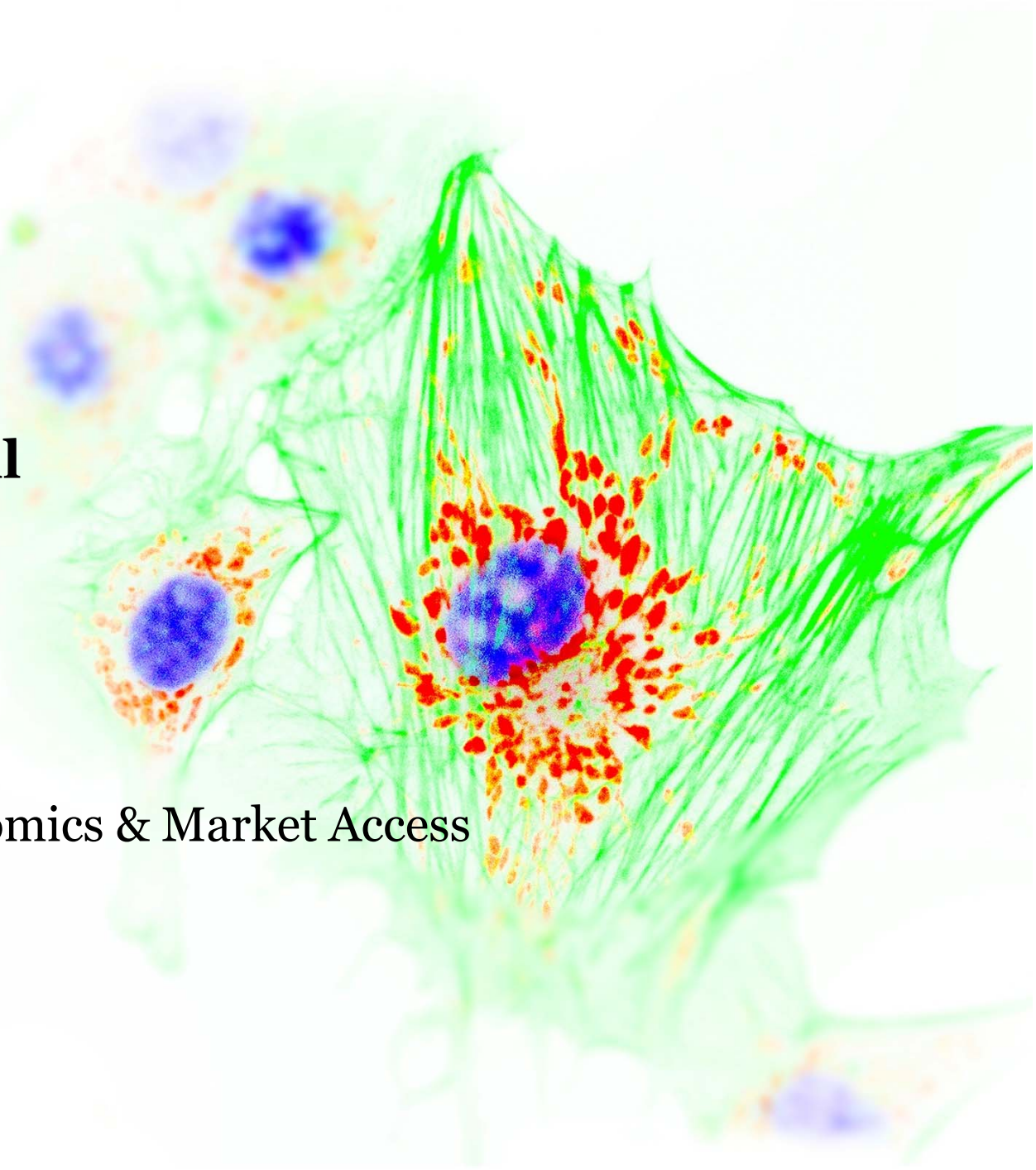
CATAPULT
Cell Therapy

Assessment of reimbursed price potential for a cell therapy in development



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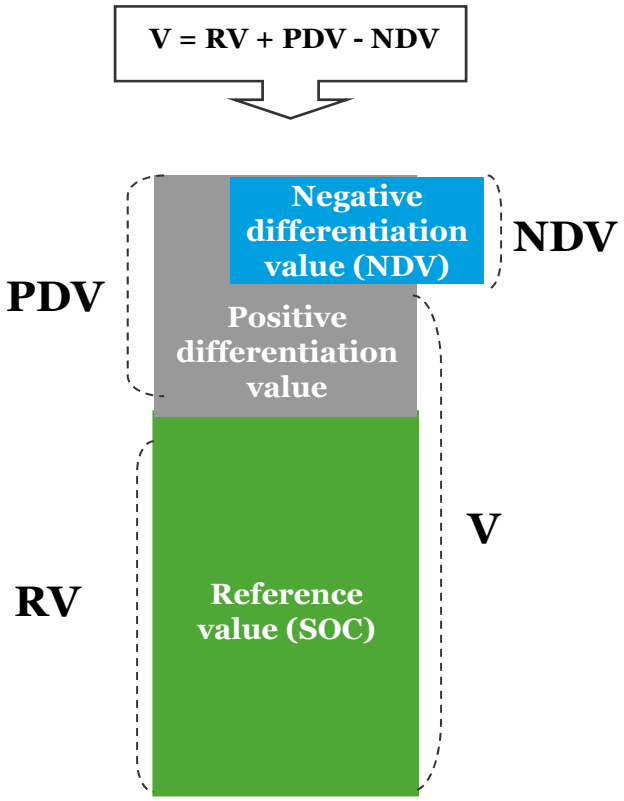
Catapult is an Innovate UK programme.

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

	Cost-based	Competitor-based	Value-based
What is it?	• Price is set by assumptions on costs, expected sales volumes and margins	• Price is driven by the pricing of competitor products	• Price is based upon therapeutic /economic value to the customer
Examples	• Cost-plus pricing • ROI based pricing (e.g. PPRS in UK)	• Penetration pricing • Reference group pricing	• Value-based pricing
Comments	• Becoming obsolete; no longer resonates with payers	• Enforced by many reimbursement systems for "**undifferentiated**" products	• Typical approach for **differentiated** products

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



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

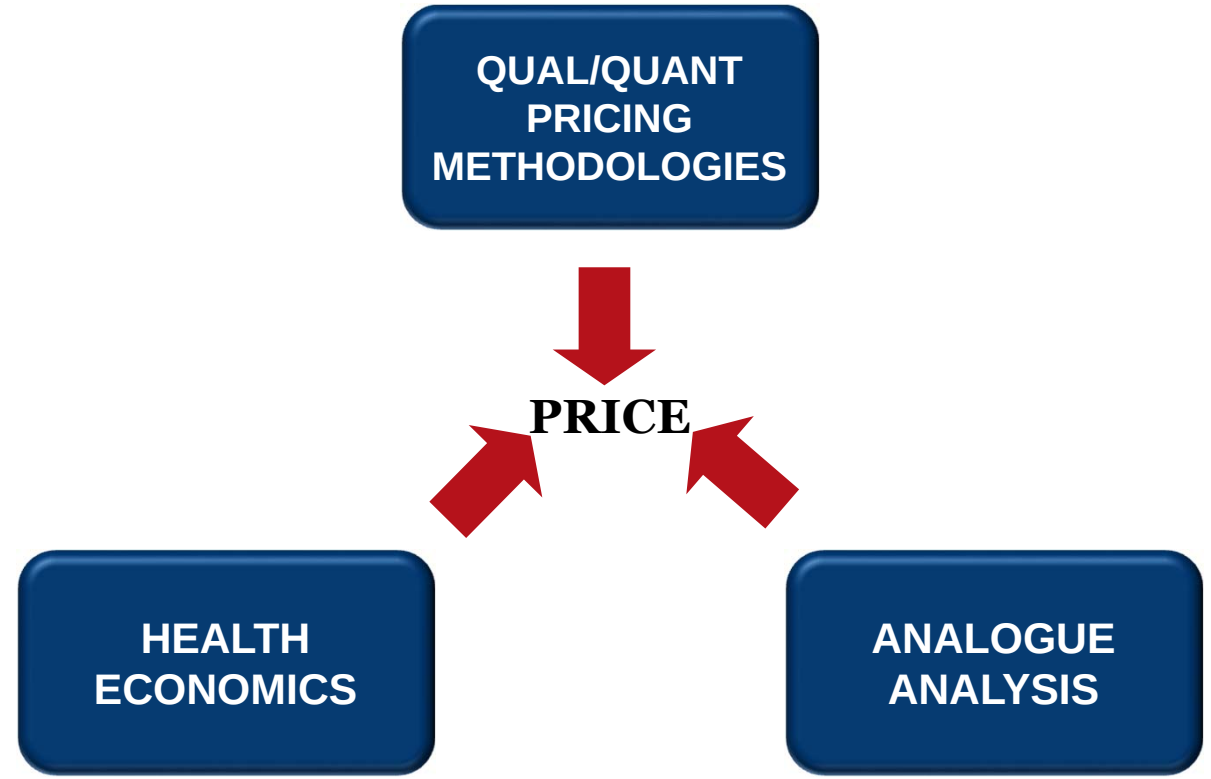
Value (V)

- 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



The role of health economics varies by geography

Our HE analysis starts with an NHS England perspective and a cost-utility framework

For therapies that meet NICE TA selection criteria, its ICER threshold is used to inform price potential

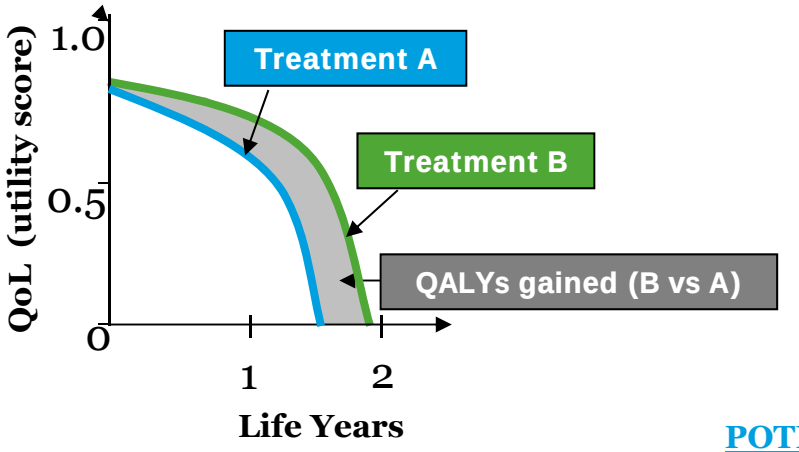
$$ICER = \frac{Cost\ B - Cost\ A}{QALY\ B - QALY\ A}$$

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

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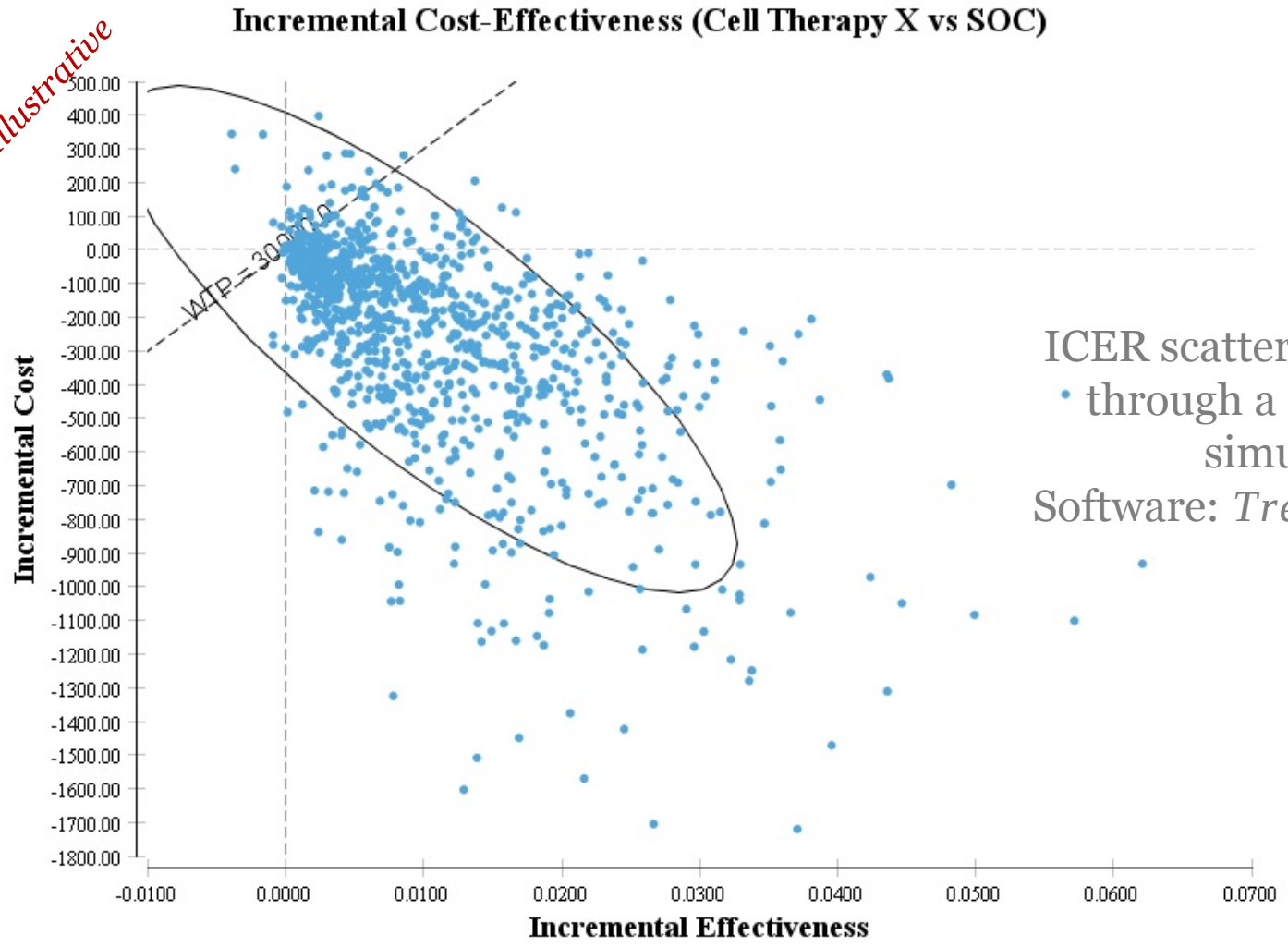
Given a certain level of uncertainty in model variables, a health economically justified price results in the majority of ICER values falling below the WTP* threshold



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

Illustrative pricing criteria by market			
Country	**A**	**B**	**C**
Clinical Effectiveness	✓	✓	✓
Cost Effectiveness	✗	✓	✗
Cost utility	✓	✗	✗
Cost consequence	✗	✗	✓
Budget impact	✓	✓	✓
Cross country price referencing	✗	✗	✓

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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

Typical EU Scope: **France** **Germany** **Italy** **Spain** **UK**

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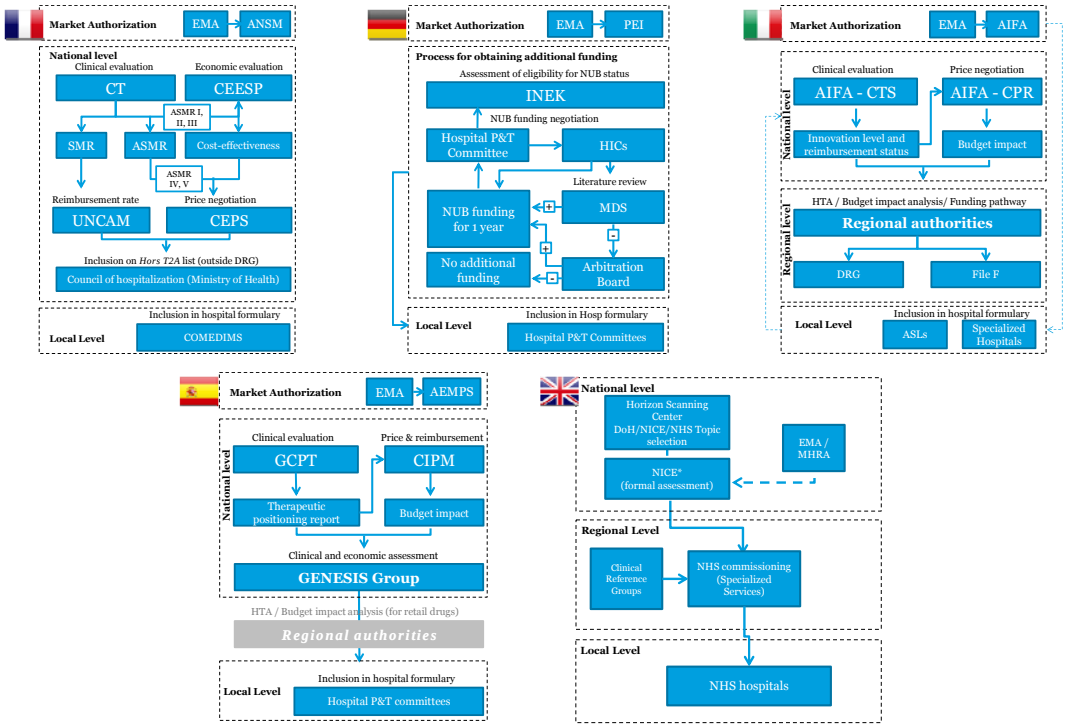
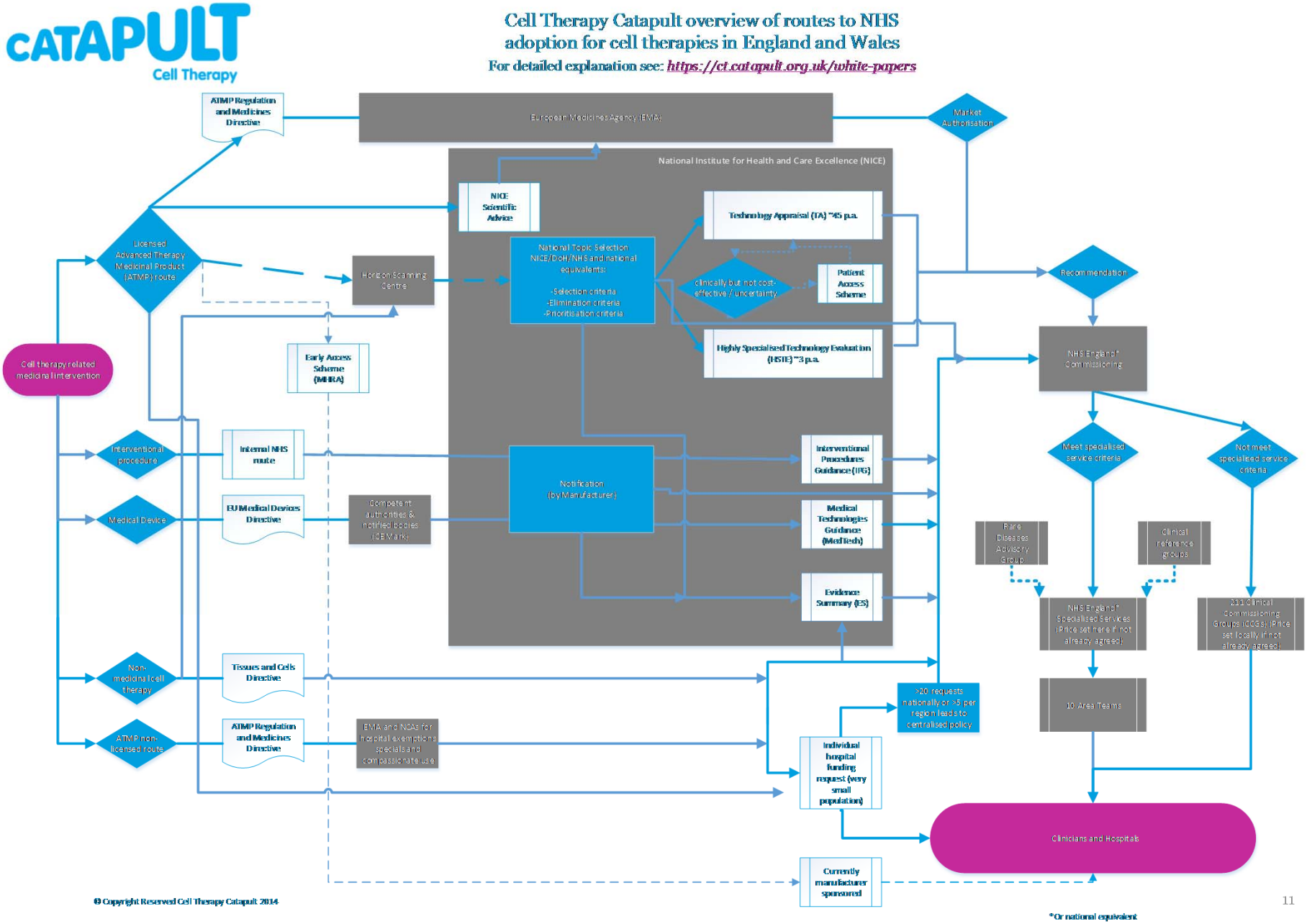
Understanding national, regional and local market access processes for a given cell therapy is key in formulating an effective stakeholder engagement strategy

- 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

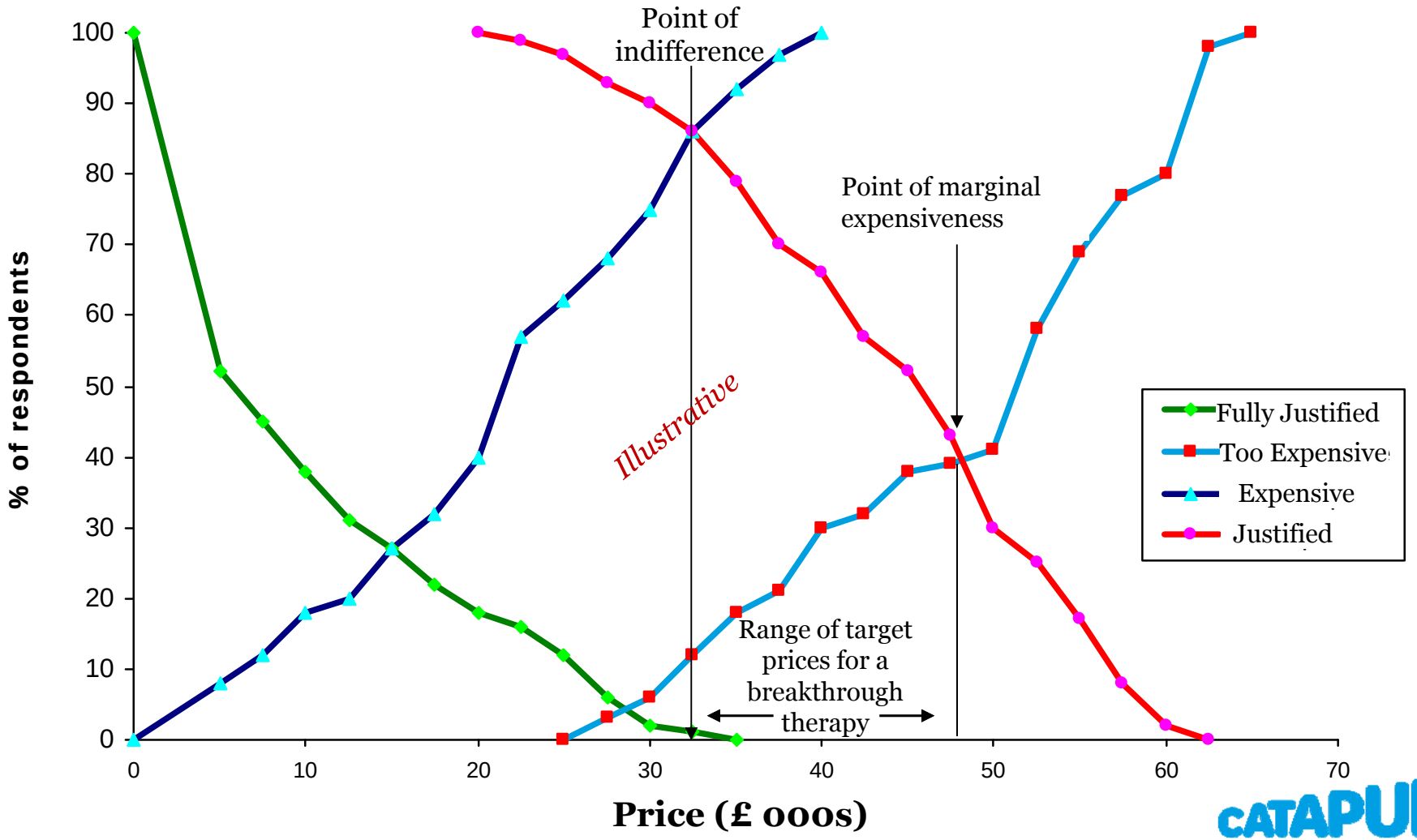


Routes to NHS adoption for cell therapies (England & Wales)



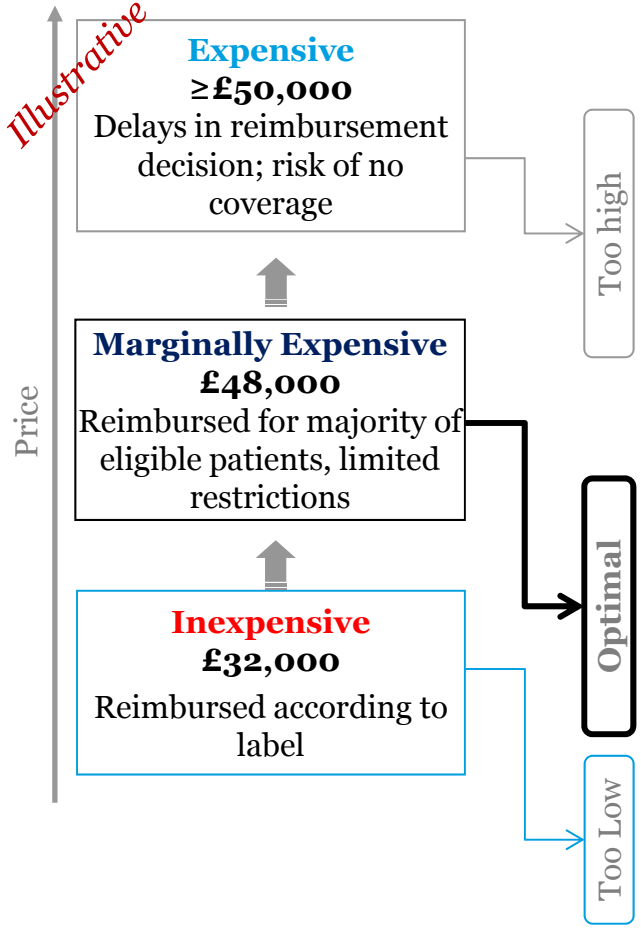
Semi-quantitative pricing research methodologies are useful for assessing WTP of EU market access stakeholders; fully quant approaches are feasible with US payers…

Van Westendorp pricing sensitivity meter



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

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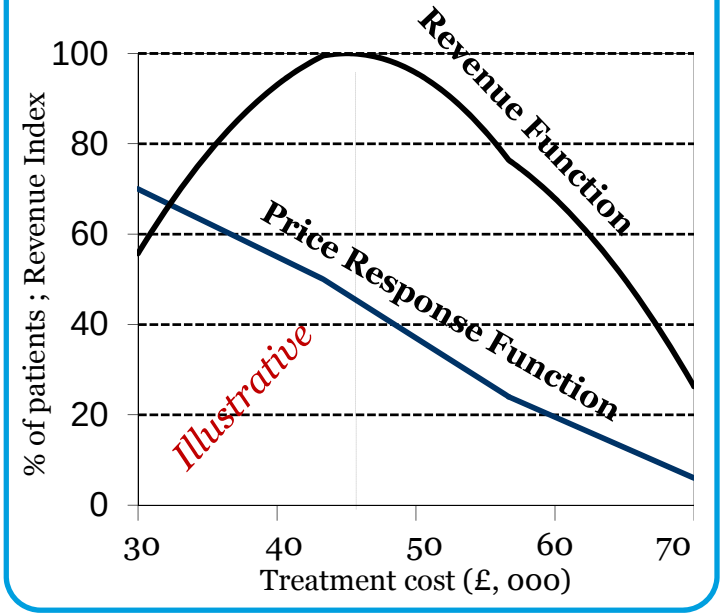
The insights generated from the pricing research can be used to develop price-volume trade off curves and identify the revenue-maximising price

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

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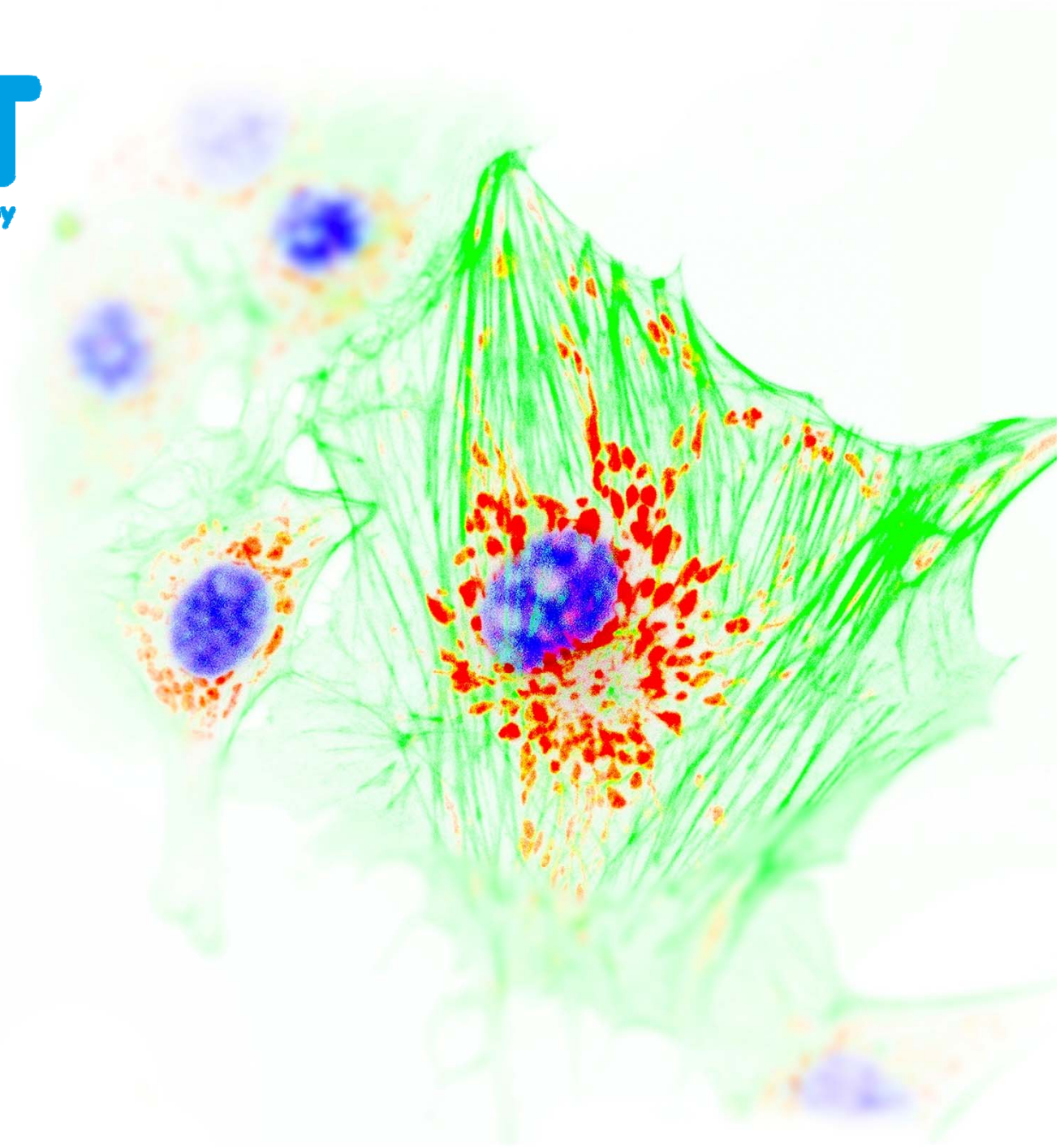

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Catapult is an Innovate UK programme.