Assessment of reimbursed price potential for a cell therapy in development

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Globally, pricing approaches in healthcare are shifting towards value-based models

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<tr>
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<th>Cost-based</th>
<th>Competitor-based</th>
<th>Value-based</th>
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<tbody>
<tr>
<td><strong>What is it?</strong></td>
<td>• Price is set by assumptions on costs, expected sales volumes and margins</td>
<td>• Price is driven by the pricing of competitor products</td>
<td>• Price is based upon therapeutic/economic value to the customer</td>
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<td><strong>Examples</strong></td>
<td>• Cost-plus pricing</td>
<td>• Penetration pricing</td>
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<td>• ROI based pricing (e.g. PPRS in UK)</td>
<td>• Reference group pricing</td>
<td>• Value-based pricing</td>
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<td><strong>Comments</strong></td>
<td>• Becoming obsolete; no longer resonates with payers</td>
<td>• Enforced by many reimbursement systems for “undifferentiated” products</td>
<td>• Typical approach for differentiated products</td>
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Value-based assessments link price potential to the magnitude of the novel therapy’s added-value over the standard of care (SOC)

**PRINCIPLES OF VALUE-BASED ASSESSMENTS**

\[ V = RV + PDV - NDV \]

**Differentiating Value (based on TPP*)**

- Includes:
  - Clinical effectiveness
  - Economic effectiveness: budget impact, cost-minimisation, cost-effectiveness, cost-utility, cost-consequence

- Comparative data against the SOC is required:
  - *Head-to-head* comparisons demonstrating superiority or non-inferiority is preferred
  - Indirect comparisons may only suffice for non-inferiority claims

**Value (V)**

- For a given indication, “V” varies depending on the intervention’s positioning in the treatment algorithm and subpopulation

*TPP: Target Product Profile*
In developing a robust pricing strategy for innovative therapies we leverage multiple frameworks.

Methodology Triangulation

QUAL/QUANT PRICING METHODOLOGIES

PRICE

HEALTH ECONOMICS

ANALOGUE ANALYSIS

The role of health economics varies by geography.
For therapies that meet NICE TA selection criteria, its ICER threshold is used to inform price potential

\[
\text{ICER} = \frac{\text{Cost B} - \text{Cost A}}{\text{QALY B} - \text{QALY A}}
\]

QALYs = Life expectancy (life years) x Quality of life (utility)
- Utility ranges from 0 (death) to 1 (full health)

Costs
- Direct healthcare costs rather than societal costs

**NICE ICER thresholds**
- £20-30K/QALY; exact figure depends on:
  - Certainty around ICER
  - How adequately QoL is captured
  - How innovative the technology is
- For end-of-life treatments and for small populations a higher threshold might be considered by the Committee
  - provided they extend life by ≥3 months

**POTENTIAL REFORMS**
Incorporation of wider societal impact and disease burden
To calculate the ICER a model is developed that captures health states, time-dependent transitions, outcomes and uncertainty

**We define:**

- Perspective (NHS, Societal)
- Target population (based on TPP)
- Current therapeutic approaches (comparators)
- Health States, transitions and outcomes (cost, utility and life years)
  - based on systematic evidence review, chart reviews, KOL input, TPP
- Time horizon (based on survival data)
- Model Type: decision tree, state transition Markov model, DES, other
- Analysis: Cohort simulation, Microsimulation
- Sensitivity analysis:
  - Deterministic: univariate / multivariate
  - Probabilistic: parametric / non-parametric (bootstrapping)
  - Structural
Given a certain level of uncertainty in model variables, a health economically justified price results in the majority of ICER values falling below the WTP* threshold.

**Illustrative**

Incremental Cost-Effectiveness (Cell Therapy X vs SOC)

ICER scatterplot generated through a Monte Carlo simulation
Software: TreeAge Pro 2014

* WTP: Willingness-to-Pay
When WTP thresholds are not clearly defined, HE analysis alone is of limited value

- WTP thresholds per QALY / LY / event avoided, are undefined in many countries
  - Including England when therapy does not meet TA selection criteria
- Furthermore there are variations in criteria applied to determine reimbursed price across countries and regions e.g.
  - Cost Effectiveness / Cost-utility / Cost-consequence
  - Budget Impact
  - Disease Burden / Unmet Need
  - Disease priority
    - e.g. paediatric vs geriatric
  - Political imperative
  - International price referencing

<table>
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<tr>
<th>Illustrative pricing criteria by market</th>
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<tr>
<td><strong>Country</strong></td>
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<tr>
<td>Clinical Effectiveness</td>
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<td>Cost Effectiveness</td>
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<td>Cost utility</td>
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<td>Cost consequence</td>
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<td>Budget impact</td>
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<td>Cross country price referencing</td>
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Pricing research with key market access stakeholders can help reduce uncertainty on WTP

**Pricing research can generate insights on:**

- Impact of clinical and HE arguments on willingness-to-pay and adopt
- Interrelationship between:
  - Price
  - Positioning (Tx algorithm, subpopulations)
  - Reimbursement restrictions
  - Supporting data

**It can also help:**

- Refine the economic models by generating:
  - Generic inputs
  - Country-specific adaptations
- Inform evidence generation activities and value story
Understanding national, regional and local market access processes for a given cell therapy is key in formulating an effective stakeholder engagement strategy.

- Market access stakeholders, evaluation methodologies and funding options can vary depending on:
  - **Regulatory status**
    - E.g. ATMP, non-medicinal cell therapies, cell therapies not intended for licensing, Early Access Schemes
  - **Size of target population**
  - **Setting of care**
    - E.g. centre of excellence; inpatient vs outpatient
  - **Unmet need, magnitude of incremental benefit claims and costs**
Routes to NHS adoption for cell therapies (England & Wales)
Semi-quantitative pricing research methodologies are useful for assessing WTP of EU market access stakeholders; fully quant approaches are feasible with US payers...

**Van Westendorp pricing sensitivity meter**

- **Point of indifference**
- **Point of marginal expensiveness**
- **Range of target prices for a breakthrough therapy**

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**Legend**
- Fully Justified
- Too Expensive
- Expensive
- Justified

**Axes**
- % of respondents
- Price (£ 000s)

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**CATAPULT Cell Therapy**
Pricing research methodology should be tailored to explore the interrelationship between WTP, reimbursement restrictions and supporting data requirements.

At higher prices, the risk of restrictions increases as well as the requirements for subpopulation analysis, long-term data generation and risk-sharing agreements.

**Requirements for favourable access**

- Subpopulation specific data demonstrating incremental benefit
  - Highly restricted use
- Robust plans for manufacturer sponsored real-world data generation / registries
- Risk-sharing agreements
- Discounts at local level
- Additional controls required e.g.
  - Prior-authorization
  - Specialist-center only
- Risk-sharing schemes can help address uncertainty; especially when long-term claims are made
- Registries/real-world data generation

**Price Illustration**

- **Expensive**
  - £50,000 or more
  - Delays in reimbursement decision; risk of no coverage
- **Marginal Expensive**
  - £48,000
  - Reimbursed for majority of eligible patients, limited restrictions
- **Inexpensive**
  - £32,000
  - Reimbursed according to label
- **Optimal**
- **Too Low**
- **Too High**
The insights generated from the pricing research can be used to develop price-volume trade off curves and identify the revenue-maximising price.

**Gabor Granger methodology**

- Derives a relationship between price and volume and identifies the revenue price.

Moving forward....

- HE analysis & pricing research repeated as additional evidence is generated.
- Prior to launch:
  - Assess impact of cross-country price-referencing
    - Identify optimal launch sequence
  - Develop risk-sharing contingency plans
  - Develop post-launch data generation plans