# Economic burden of transfusion-dependent Chinese adult ß-thalassemia patients from a social perspective


**Yu Jia,[1] Xuemei Zhen,[2] Zijing Wang[3]**

[1]Bristol-Myers Squibb (China) Investment Co., Ltd., Shanghai; [2]Centre for Health Management and Policy Research, School of Public Health, Cheeloo College of Medicine, Shandong University, Jinan; [3]Beijing New Sunshine Charity Foundation, Beijing; China


## Introduction

- ß-thalassemia is a genetic blood disorder marked by ineffective erythropoiesis and anemia[1]
- Patients with ß-thalassemia requiring regular red blood cell transfusions (RBCTs) are considered transfusion-dependent (TD) and often need iron chelation therapy (ICT)[2]
- RBCTs are associated with serious complications and morbidity, mainly related to iron overload, which leads to diabetes, hepatic disease, and cardiac death[1]
- The lifelong management of ß-thalassemia and its related complications impose a severe burden on patients and society
- The estimated prevalence of ß-thalassemia in China has not been accurately quantified since the 1980s, and was 0.67% at the time[3,4]
- The need for RBCTs and ICT increases with age and weight, as does the corresponding economic burden of managing patients with ß-thalassemia

## Objective

- To investigate the economic burden of managing patients with TD ß-thalassemia (TDT) in China

## Methods

- This literature review and patient survey evaluated the economic burden of TDT from a social perspective

### Literature search

- A literature search of Google Scholar identified clinical data (incidence of disease and complications, blood transfusion volume, and other key metrics), healthcare practices, and related costs
- The literature search focused on Chinese patients primarily; where data on disease complications were limited, the search was widened to Asia and the rest of the world
- The following search terms were used: ß-thalassemia, transfusion-dependent, transfusion, iron chelation, heart failure, arrhythmia, examination, complication rate, and cost

### Patient survey

- To validate data identified in the literature search, a questionnaire was administered to adults with TDT (≥ 18 years of age) regarding weight and non-medical costs
- Participants were recruited by physicians, with snowball sampling used to recruit additional survey participants

### Costs

- Annual expected costs of TDT complications were calculated by multiplying the incidence rate by cost data from the literature, using the formula $\Sigma P_i * cost_i$
  - Cost data from the literature were adjusted to 2021 costs in Chinese yen (CNY) based on the Consumer Price Index
  - US dollar (USD) costs were calculated using an exchange rate of 6.32
- Findings were analyzed using descriptive statistics

## Results

- A total of 136 studies were identified in the literature search, of which 15 met the eligibility criteria and were included in this analysis
- 81 patients with TDT responded to the survey, of whom 70 provided weight data (median, 50 kg; mean, 51.32 kg [standard deviation, 7.67 kg]) used to calculate the cost of RBCTs and ICT
- Annual treatment costs for a patient with TDT in China were CNY 83,464 (USD 13,206) (**Table 1**)
  - Per-patient transfusion costs were CNY 8593 (USD 1360) using 39 red blood cell (RBC) units
  - Per-patient ICT costs were CNY 71,282 (USD 11,279)
  - Other per-patient hospital costs were CNY 3589 (USD 568)
- The expected mean annual cost per patient-year for complications was CNY 104,179 (USD 16,484) (**Table 2**)
- The mean annual non-medical direct cost per patient was CNY 8181 (USD 1294), which included transportation, accommodation, catering expenses, and nursing fees (**Figure 1**)
- The mean annual indirect cost of disease-related time lost from work for the patient and their family was CNY 31,660 (USD 5009) (**Table 3**)
- The mean annual intangible cost due to disease-related psychological impact was CNY 392,939 (USD 62,174)
- The total of all direct and indirect costs was CNY 620,423 (USD 98,167) (**Table 4**)

**Table 1. Annual direct costs per patient**

| Items | Mean annual cost per patient, CNY | Calculation[a] | Reference/ source |
|---|---|---|---|
| RBCT | 8593 | RBC unit price (220) × average unit per time (2.79) × average times per patient-year (14) | 5,6 |
| ICT | 71,282 | Average cost per patient month (5940.21) × 12 months | 6 |
| Examination before RBCT or ICT | 2800 | Average cost per time (200) × average times per patient-year (14) | 6 |
| Hospitalization | 789 | Average times per 12 weeks (2.6) × (52/12) × hospitalization cost per day (70) | Assumption from clinician |
| Subtotal | **83,464** | | |

[a]Calculations were based on the median patient-reported weight of 50 kg.

**Table 2. Expected costs for complications per patient-year**

| Complication | Incidence, % | Mean annual treatment cost,[a] CNY | Expected cost (= incidence × annual treatment cost),[b] CNY | Reference |
|---|---|---|---|---|
| **Caused by iron overload** | | | | |
| Heart failure | 25.18 | 73,575.13 | 18,528 | 7-9 |
| Arrhythmia | 30.28 | 95,006.43 | 28,771 | 7,10 |
| Pulmonary hypertension | 44.53 | 4150.01 | 1848 | 7,11 |
| Diseases of the endocrine system (diabetes mellitus) | 33.19 | 9195.06 | 3052 | 7,12 |
| Liver disease | 38.96 | 69,164.86 | 26,947 | 7,13 |
| Gonadal dysfunction | 3.14 | 19,515.85 | 613 | 7,14 |
| Hypothyroidism | 5.63 | 9938.06 | 560 | 7,13 |
| Hypoparathyroidism | 2.00 | 2359.67 | 47 | 13,15 |
| **Caused by anemia** | | | | |
| Osteoporosis | 54.10 | 20,493.79 | 11,087 | 16,17 |
| **Caused by blood transfusion** | | | | |
| Cerebrovascular disease (cerebral infarction or hemorrhage) | 60.7 | 20,068.36 | 12,181 | 18,19 |
| Blood-borne diseases (eg, HCV) | 3.89 | 14,000.51 | 545 | 20,21 |
| **Subtotal** | | | **104,179** | |

[a]Cost data from the literature were adjusted to 2021 costs based on the Consumer Price Index. [b]Values may differ due to rounding. HCV, hepatitis C virus.

**Figure 1. Annual non-medical direct costs per patient (from patient survey)**

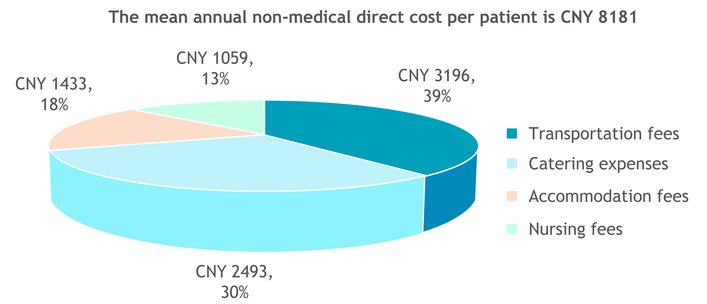


**Table 3. Annual indirect costs**

| Items | Mean annual cost, CNY | Reference |
|---|---|---|
| Disease-related loss of working time | 31,660 | 22 |
| Intangible cost due to poor quality of life (such as psychological impact and social isolation, etc) | 392,939 | 22 |
| Subtotal | **424,599** | |

**Table 4. Total costs of managing TDT**

| Cost component | Annual cost,[a] CNY | Annual cost,[b] USD |
|---|---|---|
| Direct treatment costs | 83,464 | 13,206 |
| Expected cost of complications | 104,179 | 16,484 |
| Non-medical direct costs | 8181 | 1294 |
| Indirect costs | 424,599 | 67,183 |
| Total costs | **620,423** | 98,167 |

[a]Cost data from the literature were adjusted to 2021 costs based on the Consumer Price Index; [b]The exchange rate of the USD to the CNY was 6.32.

## Discussion

- The following limitations apply to this study:
  - Few studies were available reporting the costs of TDT among adults in China
  - Based on the data sources and sample size, which were limited and heterogeneous, these findings may be subject to uncertainty
  - "Expected cost" was used due to a lack of real-world costs available in the literature
- These findings provide a preliminary estimate of the economic burden of managing TDT patients in China; however, further research is needed to better quantify these costs to patients and society

## Conclusions

- The expected costs of complications of TDT were substantial, as were non-medical direct costs
  - The indirect and intangible cost burden to patients was higher due to time lost from work and impact on mental health
- These data suggest that both direct and indirect costs of TDT should be considered in healthcare policy decisions impacting patients and society in China

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Email: Yu.Jia@bms.com