

A Multilevel Access Evidence Framework for Innovative Technologies Specific to Haematological Malignancies

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Objective – Making innovative treatments available across the European Union is a complex process due to differing evidence requirements, assessment methods and value judgements by HTA agencies. The HARMONY Alliance is a big data for better outcomes project of the Innovative Medicines Initiative (IMI). HARMONY is a public-private partnership with over 90 organisations from 22 European countries with varying expertise in evidence development strategies to support new treatments and indications.

We aimed to produce a guide for the evidence required for market access and coverage/ reimbursement ("access evidence") focussing on challenges facing innovative haematological malignancies (HM) drugs and the jurisdictional differences across Europe.

Methods and results – We developed a multilevel framework based on methodology guidelines, interviews and previous submission reports and documentation from key selected countries: France, Spain, Norway, Sweden, England, Ireland, Germany, Italy, Poland, Estonia and Slovenia and EU wide (EMA, EUnetHTA) and umbrella payer organisations.

Level 1: The Market Access Landscape Cyclopaedia

Interviews, response rates were 70% (19/27) with HTA agencies (n=13), regulators (n=6) and payers (n=2) focusing on the themes detailed in diagram 1 along with methodology guidance and pragmatic literature searches forming the basis of the cyclopaedia.

This level is designed to act as a signposting tool to raise awareness of the regulatory/HTA and payer landscape and to present terminology, highlighting HM specific outcome requirements where available. Additionally this level has country specific information pages /dash boards with links to methodology and guidance documents at national level HTA, payers, regulatory bodies, service provision and patient co-pays.

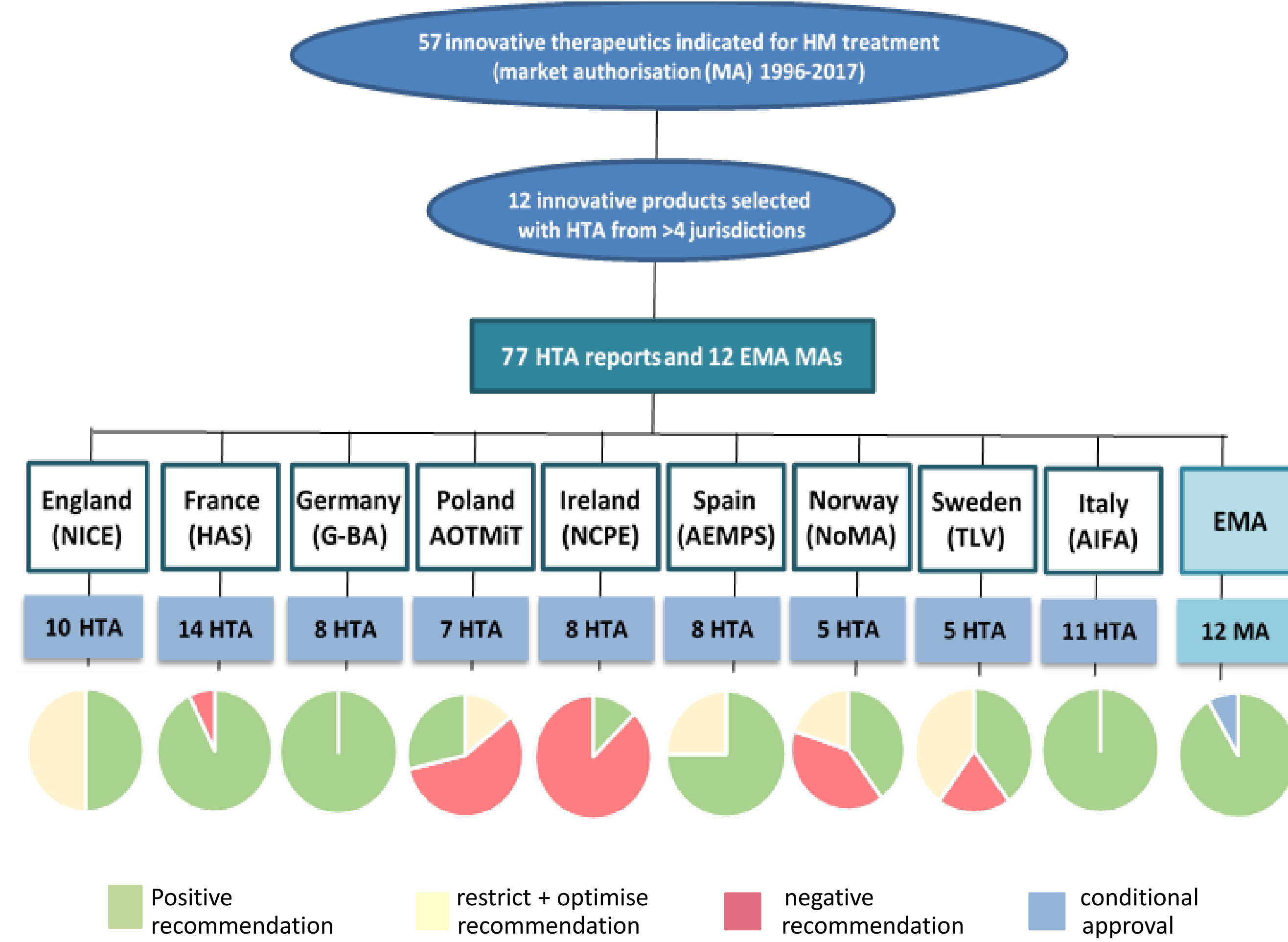
Level 2: HM Outcome and Evidence Inventory

Consists of a series of easy access dashboards incorporating domains and topics from the HTA Core Model® including safety, clinical effectiveness, cost and economic evaluation and ethical and organisational aspects. This level is populated from data extracted from 77 previous reimbursement decisions on 12 HM products across 9 HTAs. The framework highlights key differences/requirements and evidence use by both HTA and disease indication, and how these may impact on recommendations for reimbursement. Each series of dashboards includes summary text placing the findings in context with current literature and HTA method guidelines.

Practical implications – The freely available access evidence framework is anticipated to have several benefits for HARMONY and HM researchers. Developed as a unique resource for this clinical area it provides a concise point of reference with key learnings and requirements for HM evidence generation across the EU. It allows a direct comparison across differing agencies on the acceptability and use of evidence for key markets and could potentially increase the efficiency in generating and preparing relevant access evidence but also of predictability of HTA outcomes.

Resources and data underpinning the framework

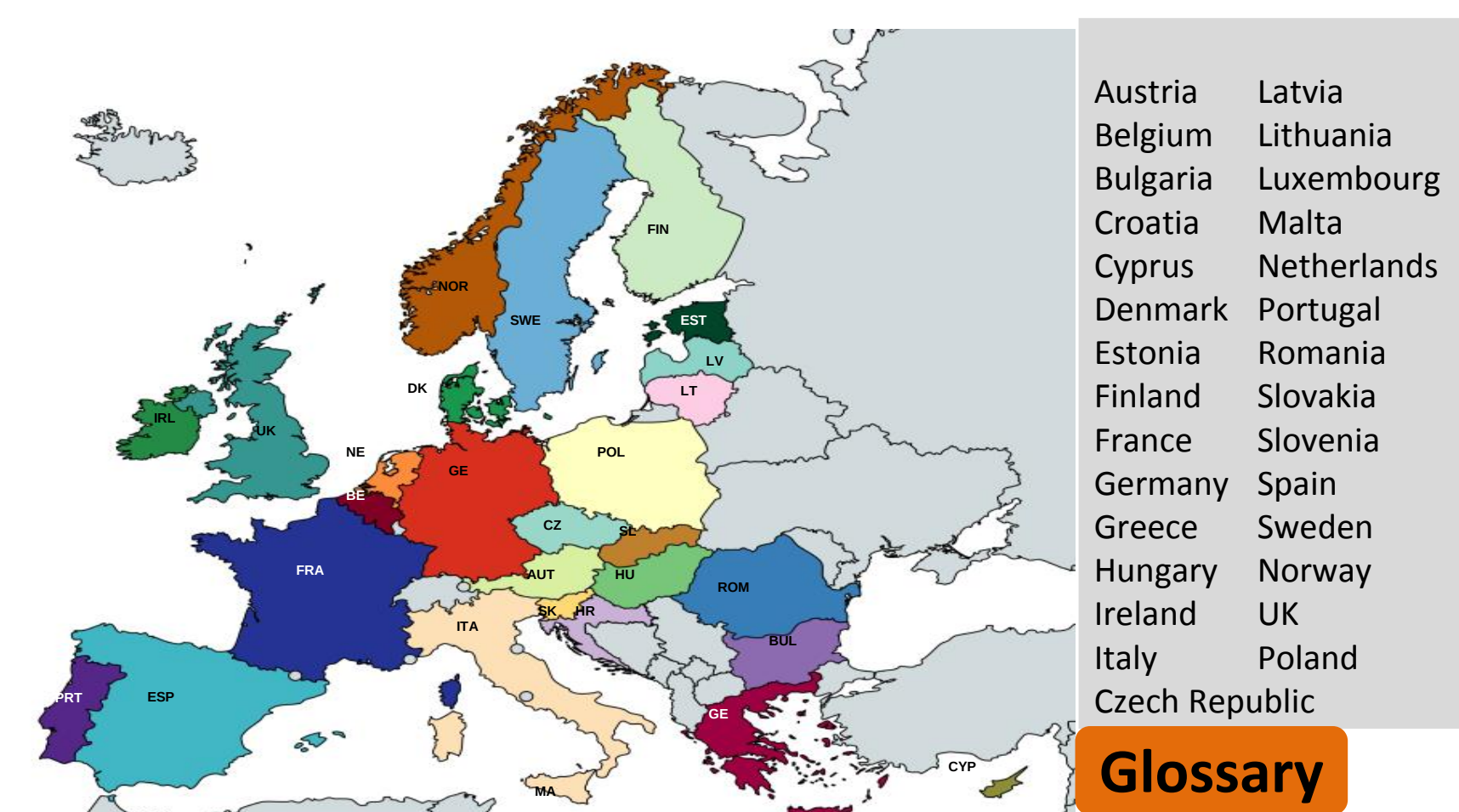
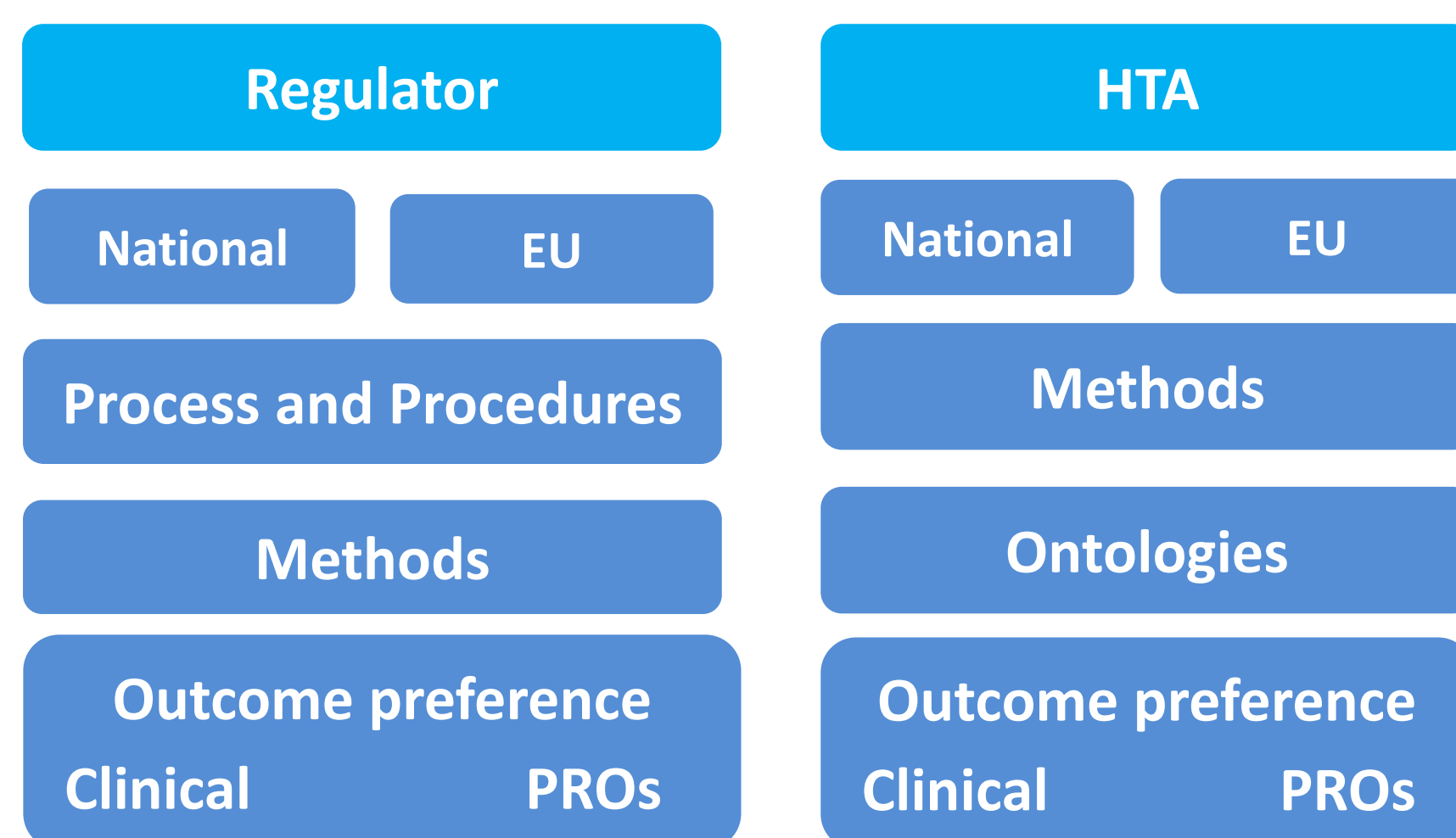
Diagram 1: Survey, interviews and web based searches with HTA/regulators and payers



HM Access Evidence Framework & illustrative dashboards

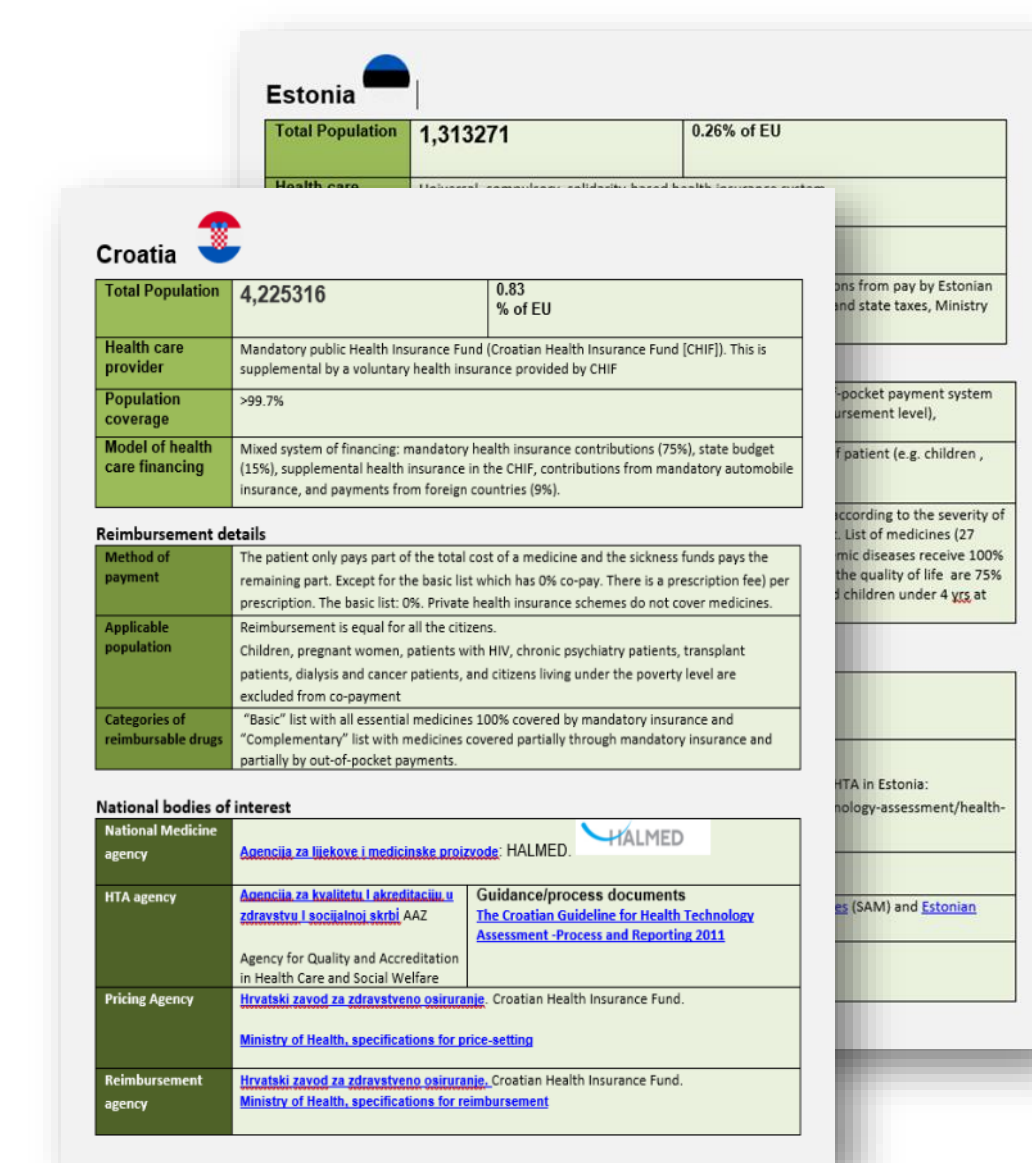
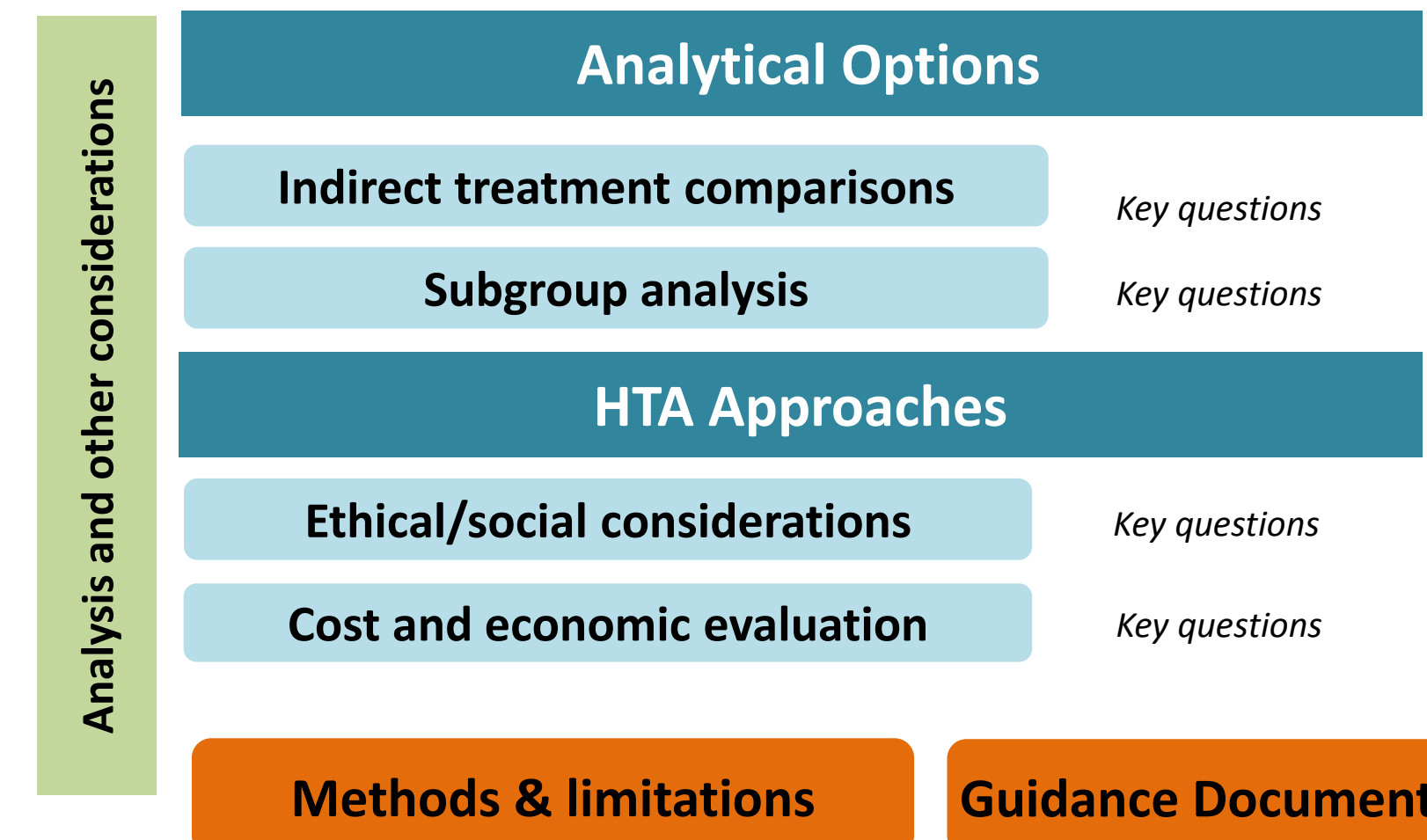
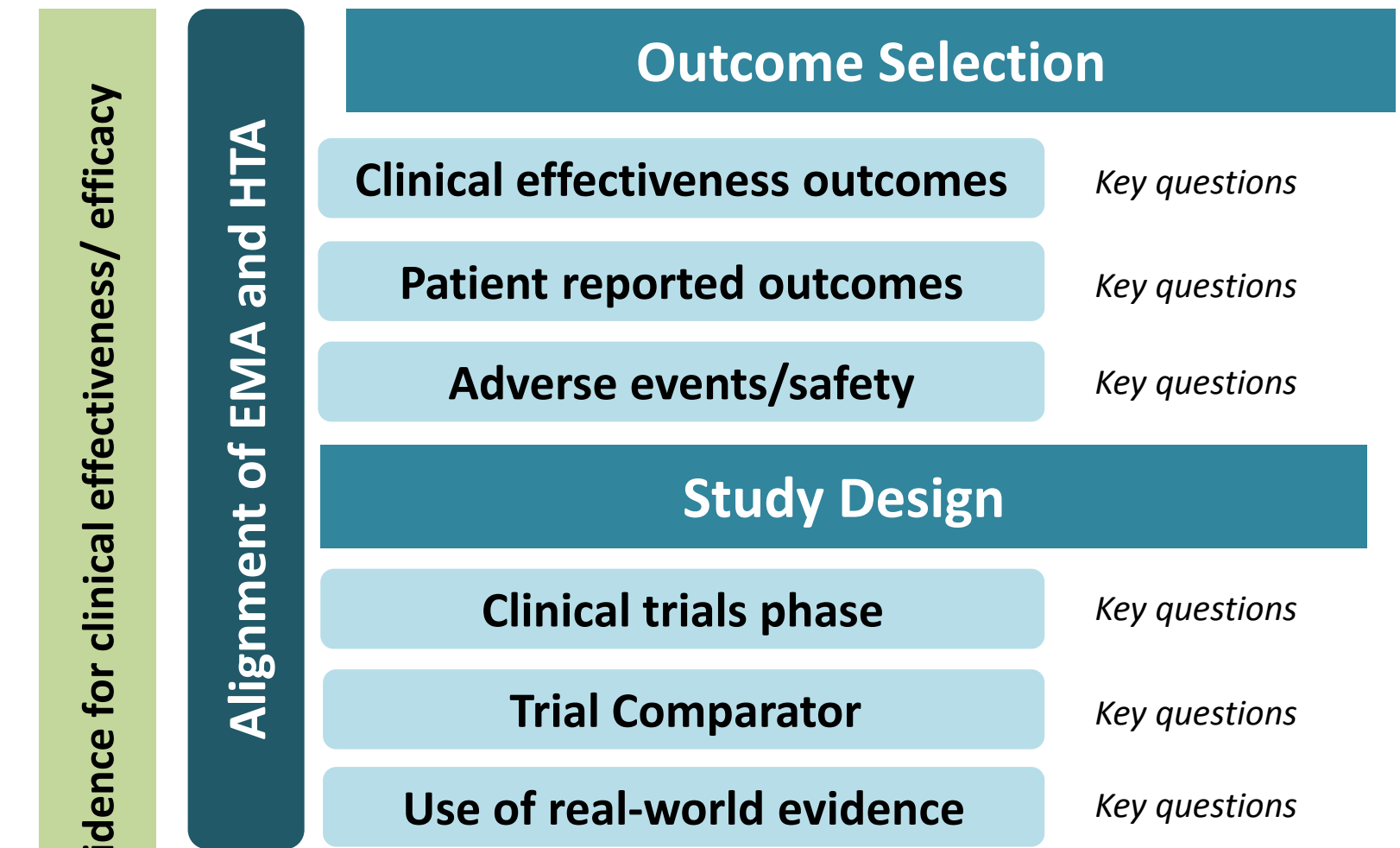
Level 1: The Market Access Landscape Cyclopaedia

Overall EU pharmaceutical market access process



Glossary

Level 2: HM Outcome and Evidence Inventory



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