Kick-off meeting
January 24-25, 2023
Rotterdam, the Netherlands

Summary Roundtables

Overall

1. Efficient access to cancer care: Improving access to cancer care is an overarching goal of all the roundtables. The discussions centered around the need for a fair process for pricing and reimbursement decisions, the importance of timely and accurate cancer diagnosis, and the need for a better patient pathway.
2. Pricing and Reimbursement: The participants acknowledged the connection between pricing and reimbursement and emphasized the need for a fair process that ensures equitable access to health technologies in the EU while supporting innovation and competitiveness. A fair process was seen as one that considers the unmet needs of patients and the sustainability of the healthcare system while ensuring equal access to information and accountability.
3. Access to innovative treatments: Accessibility to innovative treatments could be improved through collaboration between member states and pharmaceutical companies, the use of outcome-based measures, and the implementation of performance measures. Contractual agreements on reimbursement models should be transparent, outcome-based, and include a risk-sharing component.
4. Diagnosis: Timely and accurate cancer diagnosis was seen as essential for patient outcomes and the overall success of the cancer care pathway. The importance of access to specialized diagnostic techniques and services was highlighted, as was the need for collaboration between diagnostic and treatment providers.
5. Patient pathway: A better patient pathway was seen as necessary for improved patient outcomes and access to care. This included the need for more patient-centered approaches, better coordination between different stages of the pathway, and the use of technology to improve the overall experience for patients.
6. Support for cancer research: The importance of cancer research for improving patient outcomes and access to care was emphasized. This included the need for increased investment in cancer research, the importance of collaboration between researchers and healthcare providers and other stakeholders, and the need for better sharing of data and results.

Successful outcomes of the ASCERTAIN project with regard to an enhanced approach in pricing of innovative health technologies

1. Patients have access to at least 1 good and affordable treatment option in Europe.
2. Having methodology/ tools that other projects can use to better determine price/ a fair price.
3. More transparency and criteria that determine price.
4. Value elements that could be weighted and help determine price and every stakeholder agrees upon.
5. Harmonization on EU level.
6. Consensus on specific factors to integrate in a method to determine price and have all stakeholders embrace this.
7. Agreements on key performance indicators between countries to have a stronger negotiation position against pharmaceutical companies and companies that produce medical devices.

Highlights

Topic: Pricing

High prices of new medicines are frequently mentioned as a key challenge with regard to access to medicine. New approaches for pricing innovative technologies should try to find a balance between improving patient access and stimulating entrepreneurship.

- To determine the price, you should balance: what is a healthy company and a healthy society.
- Important aspects of the product that should determine the price:
    - Quality of the product
    - Safety of the product
    - Risks versus benefits.
- The unit price of a product should be the same for all different indications.
- We could also use a cap just as was done for gas prices to reduce excesses. But we should keep in mind that prices also should not be too low, to prevent out of stock.
- What are objective criteria to set limits to prices:
    - The price should depend on how much better treatment x is compared to the current usual care
    - A good level of healthcare
    - Income level of a country
    - A threshold per QALY (depending on national income)
    - A threshold for budget impact (depending on national income)
    - Quality recalls happen often for medical devices: the higher the number of recalls the lower the price should be.

Model options:

- ✓ A new product should be priced ± x percentage (e.g. 10%) lower than the current alternative treatment and must be at least as save as the current alternative treatment.
- ✓ Guaranteed price volume arrangements.
- ✓ Use a yearly tender, which will be set out by the EU (as "one country").

Additionally:

- More regulation is needed for medical devices
- HTA specific for medical devices is needed
- Healthcare professionals seem to have a stronger relationship with a medical device than a drug.

- Important to distinct between 'value' for each different stakeholder perspective: Patients, Society, Industry, Investors.
- Currently, 'value-based pricing' refers both to marketing-driven value prepositions created by industry and real experienced patient benefits. This makes it unclear what we mean by 'value' when discussing value-based pricing.
- Proposals for pricing methods that reward innovation.
- Financially delinking R&D phase of a drug from commercial/production phase
- Accounting for/correcting for mergers and acquisition.
- Using a sort of 'pricing equation' with all value elements:
  Example Price= (production costs + R&D + profit margin) + (innovation parameter + value to patients + value to society) + (environmental impact)
- Important to have an access component in pricing model; since price predicts access to patients and this is a value element.
- Stimulating competition.
- Transparency of pricing and prices:
  - Industry perspective: prices are not maximized but optimized
  - Payer perspective: if 'optimization' means adapting the price based on prices of other products and potential impaired access, then 'optimization' is also unethical
  - This seems to connect to the overarching issue: can we expect pharmaceutical industry to not optimize prices if we allow them to be profit-oriented companies in a capitalist market? → important to define what we mean by 'market failures' and when governments are allowed to correct for these.
- Payer stakeholders point out: transparency is needed on costs of failures and other costs that eventually determine profit margin.
- Issue of strategic pricing by industry:
  - Industry perspective: prices are not maximized but optimized
  - Payer perspective: if 'optimization' means adapting the price based on prices of other products and potential impaired access, then 'optimization' is also unethical
  - This seems to connect to the overarching issue: can we expect pharmaceutical industry to not optimize prices if we allow them to be profit-oriented companies in a capitalist market? In that respect it is important to define what we mean by 'market failures' and when governments are allowed to correct for these.

Topic: Cost-effectiveness

Challenges for decision-making in EU countries.
HTA and ICER should be used in every country, but this requires sufficient data.
In some cases, i.e., rare diseases, it's challenging to have an RCT. However, these patients also need access to the new medication.
Potential solutions for this can be:

1. Single-arm trial with external control arm
2. Modeling.

The patient treatment is a lot more than just the medication; the differences in diagnosis or daily practice also need to be adjusted when using this model in different countries. In this case, there couldn't and shouldn't have one "Golden standard" for all EU countries. However, countries with similar clinical practices and healthcare systems can be grouped as a cluster.

What kind of solution do you expect ASCERTAIN to bring?

a. Markov models
b. Bring the experiences of healthcare experts into HTA.
c. PICOs adapt to different countries' populations/practices.
d. A solution to help makes the objective, correct decision when there's little information available.
e. Better data collection option than a disease registry.
f. Data access would not be problematic in the long run with the help of a converged database(name?). But the costs of data could be an issue.

Others:

g. Be as transparent as possible, which can be helpful for future data updates, replacements, or adjustments.
h. A new method can be helpful: Combining CEA with BIA.
i. A shared CEA framework for all EU countries is needed.
j. Consider patients' compliance in the CEA model to better reflect real-world situations.

Challenges for decision-making in EU countries

a. Small sample size in some rare disease studies.
b. EMA already accept single-arm trials as a substitute for RCT in the approval application. Should single-arm CEA be sufficient as well? Accepting the single-arm CEA and then reevaluating with the newly collected data afterwards can facilitate early access, with risk remaining controllable.
c. Quality of clinical trials: The RWE simulation can help with the single-arm trial but could not help with trials with a small sample size.
d. The feedback mechanism is missing after the market access. The medication price should be adjusted after the new performance data is available.
e. The ethical problem for gene therapies often leads to a single-arm trial.

Topic:Broader HTA

The domains most often mentioned are described in the figure below.

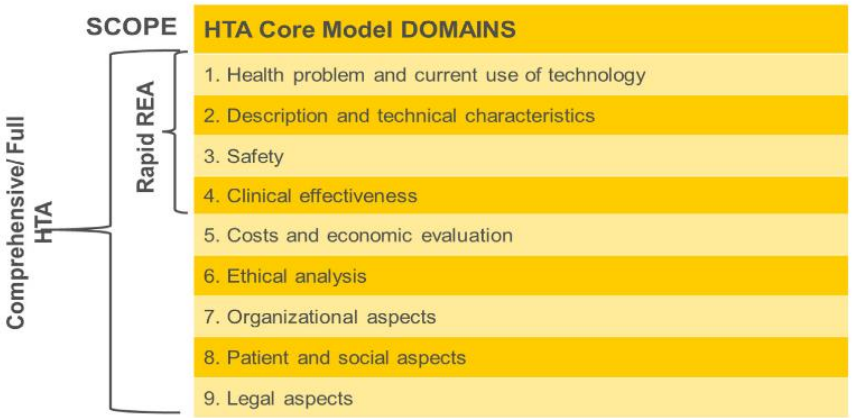
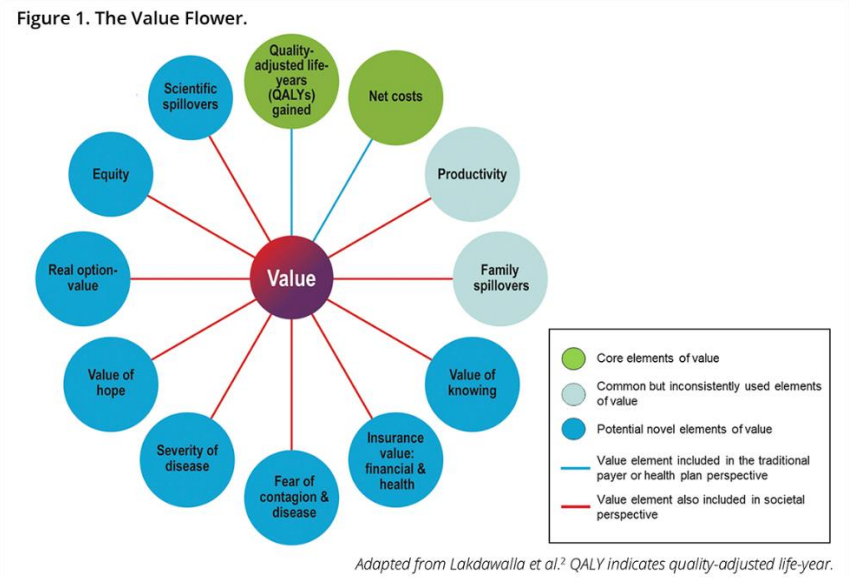


Fig. 1 – The domains of the HTA Core Model. HTA, health technology assessment; REA, relative effectiveness assessment.

Expanding Value Elements in HTA Frameworks

What are the values to include besides net costs and Qalys? See the ISPOR Value Flower. We have focused on:

- Value of hope:
- Severity of the disease
- Patients' preferences.



Environmental aspects should be added, e.g., by comparing the drug life cycles or by comparing patients' pathways of interventions.

a. The form of medicine changed (IV to OP) can already significantly impact the environment.
b. Some healthcare products or pathways are cost-effective but also impact the environment significantly.
c. The supply chains of medicine often don't consider the carbon footprint. The supply chain of a single medicine could spread across continents.
d. The logistics can be adjusted to contain the footprint if the pharmaceutical companies are incentivized to do so.

Topic: Reimbursement

The themes discussed were

- Transparency of patient access schemes (PAS)
- Running a PAS
- Education and communication of PAS
- Examples

Points for ASCERTAIN

- Where does the outcome data land and who is the owner of that.
- Broaden the maps of Myeloma Patients Europe (MPE) on medication accessibility across Europe already have for other indication.
- Form a base on education and communication on the role of PAS; for example, education on the fact that if one technology will enter the market the other will be removed.
- Broaden the field and scope of care, have a more personalized approach, and magnitude the HTA scale by for instance forming a base to assess the right order of drugs (first, second, third…. line). This could also form a base to give the physician more visibility in the financial impact and thus the financial aspects.
- Including the bigger countries of Europe, in this example France, to broaden the scope of the project.

Uncertainties and challenges

Substantial uncertainty is spurred by the imbalance of power between payer and pharmaceutical manufacturer. Moreover, it must be kept in mind that the actual decision-making process on reimbursement falls under the competence of each member state and is therefore, currently, a national matter. This means that a joint implementation of a reimbursement model might (currently) not be possible from a legal point of view. Neither is it feasible in view of the different reimbursement criteria applicable in each country. Nevertheless, especially when need is high, rigorous changes can be made (e.g., COVID initiatives) and a joint contractual agreement can lay the groundwork for the common goal of reimbursement.

Further, an important uncertainty aspect concerns the outcome-based measures underpinning the reimbursement model. Uncertainty regarding long-term outcomes constitutes a prominent obstacle. Reimbursement models should ensure a proper balance between risks and benefits for all stakeholders. When risks are transferred (either to private companies or to the health care payers) paid prices should be adjusted accordingly. In addition, performance measures in a real-life setting are subject to uncertainty.