# Understanding the Impact of Transfusion-dependent β-thalassaemia (TDT) in patients and caregivers

**Thank you for your interest in this health survey**

**[Question to identify the study subject]**

**Please, indicate which of the following profiles identifies you :**

☐ **I am an adult patient (18 years old and above) with a Transfusion-dependent β-thalassaemia (TDT) diagnosis** [SHOW ADULT PATIENT SURVEY]

☐ **I am a family member or an informal carer (i.e. not a healthcare professional) who regularly looks after a patient with a TDT diagnosis** [IF THIS IS SELECTED, PLEASE ASK]

- **How old is the patient that you are regularly taking care of?**

  ☐ **18 years old and above** [SHOW ADULT CAREGIVER SURVEY]

  ☐ **Between 2 and 15 years old** [SHOW BOTH PEADIATRIC & CAREGIVER SURVEY]

  ☐ **Between 16 and 17 years** [SHOW BOTH YOUNG-ADULT & CAREGIVER SURVEY]

# PEADIATRIC PATIENT SURVEY

Thank you for your interest in this health survey. You have been invited by the physician who treats your child/the child you are caring, or you are accessing through the SITE webpage, to participate in a study focusing on transfusion dependent Beta-thalassaemia (TDT) patients and their caregivers. In this survey, we will ask you questions about the experience of your child/the child you are caring with the disease and the impact on his/her life, as well as your experience with the disease and its impact on your daily life.

If you have any concern about participating to this study, please discuss it with your child´s/the child you are caring´s physician.

Please answer the following questions to confirm that your child/the child you are caring is eligible to participate in this survey.

## Screening Questions

S1: Has your child/the child you are caring been diagnosed by his/her physician with Beta-Thalassemia?

| | | |
|---|---|---|
| | Yes | CONTINUE |
| | No | TERMINATE AND THANK COLLABORATION |

S2: How many blood transfusions did she/he have during the past 12 months?

| | | |
|---|---|---|
| | 8 or more | CONTINUE |
| | Less than 8 | TERMINATE AND THANK COLLABORATION |

S3: Does she/he live in Italy?

| | | |
|---|---|---|
| | Yes | CONTINUE |
| | No | TERMINATE AND THANK COLLABORATION |

S4: Are you family member or a carer (but not a healthcare professional) looking after a patient with a physician confirmed diagnosis of TDT?

| | | |
|---|---|---|
| | Yes | CONTINUE |
| | No | TERMINATE AND THANK COLLABORATION |

S5: How long have you been taking care of your TDT patient?

| | | |
|---|---|---|
| | More than 6 months | CONTINUE |
| | Less than 6 months | TERMINATE AND THANK COLLABORATION |

S6: Are you able to read and understand Italian?

| | | |
|---|---|---|
| | Yes | CONTINUE |
| | No | TERMINATE AND THANK COLLABORATION |

S7: How have you been aware of this study? Please, select one answer

| | | |
|---|---|---|
| | The physician of my son/daughter/the patient I am caring asked if I was interested in participating to it | CONTINUE |
| | I was navigating into the SITE website and I found the link for this survey | CONTINUE |

Please, read the following information before consenting to participate in the study.

## Patient Information

**Study Purpose**

Your child/the child you are caring is one of about 100 people with a diagnosis of TDT, for which we are collecting data from, and you are one of about 100 caregivers of a patient with a diagnosis of TDT, who

are being asked to take a survey on behalf of your child/the child you are caring to help us understand the clinical and psychosocial burden of this disease on your child/the child you are caring in Italy. Also, in this survey we will ask you about yourself and the impact of TDT on your life.

**Study Method and Duration**

This is an online survey. We encourage you to complete the survey in one setting. It will take about 30 minutes to complete it.

**Possible Risks or Discomforts**

If any questions in the survey make you uncomfortable, you do not need to answer them.

The research study is being run by Certara on behalf of a pharmaceutical company. Certara is a research organization headquartered in the United States and with offices in several countries spanning four continents. Certara will protect your responses in accordance with the Privacy Policy available in Italy. The pharmaceutical company will receive your survey responses in aggregated manner and with no personal identifiers.

If you have concerns about your rights as participant on behalf of your child/the child you are caring you may discuss it with his/her physician.

**Benefits**

There are no direct health benefits to you or to your child/the child you are caring from participating in this study. Your responses are very important because they will help researchers to understand the clinical and psychosocial burden of the TDT.

**Confidentiality**

Many steps have been taken to protect your child´s information. Certara will only receive your answers, not your names. If the results of this study are presented at scientific meetings or published in scientific journals, no information will be included that could identify you and your child/the child or your responses personally.

An Ethic Committee (EC) has reviewed this research. The EC is a group of people who makes sure that the rights of participants in research are protected.

**Your Rights**

Your decision to take part in this research study is completely voluntary. Your decision to participate in this study will not affect the usual medical care of your child/the child you are caring. You can refuse to answer any question or stop at any point after you begin the survey.

**Compensation for your participation**

You will receive a voucher of 25€ for your time to participate in this study at the end of the survey. Please understand that you may refuse to answer any question and still receive the voucher.

**Future Contacts**

No future contact is envisioned.

**If you have read the previous screens and agree to participate by answering some questions about your child/the young adult you are caring, please click the ´Yes´ button, if not, click the ´No´ button.**

☐ Yes, I agree to participate [CONTINUE]

☐ No, I do not agree to participate. [RE-CONFIRM SELECTION]

**Are you sure you don't want to participate? Your opinions are important to us. Please select the ´Yes´ button to continue this survey; if not, select the ´No´ button to exit.**

☐ Yes, I agree to participate [CONTINUE]

☐ No, I do not agree to participate [TERMINATE AND THANK COLLABORATION]

## Questions about Your Child/the Child you are caring and His/Her Experience with TDT

**You have been invited to take part to this survey to provide data about yourself and on behalf of your child/the child you are caring** because she/he has been diagnosed with TDT and she/he receives regular blood transfusions.

Now, we would like to ask you some questions about him/her, the disease and his/her experience with it. There are no "right" or "wrong" answers.

### Please tell us about **YOUR CHILD/THE CHILD YOU ARE CARING**

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**1. What is his/her gender?**

☐ Male

☐ Female

☐ Other

☐ Prefer not to say

**2. What is his/her age ?**

☐ 2-3 years

☐ 4-5 years

☐ 6-7 years

☐ 8-9 years

☐ 10-11 years

☐ 12-13 years

☐ 14-15years

☐ Prefer not to say

**3. What is the highest level of education she/he has completed?**

☐ No formal education

☐ Nursery/Kindergarden

☐ Primary school education

☐ Secondary school education

☐ High school education

☐ None of the above

☐ Prefer not to say

**4. How old was she/he at the time of her/his thalassemia diagnosis?**

☐ 0-2 months

☐ 3-6 months

☐ 7-12 months

☐ 13-18 months

☐ 19-24 months

☐ 3-6 years old

☐ 7-10 years old

☐ 11+ years old

☐ Do not know or not sure

**5. How old was she/he at the time of her/his 1st blood transfusion?**

☐ 0-2 months

☐ 3-6 months

☐ 7-12 months

☐ 13-18 months

☐ 19-24 months

☐ 3-6 years old

☐ 7-10 years old

☐ 11+ years old

☐ Do not know or not sure

**6. In the last 12 months, how many blood transfusion did she/he receive?**

☐ Between 8 and 12

☐ More than 12

☐ Do not know or not sure

7. **In the last 12 months, how many weeks did she/he wait between two consecutives blood transfusions?**

☐ 2 weeks or less

☐ 3 – 5 weeks

☐ More than 5 weeks

☐ Do not know or not sure

8. **On average, how long does it take her/his blood transfusion (only blood transfusion time)?**

☐ Less than 1 hour

☐ 1 – 2 hours

☐ 2 – 4 hours

☐ More than 4 hours

☐ Do not know or not sure

9. **In the last 12 months, did your child/the child you are caring ever experience a time in which the blood was not available on his/her transfusion day?**

☐ Yes

☐ No

☐ Do not know or not sure

**9.1.** ASK ONLY IF Q9=1 (YES) in the last 12 months, **How many times did she/he experience a blood shortage?**

☐ 1

☐ 2 to 3

☐ 4 or more

☐ Do not know or not sure

**9.2.** ASK ONLY IF Q9=1 (YES) **In which time of the year the blood shortage occurred? (Please select all that apply)** MULTI CODE POSSIBLE

☐ Spring

☐ Summer

☐ Autumn

☐ Winter

☐ Do not know or not sure

10. **Where is the region of the hospital where your child/the young adult you are caring is attended for receiving blood transfusion?**

[Drop-down menu: Abruzzo;Basilicata;Calabria;Campania;Sardegna;Emilia Romagna;Friuli Venezia; Giulia;Lazio;Liguria;Lombardia;Marche;Molise;PiEmonte;Puglia;Sicilia;Toscana;Trentino Alto Adige; Umbria;Valle d'Aosta;Veneto]

☐ Northern Italy

☐ Central Italy

☐ Southern Italy and islands

**11. A part from your blood transfusion, how often, on average, does she/he go to the hospital for her/his visit related to your disease?**

☐ Never

☐ Less than one (1) in a month

☐ 1-2 times per month

☐ Three (3) times in a month

☐ More than three (3) times in a month

☐ Do not know or not remember

**12. Usually, how does she/he reach the hospital for your visit or blood transfusion?**

☐ By car

☐ By taxi

☐ By train or bus

☐ Other

☐ Prefer not to say

**13. On average, how long does it take travelling from her/his home to the blood transfusion centre?**

☐ Less than 30 minutes

☐ 30 minutes to 1 hour

☐ 1 to 2 hours

☐ 2 hours or more

☐ Do not know or not sure

**14. In the last 12 months, did she/he visit any specialist other than her/his hematologist for a health issue related to TDT?**

☐ Yes

☐ No

☐ Do not know or not sure

**14.1.** ASK ONLY IF Q14=1 (YES) **Please specify (please select all that apply)** MULTI CODE POSSIBLE

☐ Cardiologist

☐ Rheumatologist

☐ Endocrinologist

☐ Other (specify)

☐ Do not know or not remember

**15. In the last 12 months, did she/he undergo any medical procedure (such as imaging exam, biopsy) for the TDT?**

☐ Yes

☐ No

☐ Do not know or not remember

**15.1.** ASK ONLY IF Q15=1 (YES) **Please specify the type of procedure (please select all that apply)** MULTI CODE POSSIBLE

☐ Laboratory exam (e.g. blood count, ferritin, hemoglobin test)

☐ Biopsy

☐ Imaging exam (e.g. MRI),

☐ Other, please specify: _____

☐ Do not know or not remember

**16. In your opinion, do you think that you child/the child you are caring has difficulty remembering or concentrating?**

☐ No, no difficulty

☐ Yes, some difficulty

☐ Yes, a lot of difficulty

☐ Yes, Cannot do at all

**16.1.** ASK ONLY IF Q16=1 (YES) **How often does she/he have difficulty remembering or concentrating well?**

☐ Somewhat often

☐ Very often

☐ No Answer/Don't Know

**16.2.** ASK ONLY IF Q16=1 (YES) **Do you believe that her/his memory or concentration difficulties are …. (please select all that apply)** MULTI CODE POSSIBLE

☐ because she/he have too many things to do?
☐ because of something else?
☐ No Answer/Don't Know

**16.3.** ASK ONLY IF Q16=1 (YES) **Are there any activities that she/he cannot do because of a problem with her/his memory or concentration?**

☐ Yes. Please specify _____
☐ No
☐ No Answer/Don't Know

**16.4.** ASK ONLY IF Q16=1 (YES) **How concerned or worried is she/he about her/his ability to remember or concentrate?**

☐ Not at all
☐ Somewhat concerned
☐ Very concerned
☐ No Answer/Don't Know

**17. Because of a physical, mental of emotional health condition, does she/he have difficulty communicating (for example understanding others or others understanding you)?**

☐ No, no difficulty
☐ Yes, some difficulty
☐ Yes, a lot of difficulty
☐ Yes, Cannot do at all

**18. Does she/he have difficulty learning a new task, for example, learning how to get to a new place?**

☐ No, no difficulty
☐ Yes, some difficulty
☐ Yes, a lot of difficulty
☐ Yes, Cannot do at all

Please, tell us about **HER/HIS Experience** with TDT and Medicines used

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**19. Is she/he currently taking a medicine for iron overload *(i.e.too much iron in the body)?***

☐ Yes

☐ No

☐ Do not know or not sure

**19.1.** ASK ONLY IF Q19 =1 (YES) **How is she/he currently taking the medicine for iron overload?**

☐ Pill or tablet once per day [IF SELECTED SHOW Q19.2 and Q19.3]

☐ Pill or tablet more than once per day [IF SELECTED SHOW Q19.2 and Q19.3]

☐ Injection [IF SELECTED SHOW Q19.4]

☐ Both pill and injection [IF SELECTED SHOW Q19.2 and Q19.3 and Q19.4]

☐ Do not know or not sure

**19.2. Please choose the face that best describes your child´s/the child you are caring reaction to the taste of the medicine for iron overload immediately after putting it in his/her mouth today:**


**19.3. Please choose the face that best describes your child´s/the child you are caring reaction to the aftertaste (taste left in the mouth after swallowing) of the medicine after swallowing his/her medicine for iron overload today**


**19.4. Please choose the face that best describes your child´s/the child you are caring reaction after the injection**

**20. Do you have difficulties in remembering to give the medicines for iron overload to your child/the child you are caring?**

☐ Never

☐ Once in a while

☐ Often

☐ All the time

☐ Do not know or not sure

Please, tell us about the **Impact of TDT in YOUR child´s/the child YOU ARE CARING** Life

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1. a. **Does or did thalassaemia affect your child's/the child school progress? Position in tests/exams**:

| | |
|---|---|
| Low | [ ] |
| Middle | [ ] |
| Top | [ ] |

b. **Would he/she have done better if he/she did not have thalassaemia?**

No [ ] Yes [ ]

Why? .....

2. **On average how much time off school/work has your child/the child lost?**

| | |
|---|---|
| None | [ ] |
| One day or less per month | [ ] |
| One week or less per month | [ ] |
| More than one week per month | [ ] |

3. **a. Does thalassaemia affect your child's/the child's sport activities?**

| | |
|---|---|
| Not at all | [ ] |
| A little | [ ] |
| A lot | [ ] |

**b. Would he/she have done better if he/she did not have thalassaemia?**

Yes [ ] No [ ]

Why? .....

4. **Do you think your child/the child is different from friends/sibs ?**

Yes [ ] No [ ]

Why?.....

5. **Does your child/the child engage in school/ outdoor activities at the same level as his/her peers?**

Yes [ ] No [ ]

Why?.....

6. **Does thalassaemia affect your child's/the child's relationship with his/her friends ?**

Not at all [ ]

A little [ ]

Sometimes [ ]

A lot [ ]

Why?.....

7. **Does thalassaemia affect your child's/the child's relationship with sisters/brothers?**

No [ ]

Yes [ ]

Why? .....

8. **Do your other children express resentment about special treatment of the patient?**

Yes [ ]

No [ ]

Explain .....

**9. Do you think thalassaemia affects your child's/the child's relationship with yourself?**

No [ ]

Yes [ ]

Why? .....

10. **Is your child/the child aware of his/her illness?**

Yes [ ]

No [ ]

Don't know [ ]

11. **Is your child/the child upset by anxious, worried feelings about his/her illness?**

Not at all [ ]

A little [ ]

Sometimes [ ]

Often [ ]

12. **Does your child/the child talk about his/her problems or worries?**

Not at all [ ]

Sometimes [ ]

Often [ ]

13. **Who does your child/the hcild trust or turn to for help?**

Parents [ ]

Brothers/sisters [ ]

Relatives [ ]

Friends [ ]

Other .....

14. **Do or did your/the child's friends/schoolmates know he/she has thalassaemia?**

Yes [ ] No [ ]

Why? .....

15. **Do or did your/the child's employer/teacher know he/she has thalassaemia?**

Yes [ ] No [ ]

Why? .....

16. **Does or did your child/the child go on school trips?**

Yes [ ]

No [ ]

Why? (Is it parents or school?)

17. **Does your child/the child know other patients with thalassaemia?**

Yes [ ]

No [ ]

Please, tell us about **Your Experience in** caring a TDT patient

Now, we would like to ask you questions about your experience as caregiver, in managing your son/daughter/patient that suffer from TDT

Please tell us about **Yourself**

_____

**1. What is your gender?**

□ Male

□ Female

□ Other

□ Prefer not to say

**2. What is your age?**

□ 18-20

□ 21-25

□ 26-30

□ 31-35

□ 36-40

□ 41-50

□ 51-55

□ 56-60

□ 61-65

□ 65+

□ Prefer not to say

**3. What is the highest level of education you have completed?**

□ No formal education

□ Primary school education

□ Secondary school education

□ High school education

□ University

□ Postgraduate university degree

□ None of the above

□ Prefer not to say

**4. What is your marital status?**

□ Single/never married

□ Married/living as married/civil partnership

□ Divorced or separated

□ Widowed/surviving partner

□ Other

□ Prefer not to say

**5. Which of the following best describe your relationship to the patient?**

☐ I am the mother

☐ I am the father

☐ I am the Spouse/ Partner

☐ I am the sister/brother

☐ I am a relative

## Please, tell us about Your Experience caring a TDT patient

### CarerQol-7D

We would like to form an impression of your caregiving situation.
Please tick a box to indicate which description best fits your caregiving situation at the moment.

*Please tick only one box per description: 'no', 'some' or 'a lot of'.*

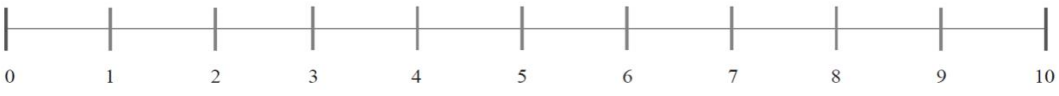
| | no | some | a lot of | |
|---|---|---|---|---|
| a. I have | | | | fulfilment from carrying out my care tasks. |
| b. I have | | | | relational problems with the care receiver (*e.g., he/she is very demanding or he/she behaves differently; we have communication problems*). |
| c. I have | | | | problems with my own mental health (*e.g., stress, fear, gloominess, depression, concern about the future*). |
| d. I have | | | | problems combining my care tasks with my daily activities (*e.g., household activities, work, study, family and leisure activities*). |
| e. I have | | | | financial problems because of my care tasks. |
| f. I have | | | | support with carrying out my care tasks, when I need it (*e.g., from family, friends, neighbours, acquaintances*). |
| g. I have | | | | problems with my own physical health (*e.g., more often sick, tiredness, physical stress*). |

### CarerQol-VAS

How happy do you feel at the moment?

*Please place a mark on the scale below that indicates how happy you feel at the moment.*

completely unhappy — completely happy



Please, tell us about **the Impact of TDT in YOUR Life**

1. **Does or did thalassaemia affect your social life?**

   Not at all [ ]

   A little [ ]

   A lot [ ]

   Why? .....

2. **Has thalassaemia affected your relationship with your friends/relations?**

   Not at all [ ]

   A little [ ]

   A lot [ ]

   Why? .....

3. **Do you worry about thalassaemia?**

   Not at all [ ]

   A little [ ]

   Sometimes [ ]

   A lot [ ]

   All the time [ ]

   Why? .....

4. **What is your biggest worry concerning your child or person you are caring?**

   **[ FREE TEXT]**

5. **How often do you talk about your problems or worries?**

   Not at all [ ]

   Sometimes [ ]

   Often [ ]

6. **Who do you trust or turn to for help?** *(please select all that apply)* **MULTI CODE POSSIBLE**

Spouse [ ]

Relatives [ ]

Friends [ ]

Doctor [ ]

Religious Guide [ ]

Support Group [ ]

Other .....

7. **Have you joined a Support Group: Thalassaemia Centre, Patient organizations, SITE foundation?**

Yes [ ] No [ ]

Why? .....

How often do you attend their meetings? .....

8. **Do you think you need more support?**

Yes [ ], If yes from where and why? ……**[ FREE TEXT]**

No [ ]

9. **Has thalassaemia affected your relationship with your spouse/partner?**

Not at all [ ]

A little [ ]

A lot [ ]

How? .....

10. **Has thalassaemia affected your relationship with your children (if appliacable)?**

Not at all [ ]

A little [ ]

A lot [ ]

How? .....

11. **Do or did your child's or person you are caring´s friends/schoolmates know he/she has thalassaemia?**

Yes [ ] No [ ]

Why ?.....

12. **Do or did your child's or person you are caring´s teacher/employer know he/she has thalassaemia?**

Yes [ ] No [ ]

Why ?.....

13. **Do you think your child or person you are caring is different from friends/sibs ?**

Yes [ ] No [ ]

Why ?.....

14. **Who told you the diagnosis and how?**

Doctor [ ]

Nurse [ ]

No one [ ]

Other [ ]

How? .....

15. **Did you understand the diagnosis at the time?**

Yes [ ] No [ ]

Explain .....

16. **Do you understand the nature of thalassaemia?**

Yes [ ] No [ ]

Explain.....

17. **Did you blame yourself for your child's or person you are caring´s condition?**

Yes [ ]

→If Yes, Do you still do? Yes [ ] No [ ]

No [ }

Why?

18. **How did you feel when you were told the diagnosis?** ***(please select all that apply)*** **MULTI CODE POSSIBLE**

Unhappy [ ]

Depressed [ ]

Anxious [ ]

Anger [ ]

Guilt [ ]

Relief [ ]

Did not believe [ ]

Don't know [ ]

Other .....

19. **How do you feel about thalassaemia now?** ***(please select all that apply)*** **MULTI CODE POSSIBLE**

Anger [ ]

Guilt [ ]

Anxious [ ]

Unhappy [ ]

Depressed [ ]

Coping [ ]

Don't know [ ]

Other .....

**20. What does it mean to you that your child or person you are caring has thalassaemia?**

Does not affect me [ ]

Constantly worried [ ]

Feeling helpless [ ]

Feeling guilty [ ]

Learned how to cope [ ]

Other.....

21. **Has the employment of anyone in the family, or the family's financial situation been affected by the illness**?

Yes [ ] No [ ]

Explain.....

**22. [ASK ONLY IF SECTION 1 - Q5 = I AM THE MOTHER] How many pregnancies have you had after your affected child and what were their outcome?**

Details: .....

**23. [ASK ONLY IF SECTION 1 - Q5 = I AM THE MOTHER] Did you have prenatal diagnosis with pregnancies after 1979?**

Yes [ ] No [ ]

Why? .....

**24. [ASK ONLY IF SECTION 1 - Q5 = I AM THE MOTHER] If you were going to get pregnant since 1979 would you consider a test in early pregnancy to see if the baby was affected with thalassaemia?**

Yes [ ] No [ ]

Why? .....

**25. [ASK ONLY IF SECTION 1 - Q5 = I AM THE MOTHER] If you were pregnant and had a test which showed that the baby has thalassaemia would you consider termination?**

Yes [ ]

No [ ]

Don't know [ ]

Why? .....

**26. [ASK ONLY IF SECTION 1 - Q5 = I AM THE MOTHER] Has your child's condition affected your plans to have more children or your attitude to testing for thalassaemia during pregnancy?**

Yes [ ] No [ ]

Explain.....

**27. What advice would you give to parents who had just found that they could have a child with thalassaemia?**

**28. What advice would you give to a young couple who planned to marry but their blood test showed that they could have a child with thalassaemia?**