	10
43			any, and whether the process will be independent from	
44			investigators and the sponsor	
45				
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47	Ethics and dissemination			
48				
49	Research ethics	24	Plans for seeking research ethics committee/institutional	12
50	approval		review board (REC/IRB) approval	
51				
52	Protocol	25	Plans for communicating important protocol modifications	12
53	amendments		(eg, changes to eligibility criteria, outcomes, analyses) to	
54			relevant parties (eg, investigators, REC/IRBs, trial	
55			participants, trial registries, journals, regulators)	
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2	Consent or assent	26a	Who will obtain informed consent or assent from potential trial participants or authorised surrogates, and how (see Item 32)	9
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6		26b	Additional consent provisions for collection and use of participant data and biological specimens in ancillary studies, if applicable	N/A
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10	Confidentiality	27	How personal information about potential and enrolled participants will be collected, shared, and maintained in order to protect confidentiality before, during, and after the trial	10
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16	Declaration of interests	28	Financial and other competing interests for principal investigators for the overall trial and each study site	15
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19	Access to data	29	Statement of who will have access to the final trial dataset, and disclosure of contractual agreements that limit such access for investigators	N/A
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24	Ancillary and post-trial care	30	Provisions, if any, for ancillary and post-trial care, and for compensation to those who suffer harm from trial participation	N/A
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28	Dissemination policy	31a	Plans for investigators and sponsor to communicate trial results to participants, healthcare professionals, the public, and other relevant groups (eg, via publication, reporting in results databases, or other data sharing arrangements), including any publication restrictions	12
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35		31b	Authorship eligibility guidelines and any intended use of professional writers	N/A
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39		31c	Plans, if any, for granting public access to the full protocol, participant-level dataset, and statistical code	12
40				
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42	Appendices			
43				
44	Informed consent materials	32	Model consent form and other related documentation given to participants and authorised surrogates	N/A
45				
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47	Biological specimens	33	Plans for collection, laboratory evaluation, and storage of biological specimens for genetic or molecular analysis in the current trial and for future use in ancillary studies, if applicable	N/A
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*It is strongly recommended that this checklist be read in conjunction with the SPIRIT 2013 Explanation & Elaboration for important clarification on the items. Amendments to the protocol should be tracked and dated. The SPIRIT checklist is copyrighted by the SPIRIT Group under the Creative Commons "[Attribution-NonCommercial-NoDerivs 3.0 Unported](https://creativecommons.org/licenses/by-nc-nd/3.0/)" license.