Health Impact of Optimizing the Time to Patient Access

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PRESENTED AT:

INTRODUCTION

- Before an oncolytic can be made available to patients under a formal reimbursement scheme, therapies are assessed first by the European Medicines Agency (EMA) and subsequently by the relevant national health technology assessment (HTA) authorities. These national authorities have their own HTA processes for assessing therapies and use their own assessment criteria (1). The length of the process differs greatly among EU member states. Consequently, the average time between marketing authorization and patient access varies greatly among the EU member states (2).

- Prolonged time between marketing authorization and reimbursement leads to missed health gains, which raises the question: what could be gained by ensuring earlier reimbursement?

METHODS

Health impact model

A health impact model was developed to calculate the impact of earlier reimbursement on the number of patients that could have been treated and the accompanying health gains of a treatment. The analysis included six European countries which together represent the diverse access contexts in Europe: England, Italy, the Netherlands, Poland, Portugal, and Sweden. Two oncolytics with positive reimbursement in multiple European countries were selected to demonstrate the health impact of access delay: midostaurin and pertuzumab.

Optimized time to reimbursement

Three hypothetical scenarios were used to represent optimized time to reimbursement:

1) Ambitious scenario: Timing of reimbursement coinciding with the European Commission (EC) marketing authorization (as is the case in Germany)

2) Best practice scenario: As fast as the fastest country

3) Basic scenario: Assessment within 180 days of the EC marketing authorization, unless the real-life situation is faster (as described in the EC Transparency Directive (3)).

Number of new patients per month

The number of new patients per month (uptake data) was retrieved from the respective companies marketing these drugs. Uptake data based on early access schemes (prior to positive reimbursement decision) was excluded. Only countries with recorded uptake data for at least one year after reimbursement were used in the analysis. In the scenarios, the number of new patients per month remained equal, but time to reimbursement (start of the uptake) changed.

Calculations

The health impact was calculated using three steps:

1. Determine the improved time to patient access by calculating the difference in days between the hypothetical scenarios and the actual reimbursement date.

2. Determine the additional number of patients that could have been treated by multiplying the difference in days with the average number of new patients per day. The average number of new patients per day can be calculated by dividing the cumulative number of new patients during peak sales (using the uptake curve and assuming peak sales will be reached at five years) by the number of days with peak sales.

3. Calculate the health gains per month based on publicly available information in the country-specific reimbursement dossiers, which could differ per country (4-12), by multiplying the number of patients with the incremental health gains per month, expressed in terms of overall survival (OS, per month), event-free survival (EFS, per month), life-years gained (LYG, translated into life-months gained, LMG) and/ or quality-adjusted life years (QALYs, translated into quality-adjusted life-months, QALMs) gained versus the comparator.

RESULTS - MIDOSTAURIN

- Uptake data of midostaurin for at least one year was available for four of the selected European countries: England, Italy, the Netherlands, and Sweden.
- In the ambitious scenario, 1,689 more patients could have been treated with midostaurin in England, Italy, the Netherlands, and Sweden, accounting for 82,920 LMG and 8,665 months of EFS (see Figure 1).
- In the best practice scenario, 672 more patients could have been treated with midostaurin in England, Italy, and the Netherlands, accounting for 33,020 LMG and 3,442 months of EFS (see Figure 2).
- In the basic scenario, 369 more patients could have been treated with midostaurin in England and Italy, accounting for 18,107 LMG and 4,876 months of EFS (see Figure 1).
- For the countries that lacked (sufficient) uptake data, even more health gains could be expected.

Figure 1. Number of days between EC authorization of midostaurin and approval by the HTA body

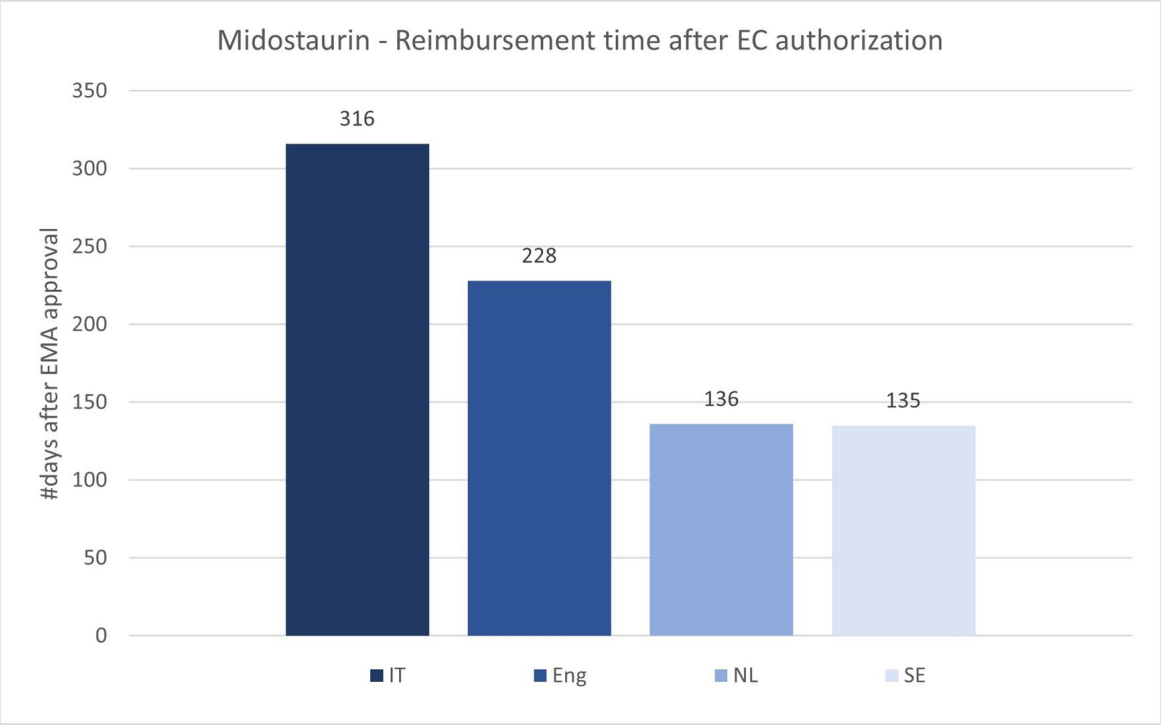


Figure 2 Number of additional patients that could have been treated with midostaurin

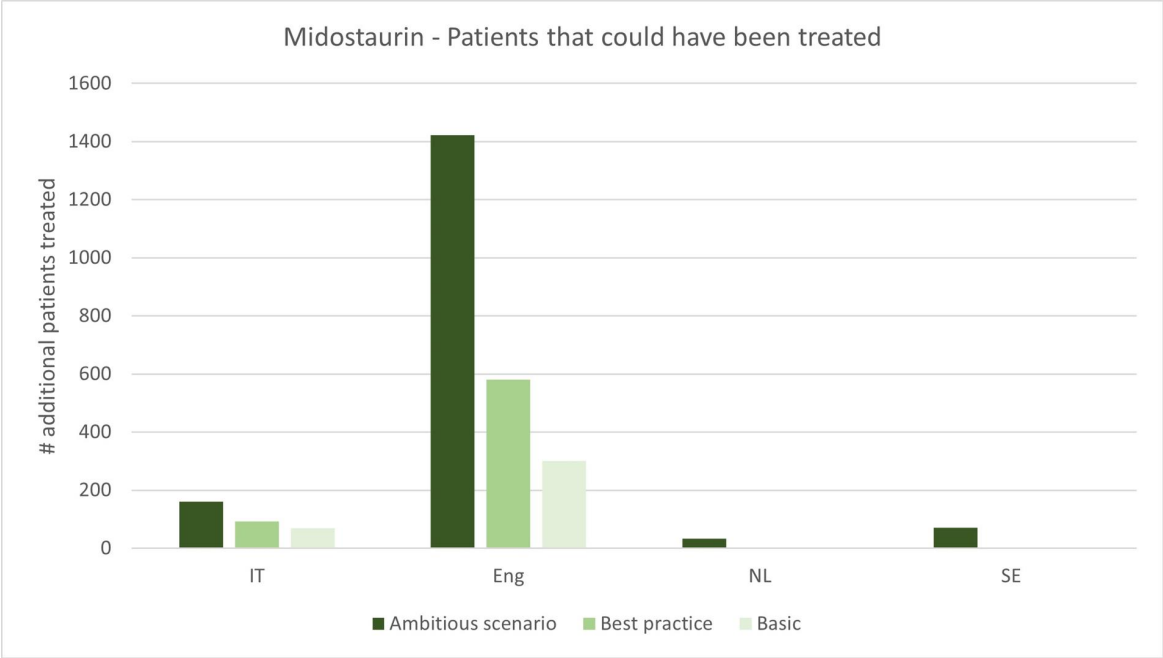


Figure 3. Number of additional life months that could have been gained with midostaurin

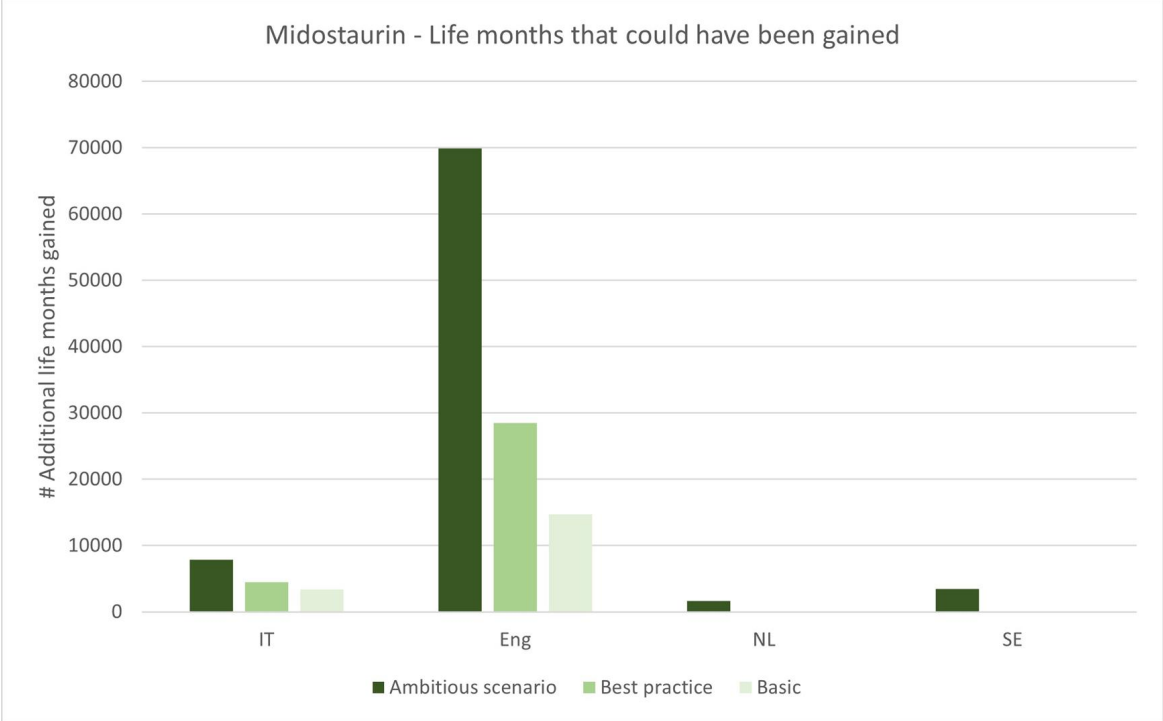
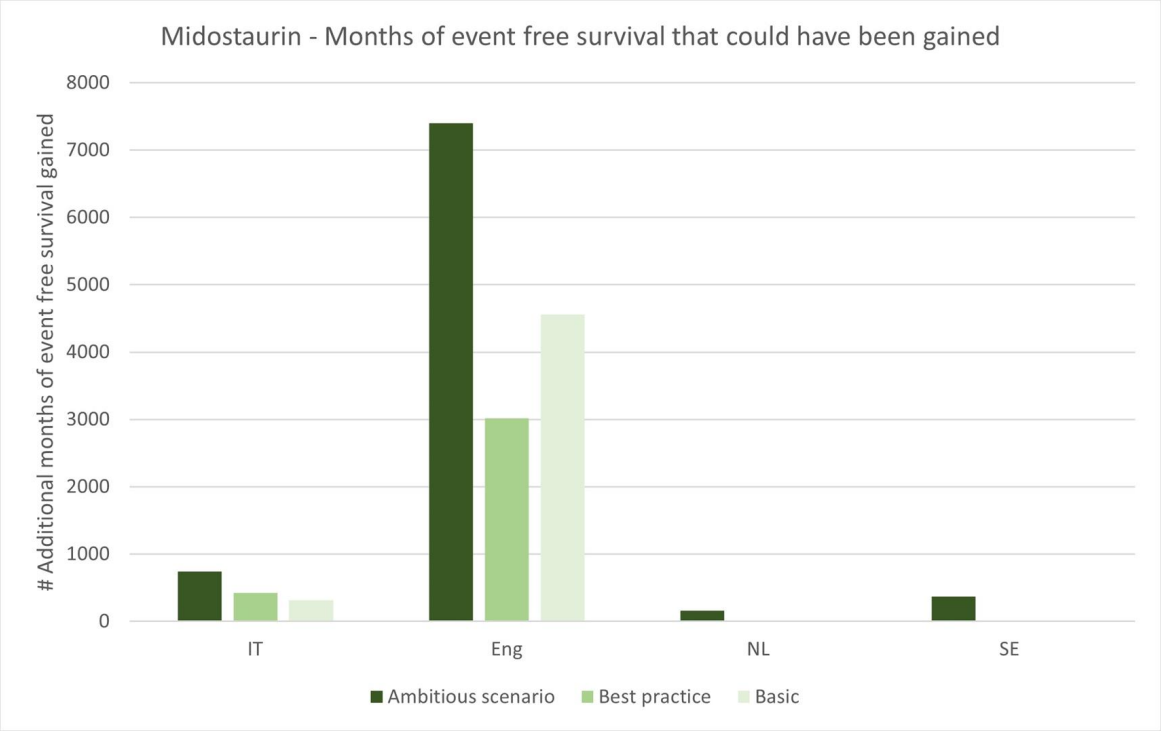


Figure 4. Additional months of event-free survival that could have been gained with midostaurin


Midostaurin - Months of event free survival that could have been gained
Additional months of event free survival gained
8000
7000
6000
5000
4000
3000
2000
1000
0
IT
Eng
NL
SE
Ambitious scenario
Best practice
Basic

RESULTS - PERTUZUMAB

- Uptake data of pertuzumab for at least one year was available for three of the selected European countries: England, the Netherlands, and Sweden.
- In the ambitious scenario, 2,181 more patients could have been treated with pertuzumab in England, the Netherlands, and Sweden, accounting for 12,718 LMG and 8,842 QALMs gained (see Figure 6).
- In the best practice scenario, 1,180 more patients could have been treated with pertuzumab in England and Sweden, accounting for 6,772 LMG and 4,797 QALMs (see Figure 7).
- In the basic scenario, 1,082 more patients could have been treated with pertuzumab in England and Sweden, accounting for 6,182 LMG and 4,407 QALMs gained (see Figure 8).
- For the countries that lacked (sufficient) uptake data, even more health gains could be expected.

Figure 5. Number of days between EC authorization of pertuzumab and approval by the HTA body

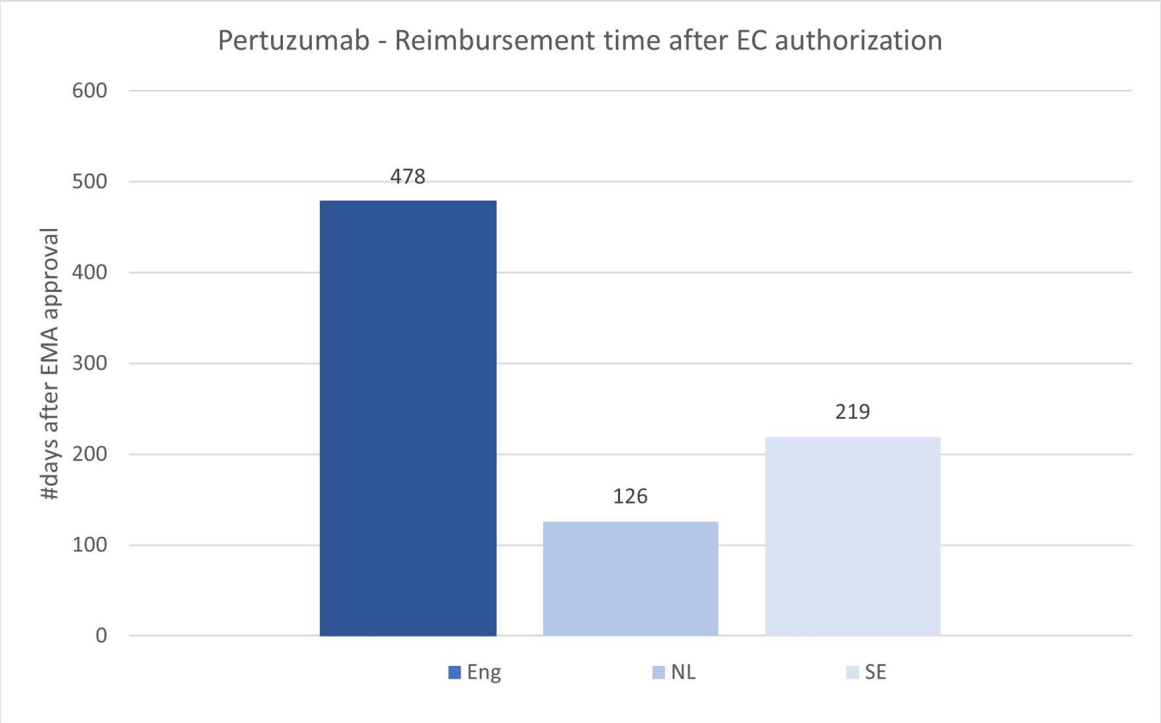


Figure 6. Number of additional patients that could have been treated with pertuzumab

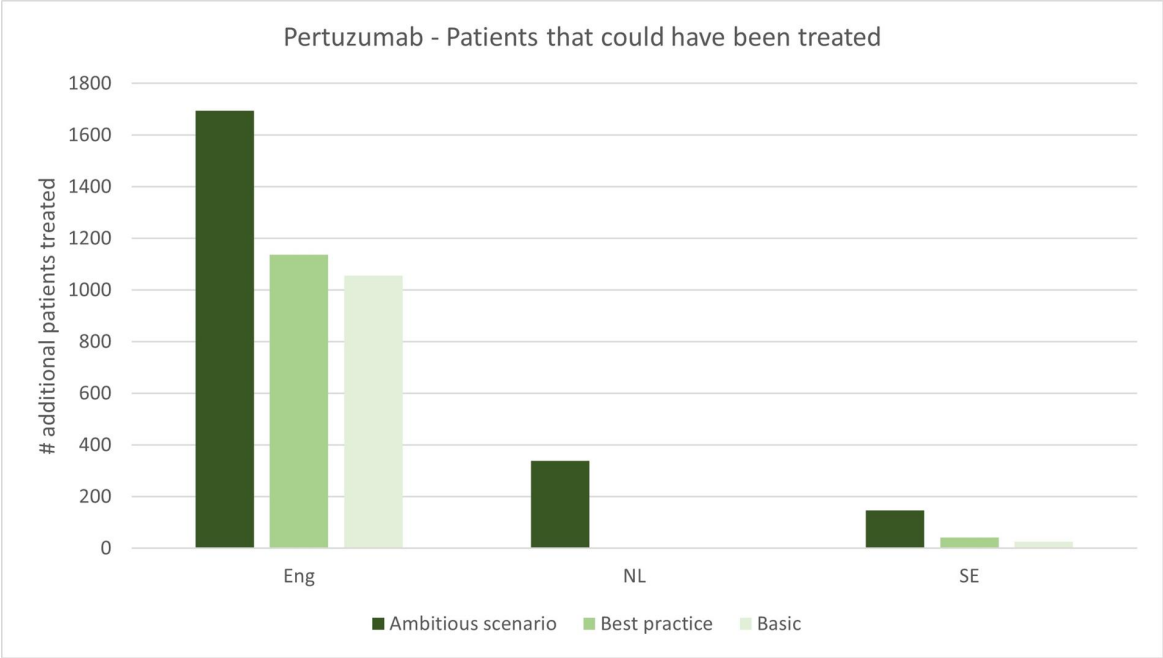


Figure 7. Number of additonal life months that could have been gained with

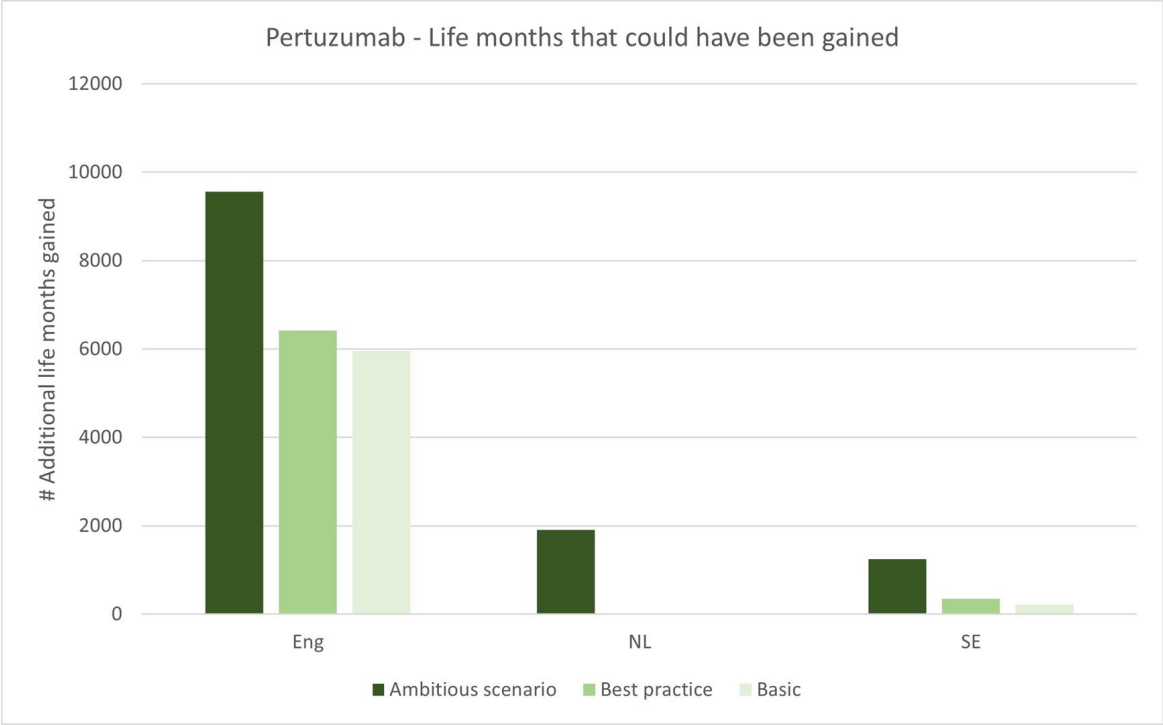
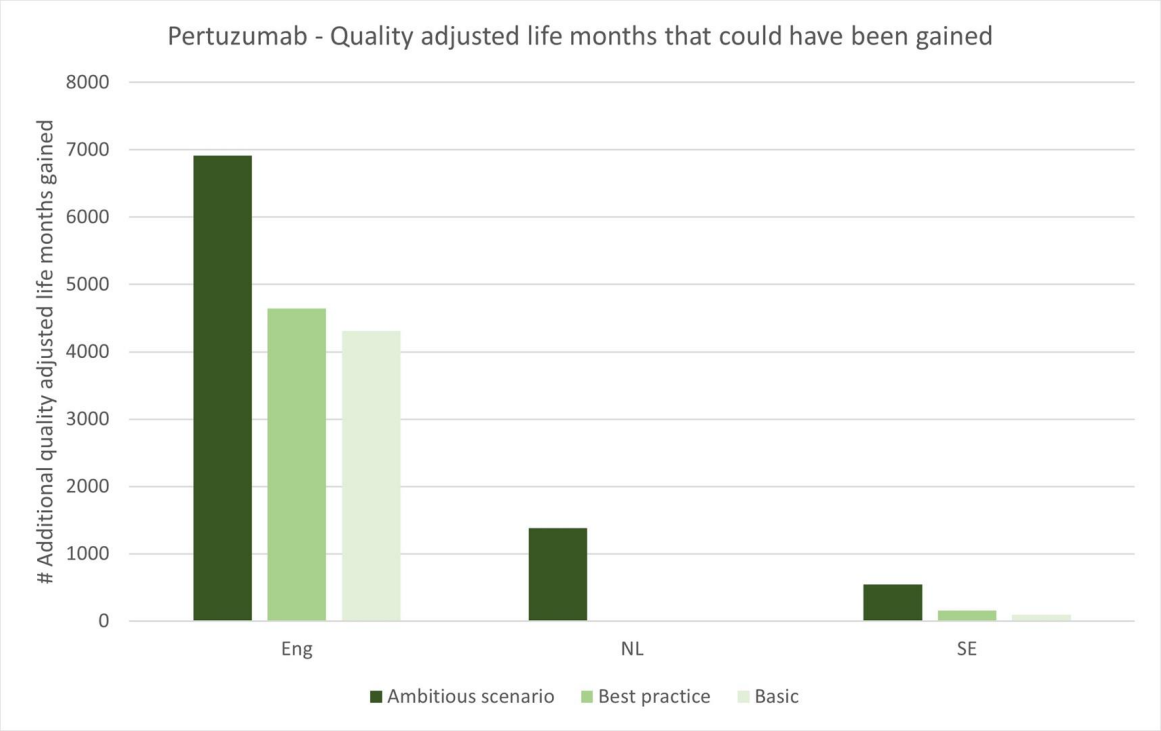


Figure 8. Additional quality of life months that could have been gained with pertuzumab


Pertuzumab - Quality adjusted life months that could have been gained
Additional quality adjusted life months gained
8000
7000
6000
5000
4000
3000
2000
1000
0
Eng
NL
SE
Ambitious scenario
Best practice
Basic

CONCLUSION

- During reimbursement discussions, time to patient access can become an abstract objective. Whereas for patients, every day counts. Today, time to reimbursement varies greatly between countries, which represents unequal access to innovative therapies within Europe. It also represents an opportunity for cross-country learning to optimize processes.
- The model demonstrated that, depending on the scenario, 369-1,689 more patients could have been treated with midostaurin in England, Italy, the Netherlands, and Sweden. It also demonstrated that, depending on the scenario, 1,082 - 2,181 more patients could have been treated with pertuzumab in Engeland, the Netherlands, and Sweden,
- This analysis quantified the health gains that can be realized through earlier reimbursement. These serve as a reminder of the fact that for patients, every day counts.

REFERENCES

1. Kleijnen S, Lipska I, Alves TL, Meijboom K, Elsada A, Vervölgyi V, et al. Relative effectiveness assessments of oncology medicines for pricing and reimbursement decisions in European countries. Ann Oncol. 2016; 27:1768–75.

2. IQVIA (2018). EFPIA Patient W.A.I.T. Indicator 2018 survey. Available from: https://www.efpia.eu/media/412747/efpia-patient-wait-indicator-study-2018-results%0A030419.pdf

3.European Commission. (1988, December 21). Council Directive 89/105/EEC relating to the transparency of measures regulating the pricing of medicinal products for human use and their inclusion in the scope of national health insurance systems. Official Journal of the European Communities.

4. EMA. (2020, March 16). EU/3/04/214. Retrieved from https://www.ema.europa.eu/en/ medicines/human/orphan-designations/eu304214

5. AIFA. (2018). Pubblicazione schede di monitoraggio Registro RYDAPT. Retrieved March 10, 2020, from http://www.agenziafarmaco.gov.it/content/pubblicazione-schede-di-monitoraggio-registro-rydapt-17082018

6. EUnetHTA. (2017). Midostaurin with standard chemotherapy in FTL3 positive Acute Myeloid Leukaemia. Retrieved March 10, 2020, from https://eunethta.eu/wp-content/ uploads/2018/01/PTJA01-Midostaurin-Final-Assessment-Report.pdf

7. Infarmed. (2019). Relatório público de avaliação de Rydapt (midostourina). Retrieved March 10, 2020, from https://www.infarmed.pt/documents/15786/1424140/Relat%C3%B3rio+p%C3%BAblico+de+avalia%C3%A7%C3%A3o+de+Rydapt+%28midostourina%29+2019/b1250881-7eb3-0d14-db6a-1cefc280f139?version=1.0

8. NICE. (2018). Midostaurin for untreated acute myeloid leukaemia. Retrieved March 10, 2020, from https://www.nice. org.uk/guidance/ta523/history

9. TLV. (2018). Rydapt ingår i läkemedelsförmånerna - 2432/2017. Retrieved March 10, 2020, from https://www.tlv. se/download/18.34b8d6741613e63f68048f6f/1517405911971/bes180131_rydapt.pdf

10. EMA. (2019). Perjeta Procedural steps taken and scientific information after the authorisation. Retrieved September 18, 2019, from https://www.ema.europa.eu/en/ documents/procedural-steps-after/perjeta-epar-procedural-steps-taken-scientific-information-after-authorisation_en.pdf

11. NICE. (2016). Single Technology Appraisal Pertuzumab for the neoadjuvant treatment of HER2-positive breast cancer [ID767]. Retrieved March 16, 2020, from https://www.nice.org.uk/guidance/ TA424/documents/committee-papers-2

12. TLV. (2015). Underlag för beslut i landstingen Perjeta (pertuzumab) Utvärderad indikation. Retrieved March 5, 2020, from https://www.tlv.se/download/18.467926b615d084471ac33ac8/1510316360905/Kunskapsunderlag_ perjeta.pdf