Clinical Practice/ Routine Monitoring Version Type 1 Gaucher Disease Patient Reported Outcome Measure (rmGD1-PROM)

PART 1: Please complete each of the questions by putting a checkmark (√) in the box which matches your best answer

| Question                                                                 | None of the time | A little of the time | Some of the time | Most of the time | All of the time | Not applicable or prefer not to say |
|-------------------------------------------------------------------------|------------------|----------------------|------------------|------------------|----------------|-----------------------------------|
| 1 Over the past month, my Gaucher disease has restricted my education/job|                   |                      |                  |                  |                |                                   |
| 2 Over the past month, my Gaucher disease has restricted my activities with friends|                   |                      |                  |                  |                |                                   |
| 3 Over the past month, my Gaucher disease has restricted my ability to have intimate relationships with my spouse/partner|                   |                      |                  |                  |                |                                   |
| 4 Over the past month, my Gaucher disease has restricted my ability to take part in hobbies and leisure activities|                   |                      |                  |                  |                |                                   |
| 5 Over the past month, I have been concerned that I am an emotional burden to others because of my Gaucher disease|                   |                      |                  |                  |                |                                   |
| 6 Because of my Gaucher disease, I am concerned I will be at risk of bone disease|                   |                      |                  |                  |                |                                   |
| 7 Because of my Gaucher disease, I am concerned I will be at risk of cancers|                   |                      |                  |                  |                |                                   |
| 8 Because of my Gaucher disease, I am concerned I will be at risk of Parkinson's disease|                   |                      |                  |                  |                |                                   |
| 9 Because of my Gaucher disease, I am concerned I will be a financial burden|                   |                      |                  |                  |                |                                   |

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10 I am concerned I will not get the best therapy because of budget issues

11 I am concerned I may not have an expert physician for advice in the future

12 My non-Gaucher problems are more concerning than the Gaucher concerns

13 Over the past month, my health in general has improved because of my Gaucher-specific medication

14 Over the past month, all of my medical concerns have been Gaucher-related

15 Over the past month, my current medication has treated my Gaucher-specific concerns
PART 2: Thinking about your Gaucher disease over the past week, please circle the number that is right for you

1. Over the past week, how dependent on others have you been because of your Gaucher disease?

   Totally independent

   0  1  2  3  4  5  6  7  8  9  10

   Totally dependent

2. Over the past week, how visibly big or swollen has your abdomen looked because of your Gaucher disease?

   Not big or swollen

   0  1  2  3  4  5  6  7  8  9  10

   Very big or swollen

3. Over the past week, how fatigued have you been because of your Gaucher disease?

   Not at all fatigued

   0  1  2  3  4  5  6  7  8  9  10

   Severely fatigued

4. Over the past week, how physically weak have you been feeling because of your Gaucher disease?

   No physical weakness

   0  1  2  3  4  5  6  7  8  9  10

   Severe physical weakness

5. Over the past week, how severe has your bone pain been because of your Gaucher disease?

   No bone pain

   0  1  2  3  4  5  6  7  8  9  10

   Severe bone pain
6. Over the past week, how **depressed** have you been because of your Gaucher disease?

Not at all depressed  |  Severely depressed
---|---
0 | 1 | 2 | 3 | 4 | 5 | 6 | 7 | 8 | 9 | 10

7. Over the past week, how **worried** have you been because of your Gaucher disease?

Not at all worried  |  Very worried
---|---
0 | 1 | 2 | 3 | 4 | 5 | 6 | 7 | 8 | 9 | 10

8. Over the past week, how have you felt about your **future** with Gaucher disease?

Very optimistic  |  Very pessimistic
---|---
0 | 1 | 2 | 3 | 4 | 5 | 6 | 7 | 8 | 9 | 10

9. Over the past month, how **satisfied** have you been with your Gaucher medical treatment?

Very Satisfied  |  Very dissatisfied
---|---
0 | 1 | 2 | 3 | 4 | 5 | 6 | 7 | 8 | 9 | 10

Thank you for completing this form