FuRST 2.0 Data Dictionary
Public and Specified Datasets

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FuRST 2.0

Cognitive Pre-Testing for a New Functional Rating Scale for Use
in Huntington's Disease

A CHDI Foundation, Inc. Project

Table of Contents

Abbreviations

CAG	Cytosine, Adenine, Guanine
CRF	Case Report Form
CTR	Centre for Trials Research
DBS	Disease Burden Score
DCL	Diagnostic Confidence Level
FuRST	Functional Rating Scale Taskforce
HD	Huntington's Disease
HDGEC	Huntington's Disease Gene Expansion Carriers
ICF	Informed Consent Form
PDS	Periodic Dataset
PI	Principal Investigator
PRO	Patient Reported Outcome
RCT	Randomised Controlled Trial
SCR	Scientific Review Committee
SPS	Specified Datasets
SRC	Scientific Review Committee
TFC	Total Functional Capacity

1 Overview

This Data Dictionary is intended to be used with all FuRST 2.0 dataset releases, including public datasets (PDS) and specific datasets (SPS).

This document contains variable information, including availability of the data release type in different types of datasets. There is also a brief overview of the constituent data sources, dataset structure, variable structure, and representation of special values.

2   Structure of the Dataset

2.1   Data Sources and Representation within the Dataset

The Functional Rating Scale Task Force (FuRST) was formed in 2010 to develop a functional rating scale for Pre-Manifest HD patients. This work resulted in the first iteration of the FuRST rating scale (FuRST 1.0). After further evaluation by rating scale development experts, it was decided not to move forward with validation due to problems with preliminary clinometric results and the cumbersome nature of the structured interview methodology. FuRST 2.0 builds on previous work to develop a functional rating scale that is clinimetrically robust and easy to administer in the clinical research setting.

The overall goal of the FuRST 2.0 program is to use state-of-the-art clinometric techniques to develop a valid and reliable functional abilities measure for use in Pre-Manifest and Early-Manifest Huntington Disease Gene Expansion Carrier (HDGEC).

In the current study, these draft items will be subjected to cognitive pre-testing using HDGECs, companions and interviewers to assess the ease-of-use of the scale, the ease of comprehension of individual items and rating anchor definitions, applicability of individual items and rating anchors, level of insight as perceived by the interviewer, as well as the comfort with addressing specific issues that may be sensitive to the participants, companions or interviewers.

FuRST study is divided into 2 rounds, and this is explained in more detail on section 2.6.

2.2   Data Files within the Dataset

FuRST 2.0 PDS releases are comprised of 3 data files, each of which falls into one of two categories:

Participant-based: *profile*

This contains general study-independent information about the participant. This information is applicable to all studies.

Visit-based: *furst2_round1, furst2_round2*

These contain all visit-dependent information for the study, combined into one data file.

Each PDS data file is described in **Table *1***.

Table 1 - FuRST 2.0 dataset release data file descriptions.

Data file	Type	Study	Description
profile	*participant*	FuRST 2.0	General and participant related data. This includes: sex and in which round the participant took part it.
furst2_round1	*visit*	FuRST 2.0	Visit-specific information about which assessments were performed at each visit on Round 1 of the study.
furst2_round2	*visit*	FuRST 2.0	Visit-specific information about which assessments were performed at each visit on Round 2 of the study.

2.3   Structure of Variables

This Data Dictionary lists all variables with the appropriate attributes specified in **Table 2**.

Table 2 - Data dictionary column fields.

Attribute	Description
Label	Variable label.
Domain	Correspondent domain (optional).
Category	Additional information about the category of the variable (optional).
Variable	Internal variable name.
Type	*Boolean:* Represents the values yes and no. *Number:* Represents integer or floating-point data values. *Text:* Represents alphanumeric string data values. *Free Text:* Represents long strings of text. *Date:* The date type is represented as the number of days relative to the date of the participant's FuRST 2.0 visit date. Note that dates that have been specified in the original data as "incomplete" (e.g. without entry of a day) have been automatically completed by the following rule: use "15" as day if day is missing and use "1" as day and "7" as month if day and month is missing. After this completion the date modification is complete, the number of days relating to the enrollment date is calculated and provided in the dataset. The information about whether a date has been automatically completed is not included in the PDS but can be obtained via SPS request. *Single choice:* Variable with assigned list of options where one item can be selected. The value provided in the dataset is taken from the available options list. The list of options is defined as parameters in the data dictionary tables.
Coding & Parameter	Parameter value of coded variables (optional).
Description	Further information about the variable, like formulas used to calculate the values, and references to papers with more details about the variable.
Transformation	One important objective of the periodic dataset is to de-identify the FuRST 2.0 data in order to minimize the possibility to identify a participant. Therefore, many variables are transformed, recoded or outliers removed/cut. These transformations are described on a variable basis.
Availability	All variables in the FuRST 2.0 dataset are listed in the Data Dictionary. This availability column allows the researcher to identify which variables are available in the PDS ("PDS"), which are available via special request ("Available upon SRC approval"), and which are restricted ("Not Available").

2.4   Representation of Special Values

Date Values

Transformation of date values

To minimize participant identification risk, the FuRST 2.0 PDS does not contain date values. Date values referring to visit dates are transformed to a numeric value, reflective of the number of days between the FuRST 2.0 baseline visit date and the date of interest.

For example, date values for a participant with a baseline visit date of 01/11/2019 (DD/MM/YYYY) would read as follows:

Entered date	Representation in dataset
01/11/2019	0
30/11/2019	29
31/10/2019	-1

In this study, since only one visit is conducted in each round, the days since baseline visit (*visdy*) are defined as 0.

Missing Values

There are two overarching categories of missing data in the dataset: system-defined missing data (indicated by blank variable 'entries'), and user-defined missing data (indicated by specific codes, which indicate reason for missingness).

System defined missing data occurs where the system dictates a missing variable field. These missing data values are indicated by **blank entries** in the dataset.

That happens for both rounds only for question/items:

-    item99_question3_1p

-    item99_question3_1l

-    item99_question3_1w

-    item99_question3_1r

-    item99_question3_1o

-    item99_question3_1o_com

This user-defined label - 'exceptional values' – is listed below. Each is represented in the dataset by a **specific code**:

Missing data is coded by the interviewers as Not Available, None, NA or Nothing (for both rounds) or 0 (only applicable on round 2).

Harmonization of missing values words

In the free text variable, some expressions with the same meaning were written in different ways. To harmonize these expressions, they were converted to be all equal across all the study (that is, applicable to **both rounds**).

- *none, none -, none,, None., 'None', none., "none"* were converted into *None*;
- *N/A -, N/A., NA, NA., not applicable, na* were converted into *N/A*;
- *'No', no, no., non, nope, Nope* were converted into *No*;
- *nothing, 'Nothing', Nothing., 'nothing.', "nothing"* were converted into *Nothing*.

Zero "0" as not available

In addition, to the usual missing data codes cited above, **only on round 2** of the FuRST 2.0 study, 0 can also be used as missing data code.

In non-numeric variables, wherever a 0 appears, it means that no answer was given by the participant and/or companion to that question.

2.5   Data Availability

This Data Dictionary details all variables collected in FuRST 2.0 study.

The **public dataset**, prepared from the FuRST 2.0 database, include *some of the variables* collected. Variables included in the PDS are highlighted in green. The FuRST 2.0 PDS goes through stringent quality control and de-identification procedures prior to release. To this end, certain variables can be transformed, aggregated, or suppressed (excluded) in the PDS to minimize participant identification.

Access to non-transformed, de-aggregated, or suppressed data may be obtained via **specified dataset** (**SPS**) request, pursuant to review and approval by the Enroll-HD Scientific Review Committee (SRC). These variables are highlighted in orange.

Certain variables are restricted, typically due to their highly identifying nature. These variables, highlighted in red, and not available via PDS or SPS request, but are included here for completeness.

The variable column 'Availability' indicates these three classes of variables using the color-coding shown below:

Availability	Meaning
PDS	Included in the PDS.
Available upon SRC approval	Not included in the PDS but available via SPS request contingent on SRC approval.
Not available	Restricted variables which are not available in the PDS or via SPS request.

2.6   FuRST 2.0: Differences between rounds

The main purpose of FuRST 2.0 of is to identify real or potential comprehension or usage problems with questionnaire items or response options. Through a process of structured cognitive de-briefing with HDGEC participants and companions, independently, followed by qualitative analysis, the final phrasing of the individual items and response options for the scale will be generated. Depending on the results of the first round of cognitive pretesting, additional rounds of cognitive pre-testing may be required. The analysis of observational and verbal reports will mainly focus on identifying:

1) Complexity or length of questions and response options that may inhibit understanding;
2) Words and concepts used in the items that respondents do not understand or understand differently;
3) Questions that respondents cannot answer accurately;
4) Scaling severity choice difficulties;
5) Questions that are strongly influenced by cultural meaning and norms or that make respondents uncomfortable;
6) Suggestions for better wording and other changes for modification.

After the initial round, recommended modifications to FuRST 2.0 scale items, response options, instructions and disclaimer statement, if warranted by the qualitative analysis results were taken into consideration to develop the round 2 of the study. On this round, the cross-sectional cognitive interview of functional rating scale administered to HDGECs and potentially, their companions. The scale will be tested as a patient reported outcome (PRO) in that the information will come directly from the HDGEC participant or the HDGEC participant together with his/her companion through self-report. Once again, the purpose is to identify real or potential comprehension or usage problems with scale items, response options, instructions and disclaimer statement. Through a structured cognitive interview with the HDGEC participants or the HDGEC participants together with their companions, followed by qualitative analysis, the final phrasing of the individual scale items, response options, instructions and disclaimer statement for the scale will be generated.

Table 3 briefly explains the main differences between the two rounds of FuRST 2.0, in particular the recruitment criteria, items answered and their answer options, and questions asked. To further details, please consult the study protocol for each round and their protocol amendments.

Table 3 - Main differences on protocol between the two rounds of the FuRST 2.0 study.

	Round 1	Round 2
Completing the questionnaire	If the HDGEC participant has a companion, they fulfill the questionnaire separately.	If the HDGEC participant has a companion, they fulfill the questionnaire together.
Overall inclusion criteria for participants	- HDGEC participant must be a participant in Enroll-HD - At least 18 years of age - Must be fluent in English and had his primary education in English - Must be willing and able to provide written informed consent.	- Identified as an active participant in Enroll-HD (participants who have completed their last onsite Enroll-HD visit within approximately 15 months) - At least 18 years of age - Fluent in English and had his/her primary education in English - Able and willing to provide critical feedback (per site principal investigator (PI) or site PI's designee discretion) - Willing and able to provide written informed consent
Pre-Manifest HD inclusion criteria	- CAG length ≥ 40 - Disease Burden Score greater than or equal to 250 (calculated by the equation: $[CAGn-35.5] \times age$) - Diagnostic Confidence Level (DCL) ≤ 3 - At least five Pre-Manifest HDGEC participants should have a companion who is willing to participate in the study and complete the scale independently.	- CAG length ≥ 40 - Disease Burden Score ≥ 250 (calculated by the equation: $[CAGn-35.5] \times age$) - Diagnostic Confidence Level (DCL) ≤ 3
Early Manifest HD Inclusion Criteria	- CAG length ≥ 36 - Diagnostic Confidence Level (DCL) = 4 - TFC ≥ 7 - Participants whose companion is willing to participate in the study and complete the scale independently	- CAG length ≥ 36 - Diagnostic Confidence Level (DCL) = 4 - TFC ≥ 11
Items FuRST 2.0	- 22 questions (see section 2.7.1) - 5 response options (Normal, Slight, Mild, Moderate, Severe) (see section 2.7.2)	- 24 questions (see section 2.7.3) - 4 response options (No, Mild, Moderate, Severe) (see section 2.7.4)
Cognitive Test Questions	- Item 0: Cognitive Test Questions for the interviewer and participant/companion regarding the questionnaire instructions. - Item 99: Debriefing interviewer and participant (after completing the questionnaire).	- Item 0: Cognitive Test Questions for the Interviewer and participant/companion regarding the questionnaire instructions - Item 98: Cognitive Test Questions for the participant and interviewer regarding the questionnaire disclaimer. - Item 99: Debriefing interviewer and participant (after completing the questionnaire).

2.7   Questionnaire

As explained in the previous section, one of the main changes between the two rounds of the FuRST 2.0 study was the improvement on the items assessed, taking into consideration the feedback provided, and the answer options were also revised.

The interviewer case report form (CRF) has a similar struture on both rounds, with the exception of the Item 98, only available for round 2 of the study. Item 0 is fulfiled before the Furst 2.0 questions being assessed, while the Items 98 and 99 are completed after the questionnaire items. As pointed on **Table 3**, the number of items and the response options are different between rounds for question 0 of the Items 1-22 (Round1) and 1-24 (Round2). The list of questions of Round 1 is available on section Questions - Round 12.7.1 and the question of Round 2 are available on section 2.7.3.

Table 4 - Questions replied on Item 0 for both rounds.

Item 0
Cognitive Test Questions for the Interviewer
1. What difficulties did you observe or hear voiced while watching the HDGEC participant read the questionnaire instructions?
Cognitive Test Questions for the HDGEC participant regarding the questionnaire instructions
2. How easy or difficult were these instructions for you to understand?
3. Which parts of the instructions were difficult to understand? What was the difficulty?
3.1 Explain why the language in the instructions made them difficult in the space below.

Table 5 - Questions on the Questionnaire for both rounds.

Items 1 – 22 (Round 1) **Items 1 – 24** (Round 2)
Questionnaire Items
0. Please write in the study participant(s) self-rating here.
Document the interviewer's answers to cognitive test questions 1 and 2 while the study participant(s) is/are reading/responding to the FuRST 2.0 item
1. What issues with the FuRST 2.0 item did you observe while the study participant(s) was/were reading or interpreting the question?
2. What issues with the FuRST 2.0 response options did you observe while the study participant(s) was/were reading or interpreting the response options?
Cognitive Test Questions to be used with the study participant(s) after they have read and responded to the FuRST2.0 item
3. Which parts of the question, if any, seemed difficult to understand?
3.1 Please tell me what made them difficult.
3.2 In your own words, what might be a better way to ask this question?
4. Overall, how easy or difficult was this question for you to understand?
5. What, if anything, made it difficult for you to select an appropriate response option?
5.1 How might you revise the response options to make selecting a response easier?
6. Overall, how easy or difficult was it for you to select a response option for that question?

Table 6 - Questions replied on Item 98 (only applicable on Round 2).

Item 98
Cognitive Test Question for the Interviewer
1. What difficulties did you observe or hear voiced while watching the study participant(s) read the questionnaire disclaimer?
Cognitive Test Questions for the study participant(s) regarding the questionnaire disclaimer
2. How easy or difficult was this disclaimer for you to understand?
3. Which parts of the disclaimer were difficult to understand? What was the difficulty?
3.1 Explain why the language in the disclaimer made it difficult in the space below.

Table 7 - Questions replied on Item 99 for both rounds.

Item 99
Study participant(s) debriefing after completing the FuRST 2.0 questionnaire
1.0 Overall, did you find the assessment questions in the questionnaire difficult?
1.1 If so, what made them difficult?
2.0 Did you find any of the assessment questions in the questionnaire upsetting?
2.1 If so, what made them upsetting?
3.0 Did you find the questions or the response options hard to read?
3.1 If yes, check all reasons that apply. - Print was too small to read easily - Lines were too close together - Words were difficult to understand - Response options were not appropriate for me - Other
Interviewer debriefing
4. Which, if any, of the questions in the FuRST 2.0 questionnaire seemed to make the study participant(s) uncomfortable? Why? Note: if information is available, please also indicate if it was the HDGEC participant or the companion who was made uncomfortable.
5. Other comments to help improve the FuRST 2.0 questionnaire items, disclaimer statement or instructions?

On the Data Dictionary, available on an Excel file, the variables associated with these items are represented as follows:

it*xx*_question*yy*, where *xx* refers to the item number and *yy* to the question number.

On Round 2, some of these items have the information on who replies to that question. On those cases, the suffixe _*resp* has been added to the variable name with the same structure to inform who completed the question.

Example

Item	Question	Respondent	Variable name in dataset
Item 20	Question 4	-	item20_question4
Item 99	Question 2.1	-	item99_question2_1
Item 0	Question 3.1	-	item00_question3_1
Item 22	Question 3.2	Yes	item22_question3_2_resp

All items and questions follow this model, with the exception of Item 99, question 3.1, that was transformed from a multiple choice variable into several boolean variables and an additional variable for the comments for the participants that chose the option *Other*. Therefore, these variables will be available as it follows:

Response option	Variable name in dataset
Print was too small to read easily	item99_question3_1p
Lines were too close together	item99_question3_1l
Words were difficult to understand	item99_question3_1w
Response options were not appropriate for me	item99_question3_1r
Other	item99_question3_1o
Other – Comments	item99_question3_1o_com

2.7.1   Questions - Round 1

1. Over the past two weeks, how well are you communicating with other people? (For example, joining in conversations or staying in touch by telephone, texting or email)

2. Over the past two weeks, how well are you able to work around the house or at your job? (For example, not making mistakes or finishing everything you wanted to get done)

3. Over the past two weeks how well are you managing your finances? (For example, being careful using your money or keeping track of how much money you have or paying your bills)

4. Over the past two weeks how well are you handling your cash or credit cards? (For example, remembering pin numbers, finding your credit cards or taking money out of your pocket, wallet or handbag easily)

5. Over the past two weeks how well can you get started doing the activities you usually do?

6. Over the past two weeks, how well can you plan your day-to-day activities?

7. Over the past two weeks how well are you getting around? (For example, getting around in a car, bus or train, or knowing how to get somewhere)

8. Over the past two weeks how well are you walking? (For example, feeling steady on your feet, going up or down stairs or walking smoothly)

9. Over the past two weeks how well are you doing your hobbies or other activities you enjoy?

10. Over the past two weeks how well are you using your hands? (For example typing, writing, turning pages, using a knife, picking things up, carrying a full cup, or turning a key)

11. Over the past two weeks how well are you talking? (For example, saying what you mean to say or having others understand what you are saying)

12. Over the past two weeks how well are you able to stay clean and neat? (For example, bathing, combing your hair, doing makeup, shaving, brushing teeth, or cutting your nails)

13. Over the past two weeks how well are you able to change your clothes or get dressed? (For example, standing on one foot to put on underclothes or pants, do up buttons and zippers, put on jewelry or tie your shoe laces)

14. Over the past two weeks how well are you keeping to your daily routine? (For example, getting up, going to bed or eating meals at your usual times)

15. Over the past two weeks how well are you getting to work or appointments on time?

16. Over the past two weeks how well are you keeping your home, garden or car clean?

17. Over the past two weeks how well are you able to do exercises you want to do? (For example, walking, jogging, swimming, or playing a sport)

18. Over the past two weeks how well are you able to keep interested in what's going on? (For example, listening to news, reading a paper or searching the Internet for updates in world affairs, sports, weather)

19. Over the past two weeks how well are you able to control your temper? (For example, not getting into more arguments than usual or not getting more irritated)

20. Over the past two weeks have you had trouble with your sexual interests or function?

21. Over the past two weeks how well are you able to drive a car? (For example, controlling your speed or steering)

22. Over the past two weeks how well are you able to sleep normally? (For example, sleeping through the night or staying awake during the day)

2.7.2   Answer Options – Round 1

0. **Normal**. No problems.

1. **Slight**: I notice some problems, but they cause no real difficulty.

2. **Mild**: These problems cause only a few difficulties.

3. **Moderate**: These problems cause more than a few difficulties.

4. **Severe**: These problems cause a lot of difficulty or prevent me from doing these activities.

2.7.3   Questions – Round 2

1. Over the past two weeks, did you have difficulty interacting with other people in person or over the phone?

2. Over the past two weeks, did you have difficulty communicating with other people using electronic devices? Some examples are difficulty texting or using email.

3. Over the past two weeks, did you have difficulty doing chores around the house?

4. Over the past two weeks, did you have difficulty working at your job? Some examples are making mistakes or not finishing everything.

5. Over the past two weeks did you have difficulty using cash or credit cards? Some examples are difficulty remembering PIN numbers, finding your credit cards or taking money out of your pocket, wallet or handbag.

6. Over the past two weeks did you have difficulty managing your finances? Some examples are difficulty keeping track of your bank accounts or paying your bills.

7. Over the past two weeks, did you have difficulty planning your day-to-day activities?

8. Over the past two weeks did you have difficulty getting started with your day-to-day activities?

9. Over the past two weeks did you have difficulty getting to where you needed to go without assistance? Some examples are difficulty getting in or out of a car, bus or train, or difficulty knowing how to get somewhere.

10. Over the past two weeks did you have difficulty walking? Some examples are difficulty feeling steady on your feet, going up or down stairs, or walking smoothly.

11. Over the past two weeks did you have difficulty doing activities you enjoy?

12. Over the past two weeks did you have difficulty using your hands? Some examples are difficulty picking things up, carrying a full cup, or turning a key.

13. Over the past two weeks did you have difficulty with your speech? Some examples are difficulty saying what you meant to say or having others understand what you said.

14. Over the past two weeks did you have difficulty grooming yourself? Some examples are difficulty bathing, combing your hair, or brushing your teeth.

15. Over the past two weeks did you have difficulty dressing yourself? Some examples are difficulty using buttons and zippers, putting on jewelry or tying your shoe laces.

16. Over the past two weeks did you have difficulty keeping to your day-to-day routine without assistance? Some examples are difficulty getting up, going to bed or eating meals at your usual times.

17. Over the past two weeks did you have difficulty getting to events on time without assistance? Some examples are difficulty getting to movies, family gathering or other appointments on time.

18. Over the past two weeks did you have difficulty keeping your surroundings clean without assistance? Some examples are difficulty cleaning your bedroom, the kitchen, or your car.

19. Over the past two weeks did you have difficulty exercising? Some examples are difficulty walking, jogging, swimming, or playing a sport.

20. Over the past two weeks did you have difficulty staying involved in what is going on around you? Some examples are difficulty staying up-to-date with important news or events.

21. Over the past two weeks did you have difficulty controlling your temper? Some examples are getting irritated or into arguments.

22. Over the past two weeks did you have difficulty with your sexual activities?

23. Over the past two weeks did you have difficulty driving a car?

24. Over the past two weeks did you have difficulty sleeping? Some examples are difficulty falling asleep, difficulty sleeping through the night, or waking too early.

2.7.4   Answer Options – Round 2

a. **No**: I had no difficulty doing this.

b. **Mild**: I had difficulty doing this but could still do it well.

c. **Moderate**: I had difficultly doing this well.

d. **Severe**: I could barely do this or I could not do it at all.