Patient- and caregiver-reported burden of transfusion-dependent β-thalassemia measured using a digital application

The Patient – Patient-Centered Outcomes Research

Clark Paramore¹, Laurice Levine², Emma Bagshaw³, Chengyu Ouyang³, Amber Kudlac³, Mark Larkin³

1bluebird bio, Cambridge, Massachusetts, US, ²Self-employed medical consultant, San Francisco, California, US, ³Vitaccess Ltd, London, UK

Corresponding author

Emma Bagshaw; emma.bagshaw@vitaccess.com
### Online resource 1 Disease-management survey

| Question                          | Individual with TDT version                                                                 | Caregiver version                                                                 | Branching logic |
|----------------------------------|---------------------------------------------------------------------------------------------|-----------------------------------------------------------------------------------|-----------------|
| Introduction                     | This is a daily survey.                                                                     | This is a daily survey.                                                           |                 |
|                                  | Introduction: These questions help us to learn about how you manage your thalassemia on a day-to-day basis. | Introduction: These questions help us to learn about how your child manages his/her thalassemia on a day-to-day basis. |                 |
|                                  | If possible, please try to take this survey later on in the day when you can recall events about that day. | If possible, please try to take this survey later on in the day when you can recall events about that day. |                 |
| 1.                               | **When did you last receive a transfusion for your thalassemia?**                            | **When did your child last receive a transfusion for their thalassemia?**          |                 |
|                                  | o [dd-mm-yyyy]                                                                             | o [dd-mm-yyyy]                                                                   |                 |
|                                  | o It was today                                                                             | o It was today                                                                    |                 |
| 2.                               | **Are you sure the last transfusion date is still correct?**                               | **Are you sure the last transfusion date is still correct?**                      |                 |
|                                  | If within the last 7 days, go to 3                                                        | If not within the last 7 days, go to 9                                             |                 |
|                                  | If a duplicate date with previous responses, go to 9                                      |                                                                                  |                 |
|   |   |   |
|---|---|---|
| 3. | What was your hemoglobin (Hb) level before this transfusion? (g/dL) | What was your child’s hemoglobin (Hb) level before this transfusion? (g/dL) |
|   | o [dd-mm-yyyy] | o [dd-mm-yyyy] |
|   | o Yes | o Yes |
| 4. | How much time did you spend travelling to your transfusion appointment? | How much time did your child spend travelling to their transfusion appointment? |
|   | *Do not include time you might have spent on other errands during the travel time.* | *Do not include time they might have spent on other errands during the travel time.* |
| 5. | How much time did you spend waiting for your transfusion? | How much time did your child spend waiting for their transfusion? |
|   | *Include all the time from when you arrived at the health center until your transfusion started.* | *Include all the time from when they arrived at the health center until their transfusion started.* |
| 6. | How long did your transfusion take, from start to finish? | How long did their transfusion take, from start to finish? |
|   | *Include all the time from when you were called through for your* | *Include all the time from when your child was called through for* |
|   |   |   | Go to 7 |
|    | appointment, sitting in the transfusion chair, speaking with the medical staff, through to when you left the center. | their appointment, sitting in the transfusion chair, speaking with the medical staff, through to when they left the center. |    |
|----|------------------------------------------------------------------------------------------------|------------------------------------------------------------------------------------------------|----|
| 7. | How much time did you spend travelling after your transfusion appointment (e.g. to school or home)? *Do not include time you might have spent on other errands during the travel time.* | How much time did your child spend travelling after their transfusion appointment (e.g. to school or home)? *Do not include time they might have spent on other errands during the travel time.* | Go to 8 |
| 8. | Have you spent any other time in the last 24 hours managing your thalassemia, in addition to your transfusion? *This includes time spent making appointments, organizing medicines, preparing and taking iron chelators, separate blood matching (e.g. type or cross matching) visits, arranging childcare, organizing time off school, college, or work, and organizing payments from health insurance.* | Have you or your child spent any other time in the last 24 hours managing their thalassemia, in addition to their transfusion? *This includes time spent making appointments, organizing medicines, preparing and giving iron chelators, separate blood matching (e.g. type or cross matching) visits, arranging childcare, organizing time off school, college, or work, and organizing payments from health insurance.* | If Yes, go to 10  
If No, go to end |
|   | 9. | Have you spent any time in the last 24 hours managing your thalassemia? This includes time spent making appointments, organizing medicines, preparing and taking iron chelators, separate blood matching (e.g. type or cross matching) visits, arranging childcare, organizing time off school, college, or work, and organizing payments from health insurance. | Have you or your child spent any time in the last 24 hours managing their thalassemia? This includes time spent making appointments, organizing medicines, preparing and giving iron chelators, separate blood matching (e.g. type or cross matching) visits, arranging childcare, organizing time off school, college, or work, and organizing payments from health insurance. | If Yes, go to 10 | If No, go to end |
|---|---|---|---|---|---|
|   |   | o Yes | o Yes |   |   |
|   |   | o No | o No |   |   |
|   | 10. | Which of the following did you do in the last 24 hours because of your thalassemia? Select all that apply. | Which of the following did you or your child do in the last 24 hours because of their thalassemia? Select all that apply. | If any activities selected, go to first activity | If None of these, go to end |
|   |   | o Made appointments for future blood transfusions | o Made appointments for future blood transfusions |   |   |
|   |   | o Organized medicines for your thalassemia treatment | o Organized medicines for their thalassemia treatment |   |   |
| Step | Description |
|------|-------------|
| 11.  | How much time did you spend in the last 24 hours making appointments for future blood transfusions? |
|      | Think about time spent on the telephone, writing emails etc. |
|      | [Number of hours and minutes] |

How much time did you or your child spend in the last 24 hours making appointments for future blood transfusions?
Think about time spent on the telephone, writing emails etc.
Add together any time you spent on this task with any time your child spent on this task.

If other activities selected in 10, go to next selected activity.
If no other activities selected...
|   | How much time did you spend in the last 24 hours organizing medicines for your thalassemia treatment?  
Include time spent writing emails, making phone calls, collecting medicines etc.  
[Number of hours and minutes] | How much time did you or your child spend in the last 24 hours organizing medicines for his/her thalassemia treatment?  
Include time spent writing emails, making phone calls, collecting medicines etc.  
Add together any time you spent on this task with any time your child spent on this task.  
[Number of hours and minutes] | If other activities selected in 10, go to next selected activity  
If no other activities selected, go to end |
|---|---|---|---|
| 12. | | | |
| 13. | How much time did you spend in the last 24 hours preparing and taking iron chelators?  
[Number of hours and minutes] | How much time did you spend in the last 24 hours preparing and giving iron chelators?  
[Number of hours and minutes] | If other activities selected in 10, go to next selected activity  
If no other activities selected, go to end |
|   | How much time in the last 24 hours did you spend on blood matching, if this was done at a separate visit from the transfusion? *Include travel time to and from the blood matching visit and time spent waiting.* | How much time in the last 24 hours did you or your child spend on blood matching, if this was done at a separate visit from the transfusion? *Include travel time to and from the blood matching visit and time spent waiting.* | If other activities selected in 10, go to next selected activity |   |
|---|---|---|---|---|
| 14. | [Number of hours and minutes] | [Number of hours and minutes] |   |   |
| 15. | How much time did you spend in the last 24 hours arranging childcare for your family while you received treatment for your thalassemia? *Include time spent writing emails, making phone calls, speaking with people etc.* | How much time did you spend in the last 24 hours arranging childcare for your family while your child received treatment for their thalassemia? *Include time spent writing emails, making phone calls, speaking with people etc.* Add together any time you spent on this task with any time your child spent on this task. | If no other activities selected, go to end |   |
| 16. | How much time did you spend in the last 24 hours organizing time | How much time did you or your child spend in the last 24 hours | If other activities |   |
|   | off work, college, or school so you could receive treatment for your thalassemia? Include time spent writing emails, making phone calls, speaking with people etc. [Number of hours and minutes] | organizing time off work, college or school so that your child could receive treatment for their thalassemia? Include time spent writing emails, making phone calls, speaking with people etc. Add together any time you spent on this task with any time your child spent on this task. [Number of hours and minutes] | selected in 10, go to next selected activity If no other activities selected, go to end |
|---|---|---|---|
| 17. | How much time did you spend in the last 24 hours organizing payments from health insurance? Include time spent writing emails, making telephone calls, completing paperwork etc. [Number of hours and minutes] | How much time did you or your child spend in the last 24 hours organizing payments from health insurance for their thalassemia treatment? Include time spent writing emails, making telephone calls, completing paperwork etc. Add together any time you spent on this task with any time your child spent on this task. [Number of hours and minutes] | Go to end |

*g/dL, grams per deciliter; Hb, hemoglobin*