May 10, 2022

Institute for Clinical and Economic Review (ICER)
2 Liberty Square
Boston, MA 02109

Dear ICER Review Panel,

bluebird bio appreciates the opportunity to respond to the ICER Draft Evidence Report for betibeglogene autotemcel (beti-cel) for transfusion-dependent β-thalassemia (TDT). We thank the ICER team for their commitment to open dialogue during the process steps to date, and for their openness to feedback on elements of the model and inputs raised by our team. We look forward to advancing these ideas at the upcoming Public Meeting in June.

Beta-thalassemia patients who require regular blood transfusions are at risk for serious complications and an early death, despite recent improvements in chronic therapies. bluebird bio hears frequently from patients and clinicians about the urgent need for new treatment options that could improve health outcomes and provide freedom from lifelong transfusions. We appreciate ICER's recognition of the significant impact TDT has on patients' lives and that eliminating the need for chronic transfusions would allow for reduction in iron overload-related complications and a better likelihood of achieving major life goals. We are also pleased that ICER views beti-cel as having the potential for a substantial net health benefit. bluebird bio is aligned with the core elements of the ICER TDT economic model and agree it captures the key components driving clinical outcomes and health system costs for individuals living with TDT. We appreciate that ICER included a model validation step based on the previously published cost-effectiveness analysis for beti-cel.[i]

Below we outline our comments to this draft, including model input values that we encourage ICER to reconsider to ensure the final report is fully aligned with existing clinical and scientific evidence. In summary:

1. **ICER should update its base-case value for beti-cel treatment success (i.e., TI: transfusion independence) to 90.2%, based on the latest available data and predictive modeling.**

2. **The base-case value for beti-cel durability loss should be 0%, with a separate but scientifically plausible scenario considering potential, non-zero durability loss. This assumption is aligned with the known clinical evidence base and leading scientific expertise, which should serve as the primary sources upon which to extrapolate the durability of beti-cel treatment effect.**

3. **ICER's model incorrectly assumes a correlation between patient non-adherence and chelation costs, in the absence of data to support this assumption. TDT patient representatives have clarified that patients will continue to maintain their normal prescription refill patterns for chelators despite episodes of nonadherence. This aspect**

of the model should be removed.

4. **bluebird bio would like to clarify that any potential outcomes-based payment plan for beti-cel that is modeled by ICER should include a payback option of up to 80% if treatment with beti-cel is unsuccessful.**

Below we provide further clarification for each of our recommendations. In addition, we have included an Appendix that provides suggested changes to the wording in the clinical section of the Draft Report.

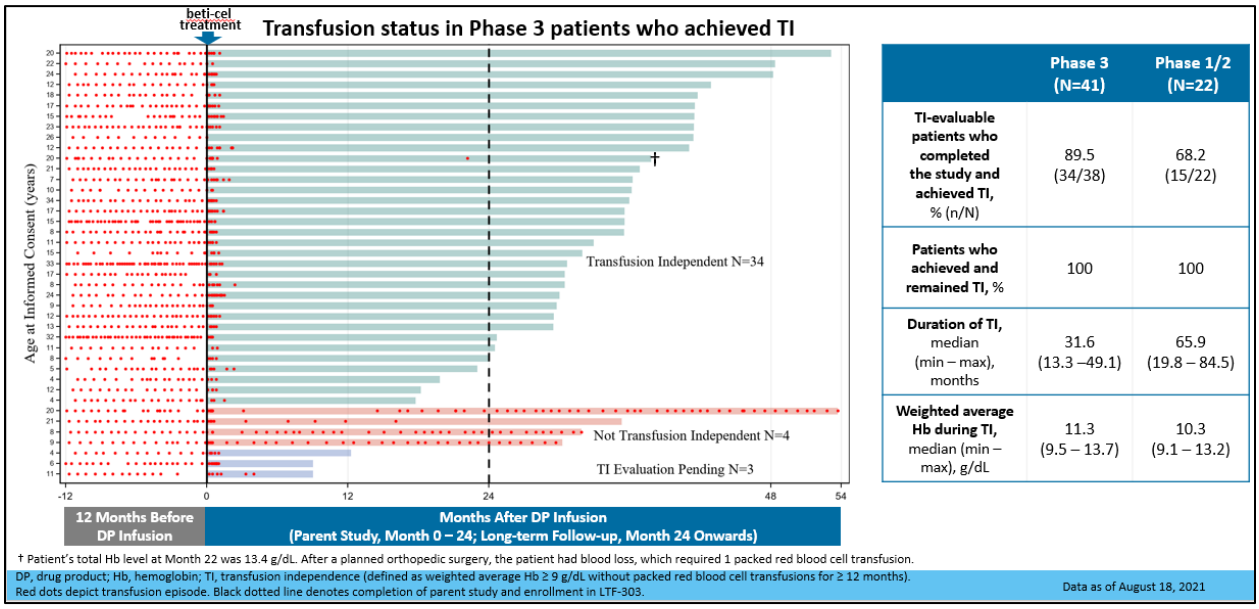
1. **ICER should update its base-case value for beti-cel treatment success (i.e. achieving TI: transfusion independence) to 90.2%, based on the latest available data and predictive modeling.**

   Based on data recently presented at the Transplantation & Cellular Therapy Meetings of ASTCT and CIBMTR or "TANDEM Meetings" in April 2022, as of the August 2021 data cut, 38 patients treated with beti-cel in our Phase 3 studies are now TI evaluable (see definition below), 34 of whom have achieved TI. This success rate is 34/38 or 89.5%. All patients who have achieved TI have maintained their TI status (see Figure 1).

   In addition, internal analyses have shown that HbA^{T87Q} values were stable and durable by month 6, and predictive of achieving TI success for ALL 34 patients who have achieved TI status to date. For the remaining 3 non-evaluable patients who have not yet had sufficient follow-up time to be evaluable for the TI end point, the predictive probability of achieving TI for ALL 3 (based on HbA^{T87Q} at month 6) is at least 99%, which would lead to a final phase 3 study TI success rate of 37/41 or 90.2%.

   - TI is defined as a weighted average Hb ≥9 g/dL without any pRBC transfusions for a continuous period of ≥12 months at any time during the study after drug product infusion.
   - Please note as per the study statistical analysis plan, the pre-specified definition for TI-evaluable is defined as patients who have completed their parent study (i.e., 24 months of follow-up), or achieved TI, or will not achieve TI in their parent study. A patient is deemed 'will not reach TI in the parent study' if he/she is receiving chronic transfusions after 324 days of follow-up (<14 months of follow-up left in parent study), or if his/her Hb level never reached t0 (Hb ≥9 g/dL with no transfusions in the preceding 60 days) by 385 days of follow-up.

Figure 1. Maintenance of Transfusion Independence for up to 4 Years of Follow-up in Phase 3 beti-cel studies[ii]



	Phase 3 (N=41)	Phase 1/2 (N=22)
TI-evaluable patients who completed the study and achieved TI, % (n/N)	89.5 (34/38)	68.2 (15/22)
Patients who achieved and remained TI, %	100	100
Duration of TI, median (min – max), months	31.6 (13.3 –49.1)	65.9 (19.8 – 84.5)
Weighted average Hb during TI, median (min – max), g/dL	11.3 (9.5 – 13.7)	10.3 (9.1 – 13.2)

2. The base-case value for beti-cel durability loss should be 0%, with a separate but scientifically plausible scenario considering potential, non-zero durability loss. This assumption is aligned with the known clinical evidence base and leading scientific expertise, which should serve as the primary sources upon which to extrapolate the durability of beti-cel treatment.

bluebird bio appreciates that for the Draft Report ICER lowered the "loss of beti-cel durability" assumption included in its Modeling Analysis Plan to a value of 0.271% starting at Year 7. However, the known evidence base to date (up to ~ Year 7) has indicated no loss of durability or expectation of loss of durability by investigators; and leading scientific experts (who have separately submitted their responses to ICER) expect lifetime durability. We strongly encourage ICER to use a base-case value that reflects both the available evidence and leading scientific expertise rather than a more theoretical assumption. We recognize that scenarios on lapse of durability will be evaluated by ICER but believe such scenarios should be secondary to the base-case.

Additional points

- Independent experts agree that following successful engraftment and achievement of TI, the effects of beti-cel are expected to be life-long.
  - See the European Medicines Agency (EMA) summary of product characteristics for beti-cel (referred to as Zynteglo)[iii]

- Tucci et al. (2021)[iv] indicate that the lentiviral vector integrates into hematopoietic stem cell genome and provides long-lasting functional copy of the defective gene

- The currently available evidence on the durability of beti-cel are more robust than evidence from prior ICER reviews of gene therapies, which assumed lifetime benefit. For example, in one of the gene therapy reviews, ICER's base-case scenario allowed for lifetime durability of the gene therapy when:
  - the longest follow-up was just 2 years (versus up to 7 years for beti-cel as of August 2021 data cut)
  - sample size was notably smaller (n=12 in one of the gene therapy trials which was basis for ICER base-case) than that of the current beti-cel data package (discussed above)
  - ICER relied upon that gene therapy's primary efficacy measure for its durability assumption, and it was considered a surrogate endpoint for future developmental milestones. In contrast, beti-cel's primary efficacy measure of TI is considered a direct clinical endpoint.

3. **ICER's model incorrectly assumes a correlation between patient non-adherence and chelation costs, in the absence of data to support this assumption. TDT patient representatives have clarified that patients will continue to maintain their normal prescription refill patterns for chelators despite episode of nonadherence. This aspect of the model should be removed.**

   bluebird bio recognizes that the current standard of care for individuals with TDT includes multiple iron chelation therapy options, each with their own advantages and disadvantages. We also recognize there is a known problem with individuals with TDT being able to maintain good adherence with their iron chelation therapy. However, we are not aware of any published evidence that indicates a direct correlation between poor adherence with iron chelators and prescription refill patterns for those iron chelation therapies. In our discussions with individuals who live with TDT, iron chelation therapy has been a long-standing chronic routine, and prescription refills are automatic and ongoing. In addition, a recently published cost of care study[v] for β-thalassemia found iron chelation therapies were a major cost driver, and the annual total average costs for chelation therapy were indicative of ongoing refills for TDT patients.

4. **bluebird bio would like to clarify that any potential outcomes-based payment plan for beti-cel that is modeled by ICER should include a payback option of up to 80% if treatment with beti-cel is unsuccessful**

   In its Scenario Analysis 4 in the Draft Report, ICER found that the incremental-cost effectiveness ratio would increase to $133,900 with a scenario where the full cost of beti-cel was paid at time of transplant (i.e. Year 1 in model). However, ICER does not include the modeled outcomes agreement (i.e. payback up to 80% if treatment fails) in this scenario.

bluebird bio plans to offer an outcomes agreement for beti-cel treatment failure that is separate and distinct from the specific payment plan (e.g. pay all upfront vs. pay over time) that may be contracted with health insurers. We want to clarify that any model scenarios run by ICER should include an assumption that bluebird bio will pay back up to 80% if a patient treated with beti-cel does not achieve TI.

In addition, we have provided suggestions/corrections for wording changes, citations and clinical data (as part of the Appendix) in the clinical section of the Draft Report. To summarize:

- Patients with beta-thalassemia receive transfusions and iron chelation as a part of standard of care; Phlebotomy refers to withdrawal of blood. We suggest you change 'phlebotomy' to 'iron chelation' on Executive Summary page ES1 and Patient and Caregiver perspectives page 3.
- We have added several reference-related comments so the statements are cited to appropriate references.
- Please note, study HGB-212 study enrolled β0/β0 and non-β0/β0 pts. Non-β0/β0 pts (included IVS-I-110 homozygous and IVS-I-110/β0 genotypes) enrolled had severe genotypes similar to β0/β0 pts.
- We have added comments to edit data included in Table 3.1, 3.2, D6 & D7.

[i] Kansal AR, Reifsnider OS, Brand SB, et al. Economic evaluation of betibeglogene autotemcel (beti-cel) gene addition therapy in transfusion-dependent β-thalassemia. J Mark Access Health Policy 2021;9(1):1-13.
[ii] Schneiderman et al. Efficacy and safety of betibeglogene autotemcel (beti-cel) gene therapy in 63 patients with TDT: 7- year post infusion follow up of Phase 1/2 and Phase 3 studies. Oral Presentation at TCT Tandem Meeting 2022, Salt Lake City, Utah. Data cut as of August 2021.
[iii] See URL: https://www.ema.europa.eu/en/medicines/human/EPAR/zynteglo#authorisation-details-section
[iv] Tucci F, Scaramuzza S, Aiuti A, Mortellaro A. Update on clinical ex vivo hematopoetic stem cell gene therapy for inherited monogenic diseases. *Mol Therapy* 2021;29(2):489-504.
[v] Weiss M, Jun MP, Sheth S. Clinical and economic burden of regularly transfused adult patients with β-thalassemia in the United States: a retrospective cohort study using payer claims. *Am J Hematol* 2019;94(5):E129-E132.

Comments on Draft Evidence Report
Betibeglogene Autotemcel for Beta Thalassemia: Effectiveness and Value

From Craig Butler, National Executive Director, Cooley's Anemia Foundation:

Thank you for the thorough and detailed draft report. The Foundation is pleased that the report emphasizes the potential lifetime benefit of the procedure under discussion, as well as acknowledges more intangible variables of importance (such as social impact, etc.)

The Foundation wishes to re-emphasize that, while individuals with thalassemia are living much longer lives if they are able to receive optimal treatment, life expectancy still continues to lag behind the average. Over the 10-year period 2011-2021, of patients included in the Cooley's Anemia Foundation patient database, for those patients who passed away for which age was known, the average age of death was 40.12 years; for those who were classified as transfusion-dependent-thalassemia, the average age was 33.99 years (median age of 37 years).

An effective and safe curative approach is an option which would be of great interest to many members of the thalassemia community.

Thank you for your interest in this area.

PIPC Partnership to Improve Patient Care

May 10, 2022

Dr. Steven D. Pearson
President
Institute for Clinical and Economic Review
Two Liberty Square, Ninth Floor
Boston, MA 02109

Dear Dr. Pearson,

The Partnership to Improve Patient Care (PIPC) appreciates this opportunity to comment on the Institute for Clinical and Economic Review's (ICER) draft evidence report on treatments for beta thalessemia. PIPC remains concerned about many of ICER's modeling choices and their impact across its studies and would encourage ICER to consider the following.

ICER continues to use the discriminatory Quality-Adjusted Life Year (QALY).

As PIPC has commented previously, the use of discriminatory metrics, including the QALY, is inappropriate and too often results in a discriminatory impact. It is acknowledged that QALYs discriminate by design against people with disabilities and chronic illnesses.[1] There should be no room for discrimination in health care decision making, and we urge ICER to cease use of discriminatory metrics.

ICER's Model Takes a Narrow Perspective

The narrow perspective of ICER's model ignores the wider benefit of reduced demand on scarce health care resources, which overlooks a valuable facet of successful treatment of transfusion dependent beta thalassemia (TDBT).

TDBT patients account for a larger percentage of blood transfusions around the world. TDBT patients make up almost 18% of all blood transfusions in Greece,[2] and almost 10% in Hong Kong.[3] This is expected to rise to 30% by 2024. In the United Kingdom it is expected that the demand for blood products for TDBT patients is likely to rise by 20% in the next five years.[4] The European Committee on Blood Transfusion reported that most of the 45 countries evaluated had a nationally coordinated blood program (85%), but 25% of countries, including many countries with a high prevalence of hemoglobinopathies such as BT could not meet their national need for blood supplies.[5]

[1] https://ncd.gov/sites/default/files/NCD_Quality_Adjusted_Life_Report_508.pdf
[2] Politis C. Haemoglobinopathies: genetic and clinical aspects with an impact on blood transfusion. ISBT Science Series. 2013 Jun;8(1):229-32.
[3] Au WY, Lee V, Lau CW, Yau J, Chan D, Chan EY, Cheung WW, Ha SY, Kho B, Lee CY, Li RC. A synopsis of current care of thalassaemia major patients in Hong Kong. Hong Kong Med J. 2011 Aug 1;17(4):261-6.
[4] Currie CJ, Patel TC, McEwan P, Dixon S. Evaluation of the future supply and demand for blood products in the United Kingdom National Health Service. Transfusion Medicine. 2004 Feb;14(1):19-24.
[5] European Directorate for the Quality of Medicines & HealthCare Report of the survey on blood supply management Available from: www.edqm.eu/medias/fichiers/report_survey_on_blood_supply_management_2012.pdf, Accessed 15th April 2022

The red blood cells required for blood transfusions are a finite resource. Given this reality, the successful treatment of people living with TDBT would lead to benefit for patients with other blood disorders, as it would be less likely they would face shortages of necessary red blood cells. This system wide value gain is not incorporated into standard cost-effectiveness modeling, and this narrow perspective ignores the effects of redistribution of scarce natural resources. It is not unprecedented to construct a model that captures a treatment's broader impact on the health care system. There have been studies evaluating the wider public health value of successful treatment of hepatitis C and the resulting impact on the freeing up of essential organs for transplant and the resulting health gain to patients with conditions other than hepatitis C in the healthcare system.[6, 7] To fully capture the societal value of gene therapy in TDBT, economic models should also capture the value of reduced supply-demand shortfall for blood products in the healthcare system and the lives saved as a result.

ICER uses a Decision-Tree Markov Model to Estimate Cost-Effectiveness, Whereas a Time-To-Event Methodology Would Be a More Appropriate Mechanism.

ICER's model is centered on transfusion dependence: how often transfusions were required and how long a patient went without needing a transfusion. Given this desired outcome, the most effective model for comparing two scenarios (with and without gene therapy in this example) would be a time-to-event methodology such as a discrete event simulation model (DES)[8],[9] or a discretely integrated condition event (DICE) model.[10,11]

The one published economic evaluation of gene therapy treatment in beta thalassemia used a DICE methodology and uses largely the same sets of inputs as the ICER model but its conclusion was that Beti-cel was much more cost-effective than that estimated by the ICER model. These methods also allow for the integration of a wider set out of outcomes of interest throughout the course of the disease and the lifetime of the patient being simulated, which would help alleviate the problem with the ICER model's overly limited concentration on one aspect of the patient journey, transfusion dependence.

The mortality ratio for transfusion dependent beta thalassemia used in the model is at the low end of recent estimates.

The model developed uses 3.5 as an estimate of the standardized mortality ratio (SMR) for transfusion dependent patients – or patients in periods of transfusion dependence. Other estimates of the SMR in the

[6] Jena AB, Stevens W, Gonzalez YS, Marx SE, Juday T, Lakdawalla DN, Philipson TJ. The wider public health value of HCV treatment accrued by liver transplant recipients. The American journal of managed care. 2016 May;22(6 Spec No.):SP212-9.
[7] Jena AB, Snider JT, Espinosa OD, Ingram A, Gonzalez YS, Lakdawalla D. How does treating chronic hepatitis C affect individuals in need of organ transplants in the United Kingdom?. Value in Health. 2019 Jun 1;22(6):669-76.
[8] Standfield L, Comans T, Scuffham P. Markov modeling and discrete event simulation in health care: a systematic comparison. International journal of technology assessment in health care. 2014 Apr;30(2):165-72.
[9] Getsios D, Blume S, Ishak KJ, Maclaine G, Hernández L. An economic evaluation of early assessment for Alzheimer's disease in the United Kingdom. Alzheimer's & Dementia. 2012 Jan 1;8(1):22-30.
[10] Moller J, Davis S, Stevenson M, et al. Validation of a DICE simulation against a discrete event simulation implemented entirely in code. Pharmacoeconomics. 2017;35(10):1103–1109.
[11] Caro JJ. Discretely integrated condition event (DICE) simulation for pharmaco-economics. Pharmacoeconomics. 2016;34(7):665–672.

literature range from 6.2[12] to 13.5.[13] This likely means that the value of reducing need for transfusions and the reduction of patients in the transfusion-dependent states would have a much higher mortality impact than is currently reported in the draft ICER report.

The number of transfusions used in the model is likely low.

ICER's model assumes that patients under 18 years of age have an average of 14.95 transfusions per year, and patients 18 years of age or older have an average of 16.1 transfusions per year, which is based on MarketScan data reported in Kansal 2021. Other studies suggest this might be an underestimate of the burden of TDBT. Recent studies suggest 17-41 transfusions per year.[14,15] It is essential that ICER's model accurately reflect the patient experience, so we would encourage ICER to revisit these inputs to assure it is captured correctly.

Conclusion

We continue to encourage ICER to reevaluate its modeling choices, particularly the use of the discriminatory QALY, in future studies. We hope that our comments are useful to ICER in developing its final evidence report.

Sincerely,

Tony Coelho
Chairman
Partnership to Improve Patient Care

[12] Jobanputra M, Paramore C, Laird SG, McGahan M, Telfer P. Co-morbidities and mortality associated with transfusion-dependent beta-thalassaemia in patients in England: a 10-year retrospective cohort analysis. British Journal of Haematology. 2020 Dec;191(5):897-905.
[13] Ladis V, Chouliaras G, Berdoukas V, Chatziliami A, Fragodimitri C, Karabatsos F, Youssef J, Kattamis A, Karagiorga-Lagana M. Survival in a large cohort of Greek patients with transfusion-dependent beta thalassaemia and mortality ratios compared to the general population. European journal of haematology. 2011 Apr;86(4):332-8.
[14] Sheth S, Weiss M, Parisi M, Ni Q. Clinical and economic burden of transfusion-dependent β-thalassemia in adult patients in the United States. Blood. 2017 Dec 8;130:2095.
[15] Alshamsi S, Hamidi S, Narci HO. Healthcare resource utilization and direct costs of transfusion-dependent thalassemia patients in Dubai, United Arab Emirates: a retrospective cost-of-illness study. BMC health services research. 2022 Dec;22(1):1-7.

THALASSAEMIA INTERNATIONAL FEDERATION
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10 May 2022

TO WHOM IT MAY CONCERN

Subject: Comments of the Thalassaemia International Federation (TIF) on the Draft Evidence Report concerning betibeglogene autotemcel for beta thalassaemia

Dear Sirs,

The Thalassaemia International Federation (TIF)[1] takes the opportunity of this communication to provide its feedback, as representatives of the thalassaemia patient community, regarding the Draft Evidence Report developed by the Institute for Clinical and Economic Review (ICER) for betibeglogene autotemcel for beta thalassaemia and published on 13 April 2022.

A curative approach via gene therapy has been the enduring dream for more than three decades now by the thalassaemia patient community. Therefore, we would like to thank ICER for the opportunity to bring this aspect of the patient perspective to forefront.

Feedback on the Draft Evidence Report:

A most comprehensive, well-designed and expressed document, the Draft Evidence Report developed by ICER constitutes a state-of-art objective representation of evidence, considerations and assumptions concerning betibeglogene autotemcel for beta thalassaemia. It is indeed commendable that the inclusion of the patient perspective has been pursued every step of the way and that the independent panel of experts has focused on the clinical value of gene therapy for thalassaemia in its own merit, and not, as others have done previously, in comparison to the standard clinical management provided to patients.

The effort made to provide clarity, openness and in ambiguity through the review of evidence as well as taking into consideration the societal and personal burden of disease finds us in agreement, and we applaud

[1] The Thalassaemia International Federation (TIF) is a patient-orientated non-profit, non-governmental umbrella federation, established in 1986 with Headquarters in Nicosia, Cyprus. Our mission is to promote access to optimal quality care for all patients with thalassaemia worldwide. To-date membership boasts 240 members from 68 countries across the globe. TIF works in official relations with the World Health Organisation (WHO) since 1996, enjoys active consultative status with the United Nations Economic and Social Council (ECOSOC) since 2017, is an official partner in the field of Health for the European Commission since 2018 and a member of the Committee of International NGOs of the Council of Europe since 2019. Most remarkably, TIF has been awarded, in the context of the 68th World Health Assembly in May 2015, the *'Dr Lee Jong-wook Memorial Prize'* for the Federation's outstanding contribution to public health. More information about the Federation is available at www.thalassaemia.org.cy

the report conclusion outlining that despite the uncertainties (as explained in depth within the document), betibeglogene autotemcel for beta thalassaemia should be at least provided as a choice to patients.

The safety and efficacy of betibeglogene autotemcel for beta thalassaemia has been demonstrated by the approval of the European Medicines Agency (EMA) in 2019 and we are confident will be recognized by the Federal Drug Administration (FDA) in due course. Facilitating the accessibility and availability of betibeglogene autotemcel for beta thalassaemia following regulatory approval will enable the collection of real world evidence, which will further corroborate this report conclusion and if required its adjustment.

The Thalassaemia International Federation – an organisation representing the voice of thalassaemia patients in over 68 countries, including the USA, for more than 30 years, supports the right of each and every patient to be provided the opportunity to choose to undergo (or not) betibeglogene autotemcel. We hope that this decision-making process, will encourage other countries will proceed with their own assessment of betibeglogene autotemcel for beta thalassaemia, realizing its ultimate value for the patients and thus making it available and accessible to their own populations.

Hence, TIF remains fully committed to continuing its efforts to enable the accessibility of gene therapy for thalassaemia patients as a choice and therefore supports ICER's efforts for providing a complete report on the value of betibeglogene autotemcel as compared to standard of care services for beta thalassaemia, realizing its ultimate value first and above all for the patients.

On behalf of the Board of Directors of Thalassaemia International Federation (TIF),

Panos Englezos
President

Dr Androulla Eleftheriou
TIF Executive Director

Dr Michael Angastiniotis
TIF Medical Advisor

Steven Pearson, MD, MSc
Institute for Clinical and Economic Review
14 Beacon St Suite 800
Boston, MA 02108

Dr. Steve Pearson:

Vertex Pharmaceuticals Incorporated (Vertex) appreciates the opportunity to submit comments to the Institute for Clinical and Economic Review (ICER) on its ongoing assessment of Betibeglogene autotemcel (beti-cel) for beta-thalassemia.

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. Vertex has multiple approved medicines that treat the underlying cause of cystic fibrosis and has a robust pipeline of investigational small molecule, cell and genetic therapies in other serious diseases where it has deep insight into causal human biology, including sickle cell disease, beta-thalassemia, APOL1-mediated kidney disease, pain, type 1 diabetes, alpha-1 antitrypsin deficiency and Duchenne muscular dystrophy.

We are writing today to emphasize four key points regarding the recent ICER review of beti-cel:

1. We appreciate the recognition of the profound impact that transfusion-dependent beta-thalassemia (TDT), the most severe form of the disease, has on patients and their caregivers, encompassing both physical and psychosocial aspects of life. As acknowledged in the draft evidence report based on patient input, the existing standard of care, largely composed of lifelong blood transfusions and iron chelation therapy, is insufficient and burdensome; and the unmet medical need for a transformative therapy of this debilitating disease is paramount.
2. We are encouraged to see ICER's recognition of the clinical value of one-time potentially curative gene therapies for TDT that are likely to transform the treatment paradigm for these patients and society at large. In the economic evaluation, it is evident that these types of therapies have the ability to improve quality and length of life and reduce the substantial costs associated with current standard of care for this debilitating disease.
3. Considering the significant total healthcare cost of TDT is currently estimated at $5.4 million over a patient's lifetime[1], gene therapies with the potential to cure can not only improve patient outcomes, but also help alleviate the overall economic burden to the healthcare system by averting the costs associated with the lifelong management of the disease.
4. The clinical value of a therapy is independent of any particular payment model; we believe that a variety of coverage solutions may be valuable in supporting patient access to innovative therapies.

[1] Projected lifetime economic burden of transfusion dependent beta-thalessemia in the United States. Udeze et al. Abstract accepted at European Hematology Association Annual Meeting; June 9-12, 2022

Vertex supports the recognition of holistic value for cell and gene therapies, backed by a commitment to work together with payers, policymakers, and governments to deliver sustainable healthcare solutions. We stand together with the beta-thalassemia patient community and are steadfast in our commitment to bringing transformative new therapies to patients in need.

Sincerely,

Jaime Rubin Cahill, MS, MPH
Vice President, Health Economics and Outcomes Research
Vertex Pharmaceuticals Incorporated

[1] Projected lifetime economic burden of transfusion dependent beta-thalessemia in the United States. Udeze et al. Abstract accepted at European Hematology Association Annual Meeting; June 9-12, 2022