EU HTA 101 (Update from October 2024)

Dr. Thomas Ecker (Editor)

EU HTA 101

How to prepare for European Health Technology Assessment for Pharmaceuticals

Update from October 2024

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Preface

European Health Technology Assessment (EU HTA) is still evolving. In fact, it has not even begun. So why already an update?

The answer is simple: Products that will undergo assessment from 2025 onwards are already in clinical phase 3. Their study design was set long ago. This means that many companies do not have the luxury of "waiting and seeing" until the dust settles. They have to make crucial decisions now to prepare as best they can for a scenario that could not have been anticipated earlier. This urgency to prepare applies not only to them but also to many others who will soon face similar decisions. Both are the audience of this book.

And a lot has happened since the publication of the first edition in October 2023, not least four Implementing Acts and six guidance documents, and one scientific specification.

Most of you might be familiar with preparing national HTA submissions. But EU HTA is different. And it also impacts national submissions. So take the time and reflect upon this new challenge.

The structure of this book follows our Market Access Core Model®. At the time of finalizing this book we are at version 2.7 and will continue to evolve, in parallel to adoption of the EU HTA process.

Three words of caution: This book was completed in October 2024, and any changes thereafter have not been taken into account. However, we aim to provide a timely update.

Additionally, it is important to note that the focus of this book is on medicinal products, specifically pharmaceuticals, rather than devices and diagnostics.

Finally, this book provides insights towards an ideal EU HTA process. As such, in bears great promises. But reality not always lives up to this ideal. And especially during the first years of implementation scope of surprise will be rich. So be careful by setting realistic expectations.

Special thanks to all members of our European Partner Network, covering all 27 EU member states, all EEA countries, as well as Switzerland and UK. You provided excellent feedback on the applicability of the Market Access Core Model® to your country, and great input towards our internal PICO exercises.

This update would not have been possible without the incredible support of our EU HTA expert group and the contributions of many authors within Ecker + Ecker and Accessus Health.

Thank you very much – you are a fantastic team!

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1 Market Access Core Model

Dr. Thomas Ecker[1]

From the outside, the proposed EU HTA might seem like a complex maze of rules and regulations: 36 articles packed into 32 pages just for the regulation itself, in addition to more than 60 high-quality deliverables from EUnetHTA 21– not to mention the various Implementing Acts and corresponding guidelines, most of them still under development. All of these components are aimed at supporting national Health Technology Assessment (HTA) and Pricing & Reimbursement (P&R) as thei main pillars of market access in 27 countries. It is easy to feel lost, even for those who consider the general concept of HTA to be common knowledge. So how can you see the forest for the trees?

This book does not aim to provide a detailed description of individual national market access systems within the context of EU HTA. Instead, it focuses on outlining general principles, enabling the reader to gain a comprehensive overview. To achieve this, adequate methodology is required. This should be generic enough to apply to most scenarios yet specific enough to offer practical guidance. This is where the Market Access Core Model comes into play.

Let's begin with the common denominator and the underlying basic idea: HTA in individual European countries predates EU HTA by several decades. As more and more countries adopted their own specific HTA procedures, the need for harmonisation and synergy at the European level became obvious. After all, the European Medicines Agency (EMA) covers 30 countries – Austria, Belgium, Bulgaria, Croatia, Cyprus, Czechia, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Iceland, Ireland, Italy, Latvia, Liechtenstein, Lithuania, Luxembourg, Malta, the Netherlands, Norway, Poland, Portugal, Romania, Slovakia, Slovenia, Spain, Sweden – and each has its own unique market access procedures. But if all of these procedures are different, what could possibly be the common denominator?

The answer lies in recognising that the individual market access procedures share a common link: the assessment of the clinical (and economic) evidence for medicinal products, specifically their HTA. It is through these interconnected assessments that the national market access procedure is formed, representing one of the key challenges when launching a new medicinal product (Figure 1).

Figure 1: National market access as the key challenge

For the scope of this book, the term "national market access" encompasses both the national Health Technology Assessment and the national Pricing & Reimbursement (P&R) processes (Figure 2).

[1] TE prepared the first edition of this chapter and updated this chapter in October 2024.

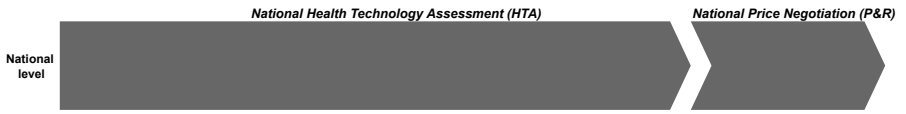

Figure 2: National market access encompassing both national HTA and national P&R

From the perspective of a pharmaceutical company, the national market access work stream can be delineated into four distinct phases (Figure 3).

Figure 3: Four phases of national market access

These four phases comprise the national work stream of the Market Access Core Model:

- National strategy development: This phase involves identifying the appropriate reimbursement process, simulating the likely outcome of the evidence evaluation and pricing decision, and developing the market access strategy based on these insights. Additionally, if applicable, the proposed approach is discussed with the pertinent authorities through a national scientific consultation, while also monitoring other (similar) procedures.
- National dossier production: In this phase, the strategy developed in the previous phase is executed by defining the outline of the evidence package (dossier), formulating the HTA Statistical Analysis Plan (SAP), and conducting the corresponding statistical analyses. It also encompasses the preparation of the actual dossier, quality check, dossier submission and product launch (if applicable).
- National assessment: Once the dossier is submitted, the national assessment phase begins. This involves developing the assessment strategy, interacting with authorities, and reviewing the final documents.
- National price negotiation: This phase encompasses developing the negotiation strategy, preparing the negotiation, conducting the actual negotiation process and resolution procedure, notifying the relevant authorities of the final price and reimbursement conditions, and, if needed, regional negotiation including tenders.

Lastly, stakeholder management and monitoring activities are crucial and should be addressed continuously throughout all phases of national market access.

So far, so good!

EU HTA is an additional work stream that runs parallel to national market access (Figure 4).