

The Health Economics & Market Access (HE&MA) function of the (CGT) Cell and Gene Therapy Catapult

June 2019

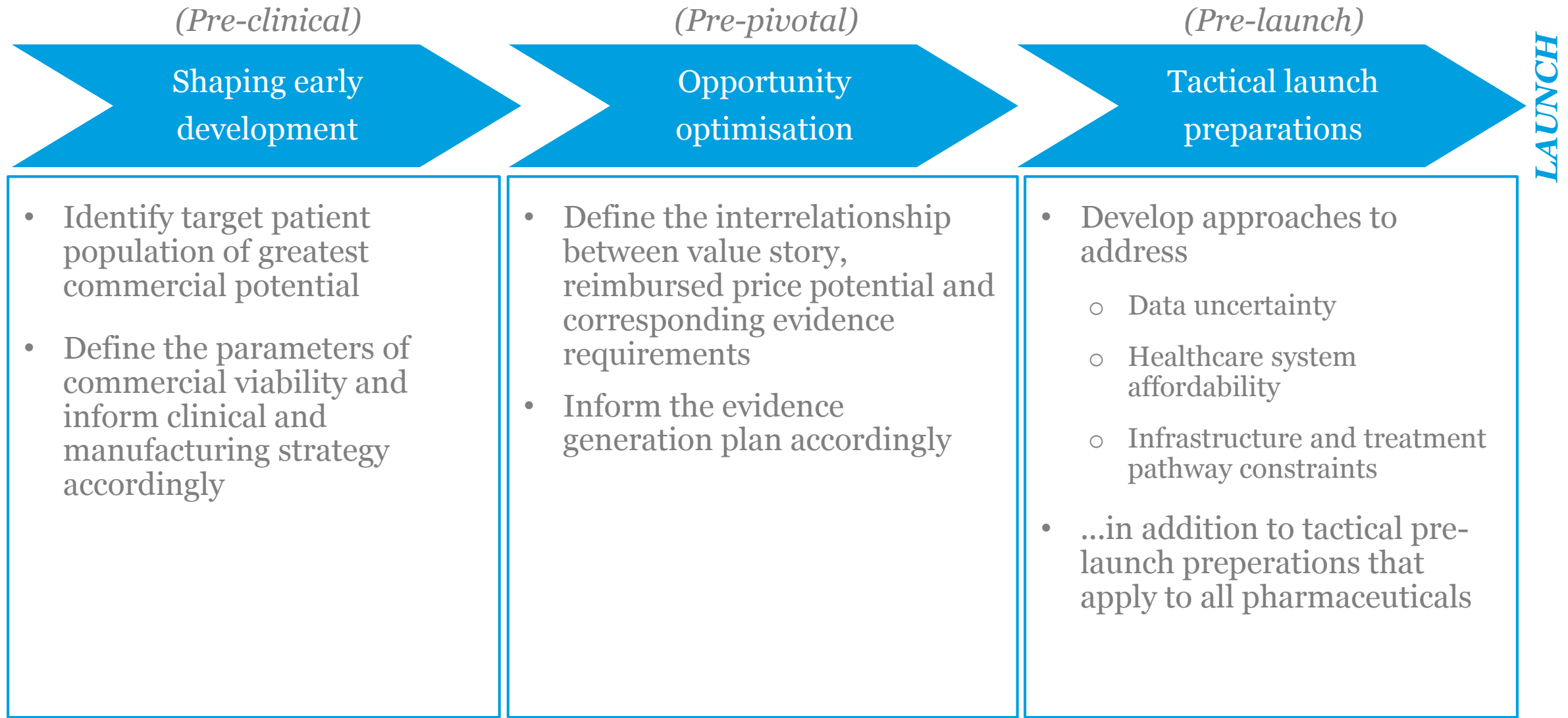


The CGT Catapult's Health Economics and Market Access (HE&MA) team provides strategic support tailored to the needs of ATMP developers

The team	<ul style="list-style-type: none">Seasoned HE&MA professionals with prior experience from senior roles with the industry and health technology assessment (HTA) bodies
The expertise	<ul style="list-style-type: none">Numerous projects across different cell and gene therapies, at different stages of development, across a variety of therapeutic areas, including:<ul style="list-style-type: none">Oncology, ophthalmic diseases, musculoskeletal disorders, solid organ transplantation immunosuppression, cardiovascular disease, respiratory disease, metabolic disease, liver disease, infectious disease, Parkinson's, haemophilia, and haemoglobinopathies

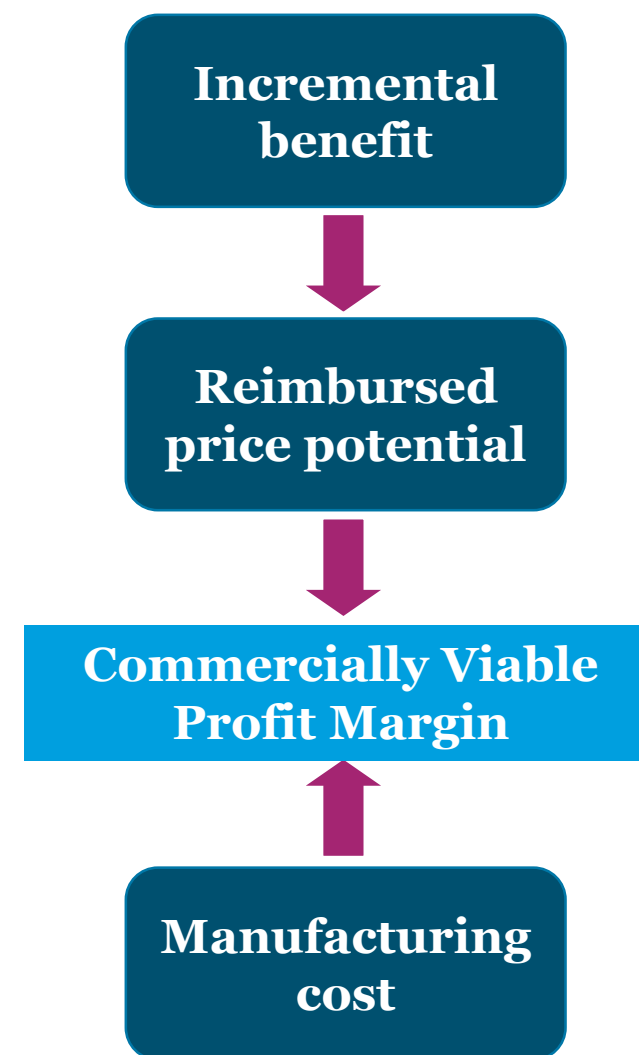
- We have refined traditional HE&MA frameworks to address the unique challenges of ATMPs
- We are working with payers across European and North American markets to develop and shape how they reimburse cell and gene therapies
 - We leverage these relationships in developing pricing and reimbursement strategies
- We have access to all the other expert teams within CGT Catapult and a track record in working seamlessly to deliver multifunctional projects

Our HE&MA offering differs according to the development stage of a therapy

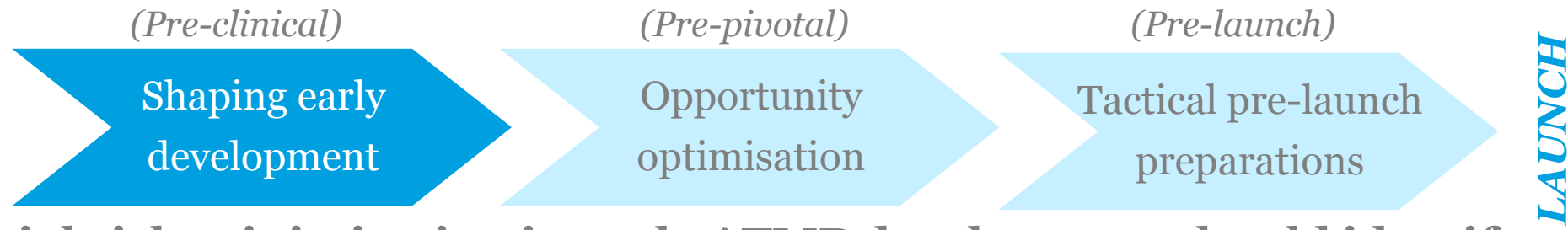


High manufacturing and delivery costs necessitate earlier consideration of reimbursement matters for ATMPs

- Majority of ATMPs are expensive to manufacture, administer and supply
- ATMPs therefore need to deliver a substantial incremental benefit (over existing therapies) in order to ensure a commercially viable profit margin
- Commercial risk mitigation focuses on:
 - Maximising incremental benefit
 - Minimising manufacturing costs
 - Reducing healthcare costs associated with the delivery of the novel therapy
- Accounting for reimbursement earlier and informing ATMP R&D strategy accordingly, is of priority...
 - ...and of even greater priority for those ATMPs qualifying for accelerated regulatory pathways



Failing to consider reimbursement matters prior to starting clinical development increases commercial risk



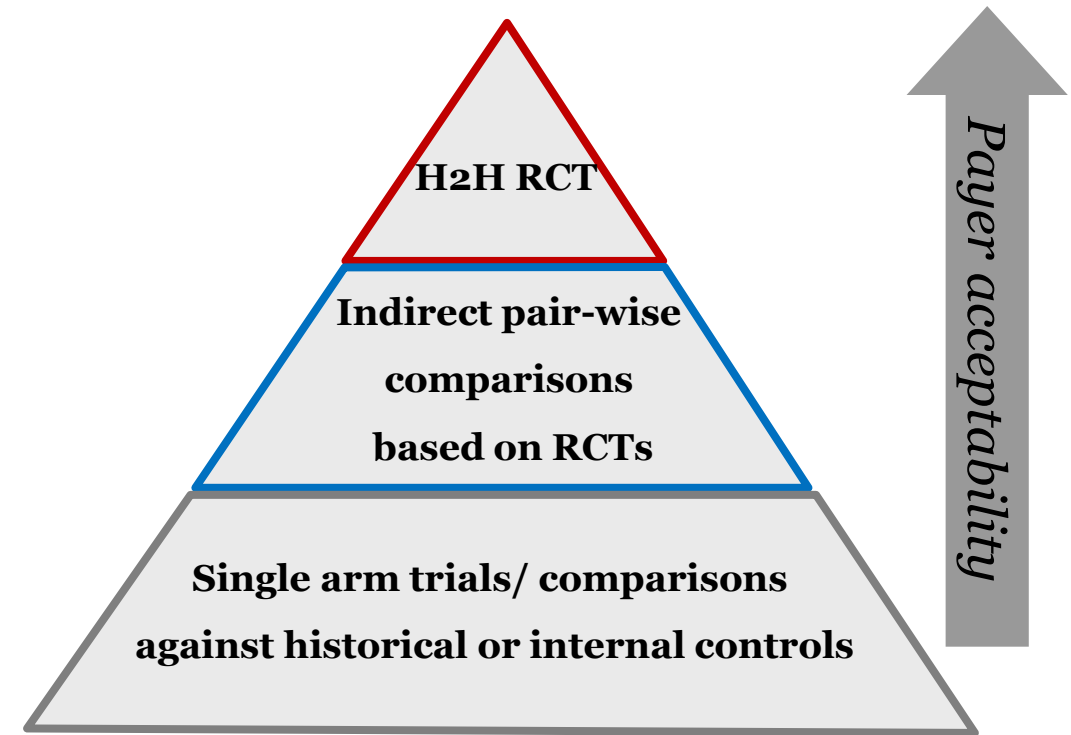
Commercial risk-minimisation in early ATMP development should identify:

- The headroom for innovation in the target indication/ therapeutic position to identify
 - The extent to which target indication can accommodate high-cost therapies
 - The target patient group with the greatest commercial potential (in the absence of clinical data)
- The value-maximising clinical, economic and humanistic outcomes
 - In order to inform the development of the early Target Product Profile (TPP) and evidence generation plan
- The interrelationship between therapy benefits and reimbursed price potential in order to define:
 - Product performance and manufacturing cost thresholds for commercial viability
 - 'Go'/'no-go' criteria for stage-gate decision-making across consecutive stages of development
- Inform clinical and manufacturing strategy accordingly

Clinical, regulatory and commercial considerations often necessitate a clinical development programme for ATMPs that payers find challenging

Common data challenges for ATMPs:

- Potential for a cure but lack of long-term data at launch
- Weak comparative effectiveness data vs. the standard of care (SOC) due to one or more of the following:
 - Head-to head (H2H) comparative data against the standard of care is not available
 - Randomised controlled trials (RCTs) not feasible, which limits prospect for indirect comparisons
 - Meaningful comparative data from single arm trials can not be generated due to e.g. limitations with the historical control data, the natural history of disease is not well known, or the patient population is heterogeneous
 - Small trials limit statistical significance of outcomes measured
 - Measuring only surrogate outcomes rather than hard clinical outcomes (risk for overestimation of benefit as per: *NICE Regenerative Medicine Study, 2016*)
 - No comparable treatment or outcome measures are available



Given the evidence generation challenges with ATMPs, it is important to engage with market access stakeholders early



There are different options for engaging with key market access stakeholders:

- Centralised at national level: Parallel Consultation with EMA and European HTA bodies
- Decentralised at national level: Individual HTA bodies in different countries
- Decentralised at national, regional and local level (traditional payer research)

The objective of engaging with market access stakeholders is to identify the evidence that optimises reimbursement potential



Optimising the evidence-generation plan

- Where no comparable treatment or outcome measures are available, manufacturers must work with KOLs, regulators and HTA bodies to agree appropriate measures
- Where head-to-head trials are not feasible, agree alternatives to generating comparative data
- Where only single-arm trials are feasible, agree how historical controls or baseline comparisons may be leveraged
- Where surrogate endpoints will be used, agree selection and validation
- Where long-term claims will be made, explore:
 - The type of modelled data that could be used to bridge the evidence gap
 - Acceptable approaches for dealing with data uncertainty at the time of launch

Developers need to establish first which value story secures commercial viability; HTA/payer advice informs the corresponding evidence requirements

Identify the commercially viable target price and population

Develop a value story that supports the target price in the target population

Create an evidence generation plan that provides the best possible support for the value story

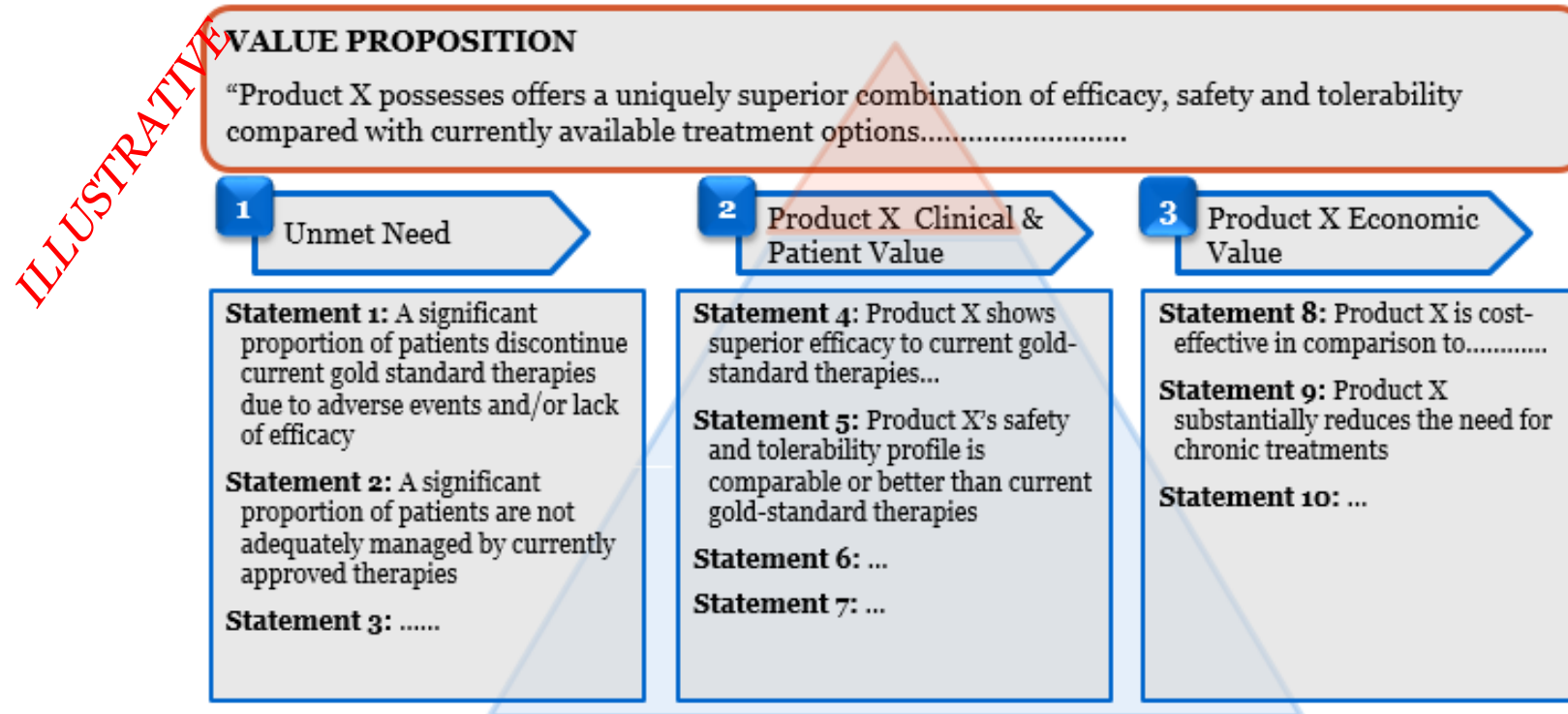
HTA/Payer Consultation

Contextualise learnings and revisit evidence generation plan

In order to engage constructively with HTA bodies/ payers the following activities should be conducted sequentially:

- Understand the value drivers for a given therapy and how these can help support a commercially viable price and volume opportunity; this forms the basis for the development of the target value story
- Develop the *briefing document*, for the consultation addressing:
 - The unmet need in the target therapy area
 - The product's target value story and how it addresses the unmet need
 - The evidence generation plan, and how this supports the target value story
 - The areas where evidence gaps may exist, and formulate questions for HTA bodies and propose potential solutions
- Explore the HTA bodies' perspective on how to best substantiate the target value story, and adjust the evidence generation plan accordingly

We employ secondary and primary research and health economic modelling to frame the value story and corresponding evidence requirements that support commercial viability

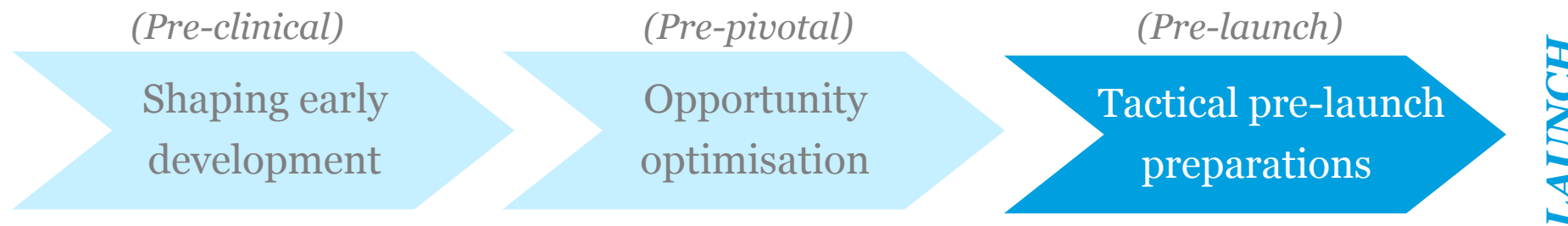


- The value story is typically structured in three domains, with an overarching paragraph that summarises the value proposition
 - **Value proposition:** Summary of incremental value of novel therapy over existing standard of care
 - **Value statements:**
 - Unmet need: it should align with the incremental benefits of the novel therapy
 - Clinical and economic value statements: describe therapy's incremental benefit in clinical and economic terms
 - Value statements should be supported by the proposed clinical and economic evidence to be generated

The outputs from the activities described so far inform the development of the target product profile (TPP) and evidence generation plan

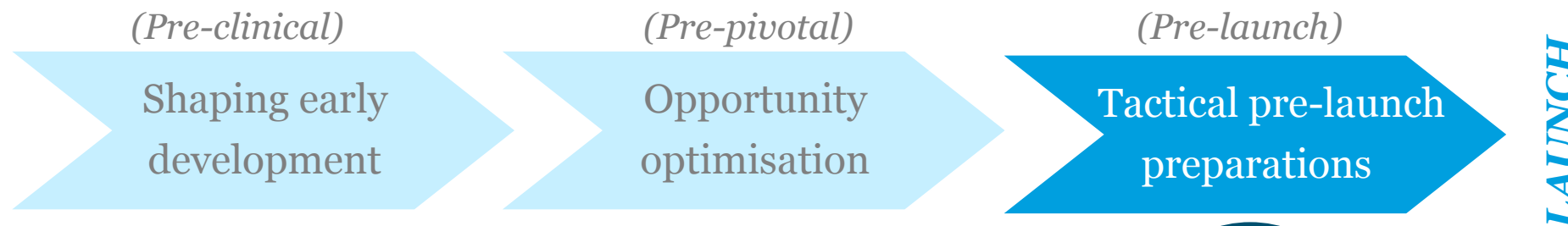
Activity	Output	Objective
Headroom for innovation	Validate that target indication/ therapeutic position can accommodate a high cost therapy	Ensure commercial viability
Pricing research and sensitivity analysis	Identify key clinical and economic drivers of product value to incorporate into TPP	
	Define product performance and manufacturing cost thresholds for commercial viability	
Clinical feasibility analysis	Understand feasibility of undertaking clinical development in target indication / therapeutic position	Ensure feasibility of running clinical trial
Engage with payers (and regulators)	Ensure agreement on therapeutic position with regulators Ensure evidence generation plan in line with expectations of regulators and key market access stakeholders	Ensure appropriateness of evidence generation plan

In preparation for launch, we focus on maximising the commercial potential in terms of price, access and revenue

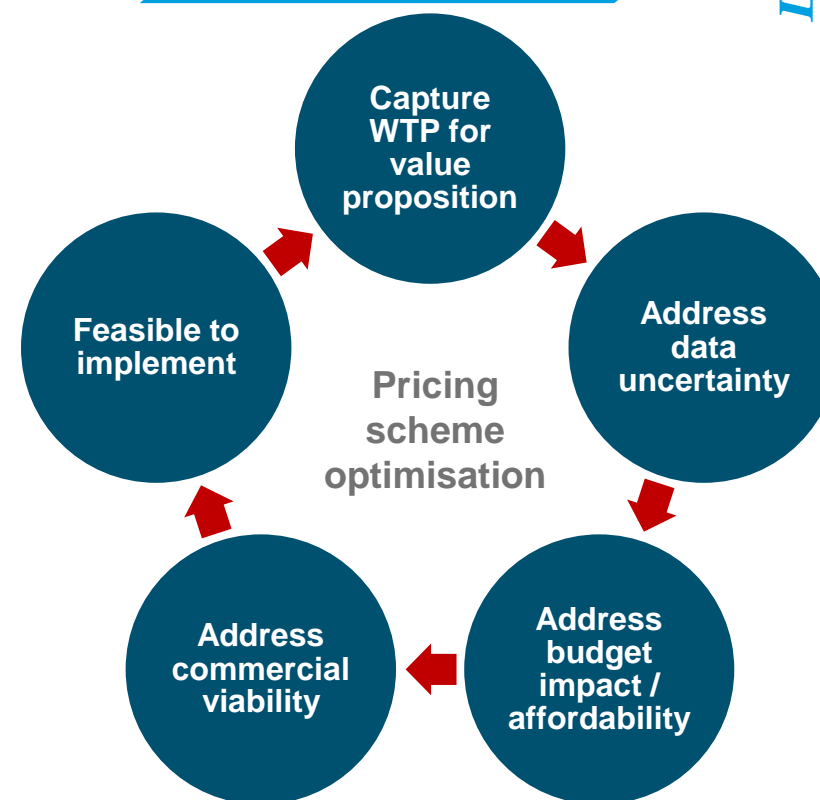


- Address clinical data gaps through data modelling where appropriate
- Finalise the health economic models
- Populate the value dossier including the value story and supporting clinical and economic evidence (customised to individual market requirements) in preparation for submission
- Identify the target price for each launch market and geographical launch sequence
- Develop strategies for maximising reimbursement and adoption potential
 - Innovative pricing schemes/Managed Entry Agreements (MEAs)
 - Post-launch evidence generation plans
- Detail the readiness of the healthcare delivery system (e.g. available infrastructure and treatment pathway) to assess potential constraints and need for process re-engineering and investment

Innovative pricing mechanisms can help address data uncertainty and affordability concerns for payers



- We help manufactures identify the optimal pricing scheme on the basis of the following considerations:
 - Scheme reflective of therapy value and willingness-to-pay (WTP)
 - Minimises implications of data uncertainty
 - Enables payer affordability
 - It is commercially viable for the manufacturer
 - It is feasible to implement within a given healthcare system without creating significant administrative burden



Methodologies and case studies



Understanding the challenges and opportunities presented by the existing pricing and reimbursement (P&R) frameworks is key

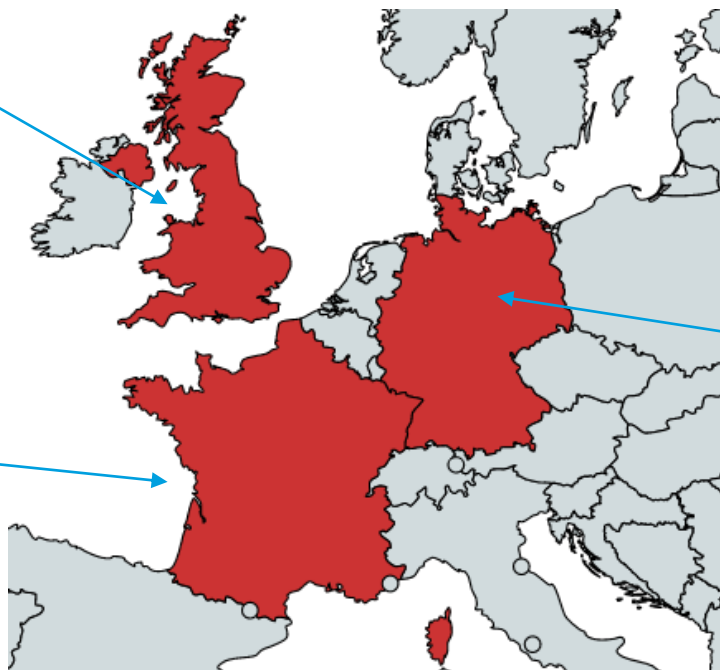
Main levers used in P&R processes in select European markets

UK

- Cost-utility analysis (CUA)
- Budget impact analysis (BIA)

France

- ASMR 1-3 (moderate to major improvement):
 - International price referencing (EU4)
 - CUA
- ASMR 4-5 (minor or no improvement):
 - Domestic comparator price
- Price-volume agreements



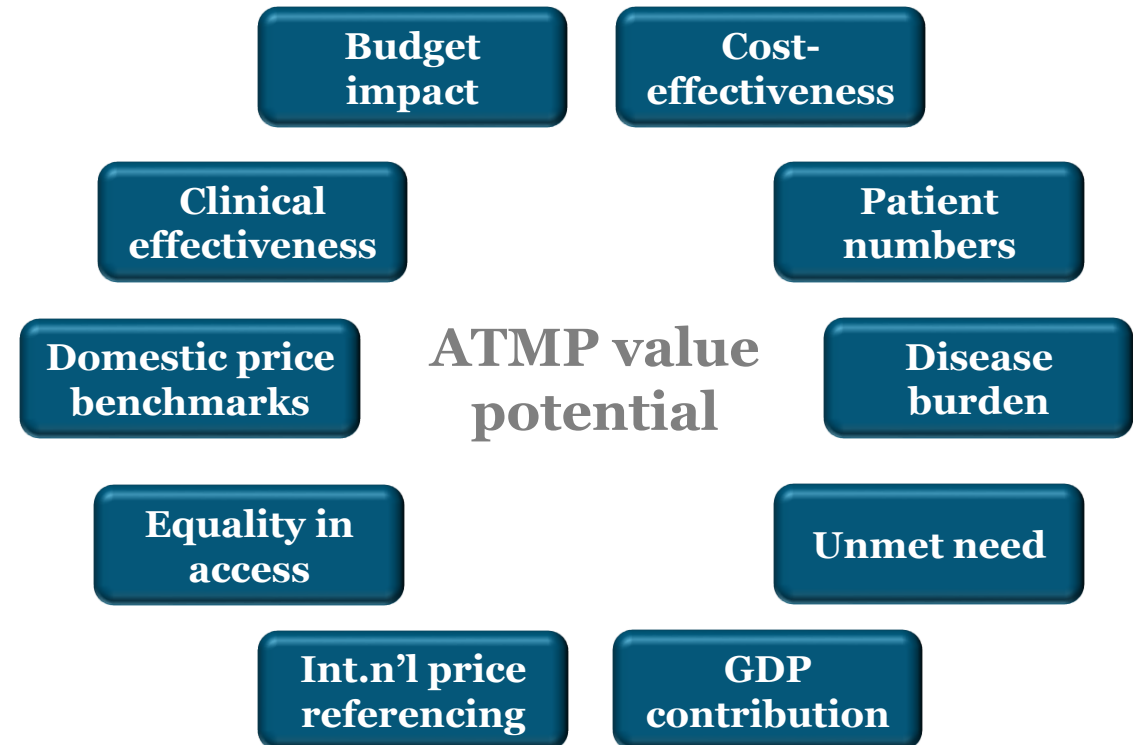
Germany

- With added benefit:
 - Price premium over domestic comparator price
 - Budget impact
- If price negotiation fails then arbitration:*
 - International price referencing (EU15)
 - Potentially followed by:*
 - Efficiency frontier analysis
- No added benefit:
 - Domestic comparator price

The CGT Catapult's HE&MA team leverages more than 30 years' experience in international pharmaceutical pricing and reimbursement

ATMPs present unique value propositions that challenge traditional pricing and reimbursement frameworks

- The frameworks used to assess conventional pharmaceuticals are well suited for valuing smaller, incremental improvements in health benefit
- However, using these frameworks to assess potentially game-changing therapies like ATMPs is more challenging, e.g. how to value:
 - One-off therapy with long-term benefits
 - Potential lifetime cure....while managing uncertainty



We leverage multiple methodological frameworks in exploring and optimising the value potential of ATMPs across different countries

- **Health economics:**

- Cost-effectiveness and budget impact analyses
- Sensitivity analyses, extrapolation and regression analyses
- Data uncertainty management

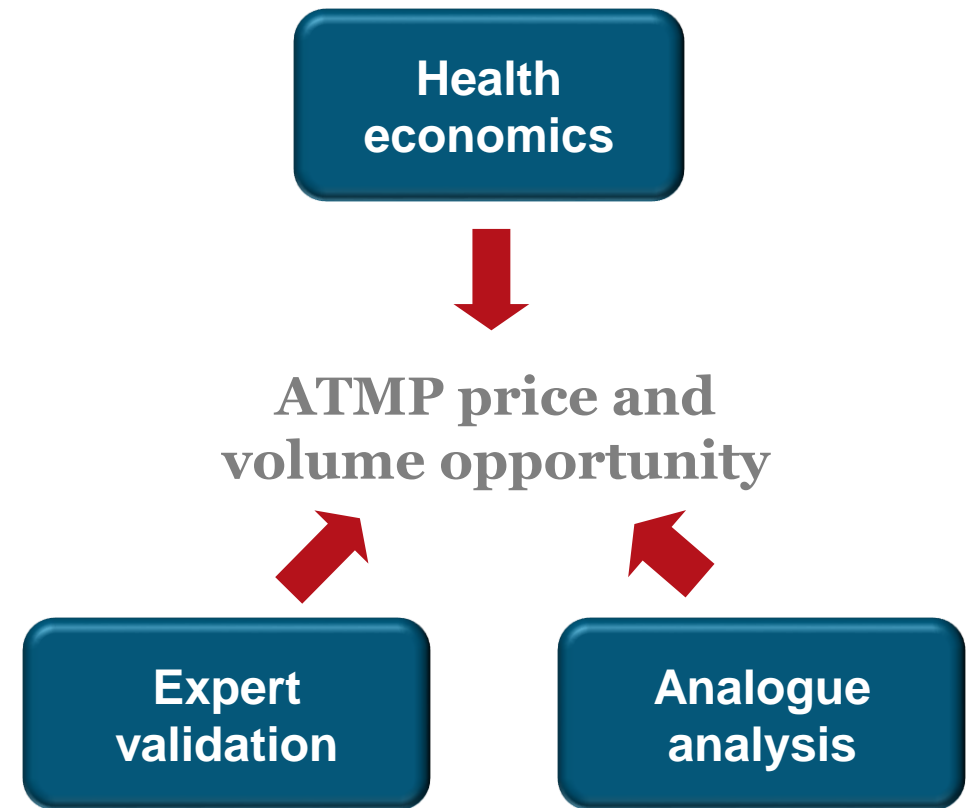
- **Analogue analyses:**

- Secondary research of relevant HTA and commissioning decisions to elicit willingness to pay and adopt

- **Expert validation:**

Interviews with payer and clinician experts to:

- Determine willingness to pay and affordability
 - Via qualitative/ quantitative pricing methodologies
- How to maximise adoption potential through optimisation of value proposition, evidence generation plan, and innovative pricing and reimbursement schemes
- Detail the need for NHS process re-engineering and clinical infrastructure to facilitate adoption



Health technology assessments (HTAs) in most countries use some form of cost-effectiveness analysis to determine value for money

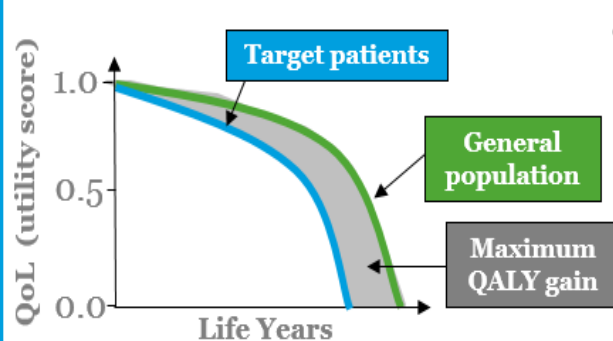


- **Cost-effectiveness analysis (CEA)** framework compares the incremental cost to the incremental health benefit of different therapies, i.e. answering the question *how much better is the new therapy in terms of health benefit, and how much more do we have to pay?*

$$\frac{\text{Lifetime cost of new therapy} - \text{Lifetime cost of standard of care}}{\text{Lifetime benefit of new therapy} - \text{Lifetime benefit of standard of care}} = \text{Incremental cost-effectiveness ratio (ICER)}$$

...however, how benefits are measured differs between territories

- **Cost-utility analysis (CUA):** Is a form of CEA where the health benefit is measured as Quality-Adjusted Life Years (QALYs)



- QALYs reflect the life expectancy (life years) and quality of life (utility, ranging from 0-1) experienced during that period

- **Other CEAs:** health benefit can be measured in a number of different ways, e.g.
 - Life years gained
 - Events avoided
 - Other relevant clinical outcomes

The CUA framework forms the basis for two analytical approaches use in shaping the early development of ATMPs



We use the CUA framework to

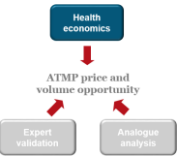
Prioritise between target patient groups (where several therapeutic targets exist)

- Identify the indication with the greatest commercial opportunity (in terms of maximum revenue potential of “cure”) as per:
 - *The headroom for innovation* (maximum lifetime value of displacing current standard of care and maximising patients’ potential health benefit)
 - Maximum patient numbers (i.e. 100% market share of target population)

Define parameters for commercial viability

- Identify product performance and manufacturing cost thresholds for commercial viability
 - Define ‘go’/’no-go’ decision-making criteria for the R&D stage-gates
 - Through sensitivity analysis identify key value drivers to inform the early stage Target Product Profile (TPP) and evidence generation plan

Case study 1: Deciding on which patient group to target is one of the most important strategic decisions in pre-clinical development

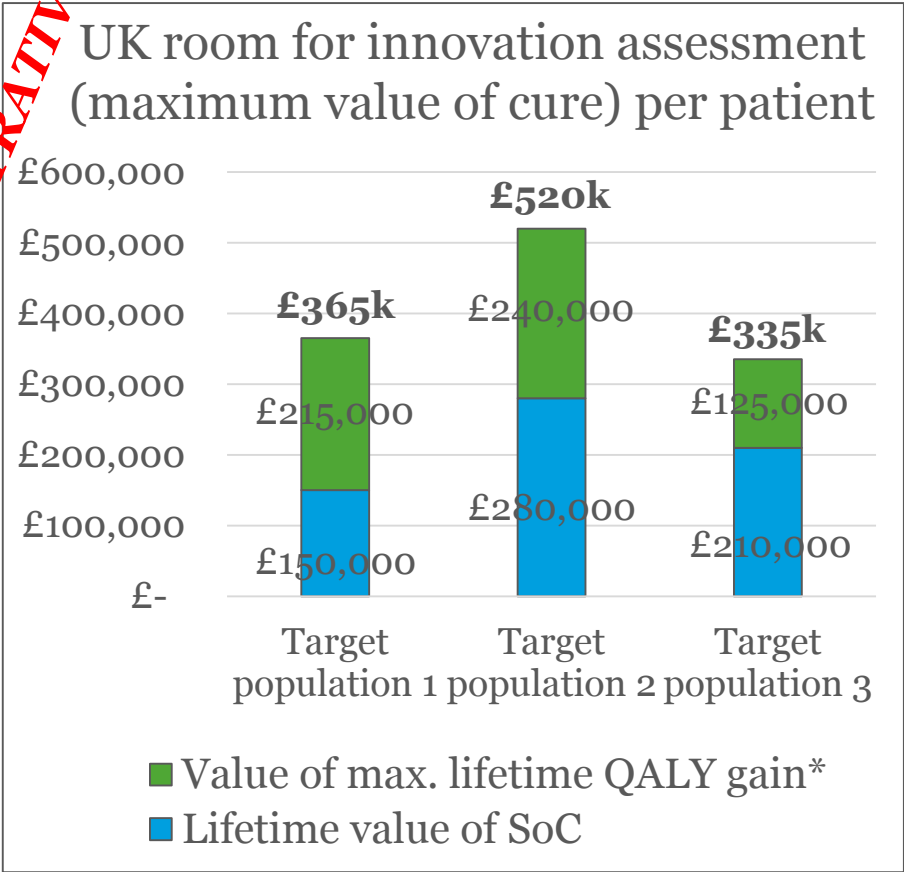


- The choice of indication or therapeutic position needs to be driven by both clinical and commercial considerations
- We enable developers compare the commercial opportunities presented by different target patient groups through:
 - A. **The headroom for innovation analysis:** estimating the maximum price potential per patient treated (i.e. “the value of cure”), using the cost-utility analysis (CUA) framework
 - B. **The size of the target population:** the maximum volume opportunity (100% market share)
- A and B are subsequently used to determine which indication presents the greatest commercial opportunity in terms of the maximum revenue potential

The outputs from our analyses allow developers to identify:

- The target population that should be prioritised in the planning of the development programme
- The target population that is likely to be the best candidate for subsequent indication extension

Case study 1: We identify the target population with the greatest commercial potential by estimating the maximum revenue potential



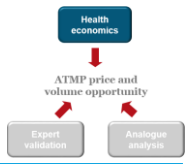
* Using WTP/QALY as per NICE guidelines

- The **headroom for innovation** analysis allows us to discern how the lifetime value of the SoC and the maximum potential health improvements differ between the three target groups
- We estimate the **maximum revenue potential** for the three target populations by multiplying the maximum value of cure per patient with the maximum number of patients (assuming 100% market share)

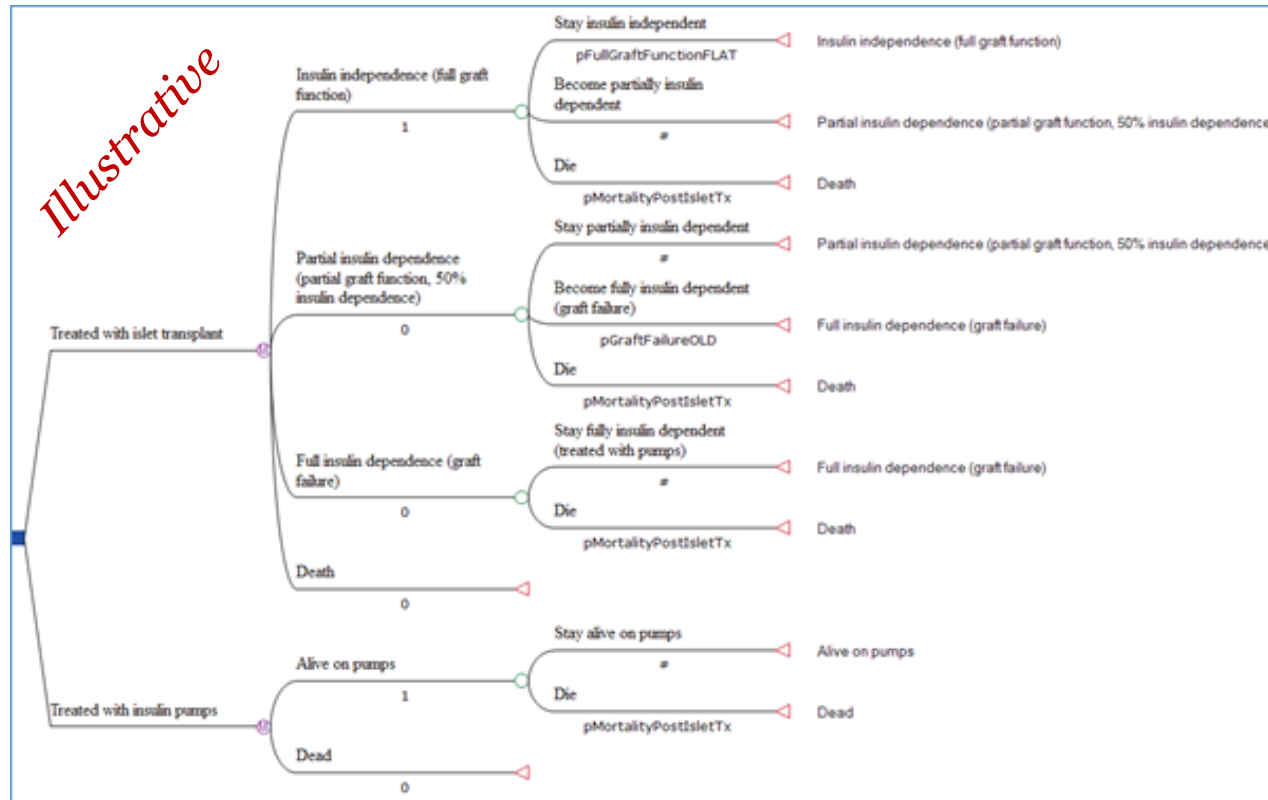
Max. value of cure/ patient	×	Max. number of patients	=	Max. revenue potential
£365k		1,200		£438 million
£520k		1,800		£936 million
£335k		1,100		£368 million

We use country-specific adaptations to extend this analysis to other countries that use the CUA framework to inform price potential

Once the target population is identified, we establish the interrelationship between levels of efficacy and commercial viability through more detailed health economic modelling



- Model types employed: Decision tree, state transition Markov model, discrete event simulation, transmission model
- Analysis types: Cohort simulation, microsimulation

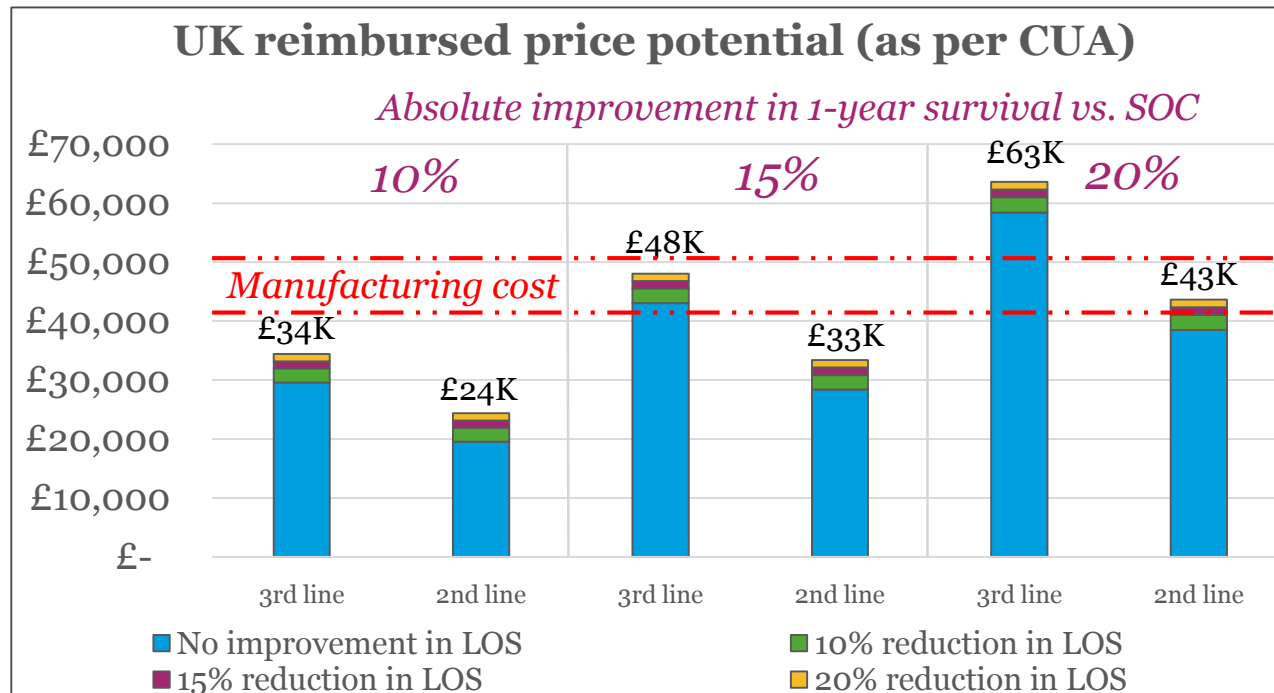


- Health states & transitions: as per disease trajectory
- The time horizon of the analysis is typically lifetime (up to 100 yearly cycles / discounted)
- Each health state is assigned cycle-specific costs and outcomes (e.g. QoL in CUA)
- Sensitivity analyses can address uncertainty
 - Deterministic: univariate/ multivariate
 - Probabilistic: parametric/ non-parametric (bootstrapping)
 - Structural

Case study 2: We assess the impact of a range of efficacy scenarios and therapeutic positions on UK price potential and commercial viability



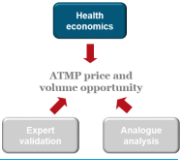
- We tested different scenarios of therapeutic positions in an acute condition (2nd and 3rd line) and relevant outcomes
- Survival and therapeutic positioning had the biggest impact on the reimbursed price potential



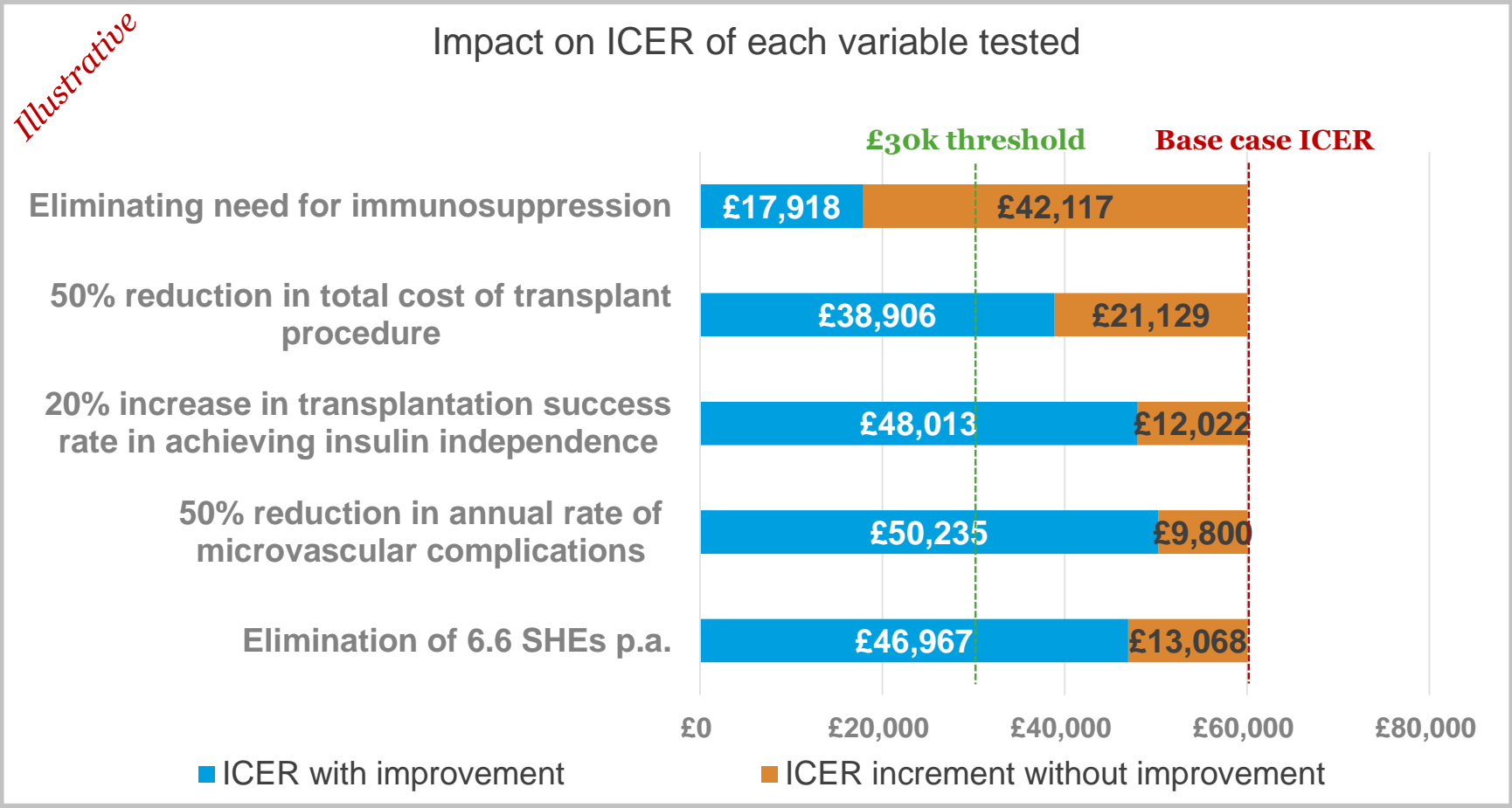
This formed the basis for the value story and the planning of data generation activities:

- Early Target Product Profile (TPP) development
- Clinical development specifications
 - Target therapeutic position
 - Trial inclusion criteria
 - Outcome measures for value maximisation
- Product performance and manufacturing cost thresholds for commercial viability; criteria for 'go'/'no-go' Stagegate decisions
- Financial forecasts providing confidence to management and investors

Case study 3: Sensitivity analysis identifies key value drivers and focus areas for R&D and evidence generation to strengthen value proposition



One-way sensitivity analysis for an islet transplantation therapy in type 1 diabetes



ICER: Incremental cost-effectiveness ratio; SHEs: Severe hypoglycaemic events

A crucial part of assessing the price potential is identifying the willingness to pay (WTP) for improvements in health benefit



Cost-utility analysis (CUA) countries:

- The WTP/QALY improvement is only explicitly stated in a small minority of countries where the CUA framework is used
 - E.g. the National Institute for Health and Care Excellence (NICE) in England has explicitly defined WTP/QALY values depending on the degree of data uncertainty, how effectively QoL has been captured, how innovative the therapy is, whether it is an end of life therapy, the size of the target population and the number of QALYs gained
- In most countries using CUA, the WTP/QALY is not explicitly stated, however, recent HTA and pricing decisions can give an indication

Countries using other CEAs:

- In countries that do not use the CUA, it is necessary to establish
 1. The most relevant measure of health improvement to use in the CEA, and
 2. The WTP per unit improvement in that measure
- The WTP in these cases are rarely explicit, and it is necessary to employ both secondary and primary research to elicit what an appropriate price premium is for a given improvement in health

To assess WTP when not explicitly stated in the public domain, we use analogue analyses and primary research with key market access stakeholders (expert validation)

Engagement with key market access stakeholders is used to provide multiple insights



Explore, validate, inform:

- The WTP for improvements in health benefit as identified through analogue analysis
- The price potential identified through health economic modelling
- The budget impact and its implications on adoption
- Strategies that mitigate risk, maximise value proposition and adoption through:
 - Optimisation of value story and evidence generation plan
 - Minimisation of consequences of data uncertainty and facilitating affordability through innovative pricing schemes
 - Accounting and planning for clinical infrastructure requirements and NHS process reengineering (where relevant)

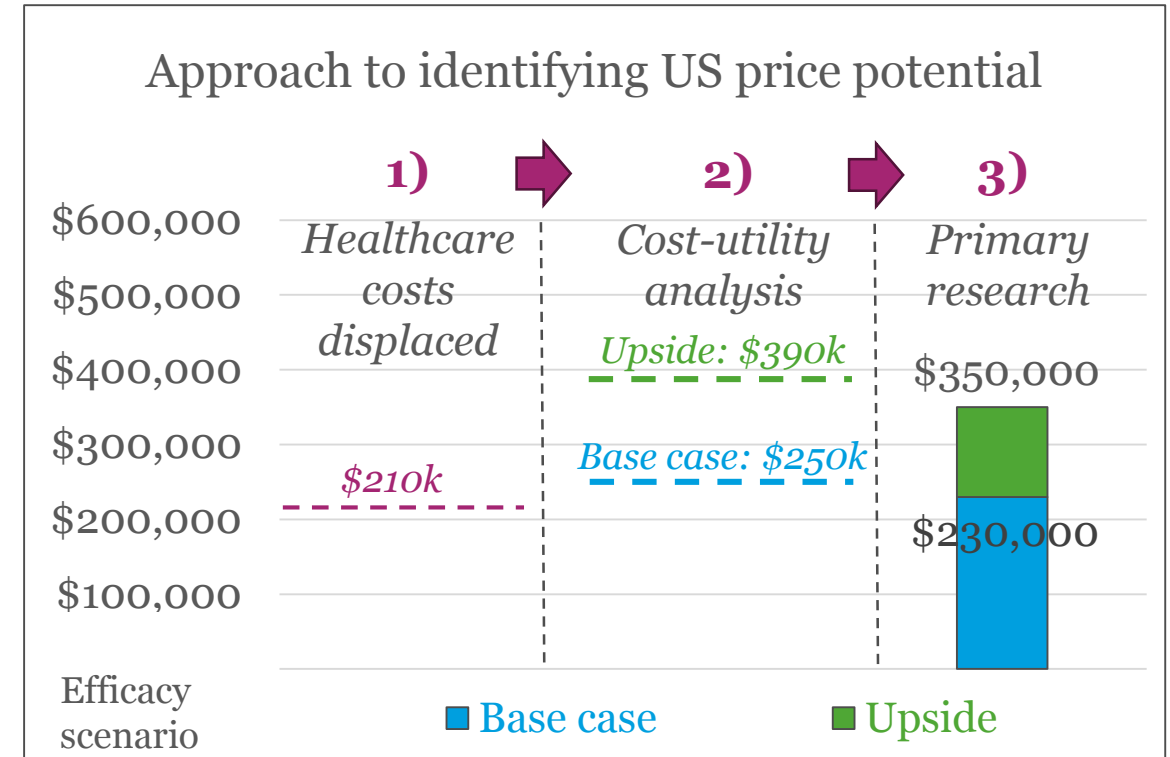
Case study 4: In markets where CUA plays a lesser role in HTAs, we identify price potential through a triangulation of pricing frameworks



- We used the triangulation approach to assess the US price potential for a novel ATMP using two efficacy scenarios (base case and upside)

- Triangulation of pricing frameworks:

- 1) Secondary research into relevant pricing benchmarks; identification of healthcare costs associated with the SOC and anticipated to be displaced by the new therapy
- 2) CUA to explore potential for price premium over displaced costs
 - WTP thresholds were informed by the Institute for Clinical and Economic Review's methods
- 3) Primary research with key US market access stakeholders to validate and inform the above



- We also explored formulary inclusion considerations and how they vary between public and private insurers, value optimization, risk-mitigation and adoption maximization strategies

Budget impact (BI) assessments are commonly used by payers to quantify the aggregate impact of introducing a novel therapy



- Key drivers:
 - Change in costs per patient from displacing existing therapies (usually healthcare budget only)
 - Number of patients treated
 - Time horizon (≤ 5 years)
- England operates a budget impact ‘test’, which assesses whether a new therapy’s aggregate additional cost to the healthcare budget exceeds the threshold value of £20 million per year
 - If the £20 million threshold level is exceeded, additional commercial negotiations and potential restrictions apply

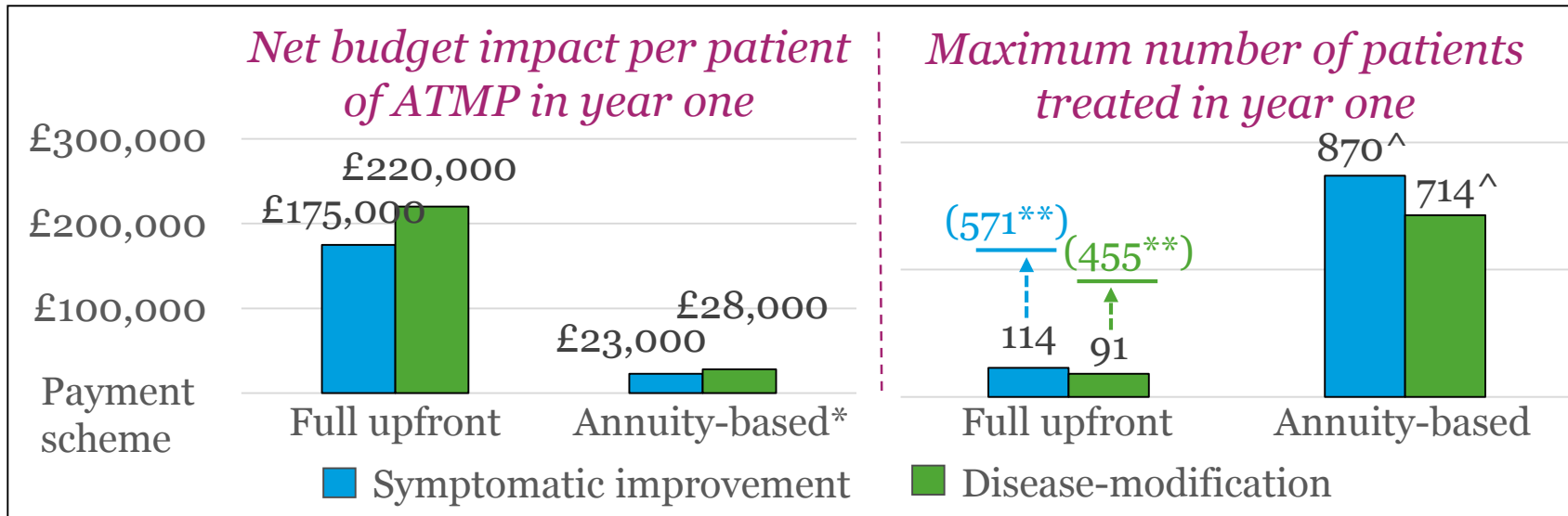
BUDGET IMPACT							
Total Population of England	50,542,505	<i>Illustrative exemplar of a novel budget neutral therapy</i>					
Target population p.a.	1,000						
SOC price per patient	£5,000						
New Therapy price per patient	£6,000						
Probability of rehospitalisation with SOC	2.00%						
Probability of rehospitalisation with New Therapy	1.00%						
Cost per rehospitalisation	£20,000						
		Year 0	Year 1	Year 2	Year 3	Year 4	Year 5
Market share of New Therapy		0%	20%	40%	60%	80%	100%
SOC Costs		£5,000,000	£4,000,000	£3,000,000	£2,000,000	£1,000,000	£0
New Therapy Costs		£0	£1,200,000	£2,400,000	£3,600,000	£4,800,000	£6,000,000
Total Drug Costs		£5,000,000	£5,200,000	£5,400,000	£5,600,000	£5,800,000	£6,000,000
Rehospitalizations Avoided		0	10	20	30	40	50
Reduction in Rehospitalization Costs		0	£200,000	£400,000	£600,000	£800,000	£1,000,000
Change in Costs							
Change in Drug Costs		£0	£200,000	£400,000	£600,000	£800,000	£1,000,000
Change in Rehospitalization Costs		£0	-£200,000	-£400,000	-£600,000	-£800,000	-£1,000,000
Total Change in Costs		£0	£0	£0	£0	£0	£0

Illustrative exemplar of a novel budget neutral therapy

Case study 5: We use budget impact analyses to understand how different pricing schemes may affect affordability and uptake



- Budget impact analyses (BIAs) assess overall affordability, which can impact price and volume potential in all markets, but nowhere is this relationship defined more explicitly than in England
- We used CUA to assess the UK price potential for a novel ATMP, and subsequently used BIA to understand the potential volume implications under the net budget impact 'test'; we showed how performance-based annuity payments can increase volumes at launch (as compared to a full upfront payment) without triggering further commercial negotiations
 - This informed the developer's revenue projections and P&R and market access strategy



* Using maximum price potential identified through CUA divided by five years (the assumed duration of the annuity scheme)

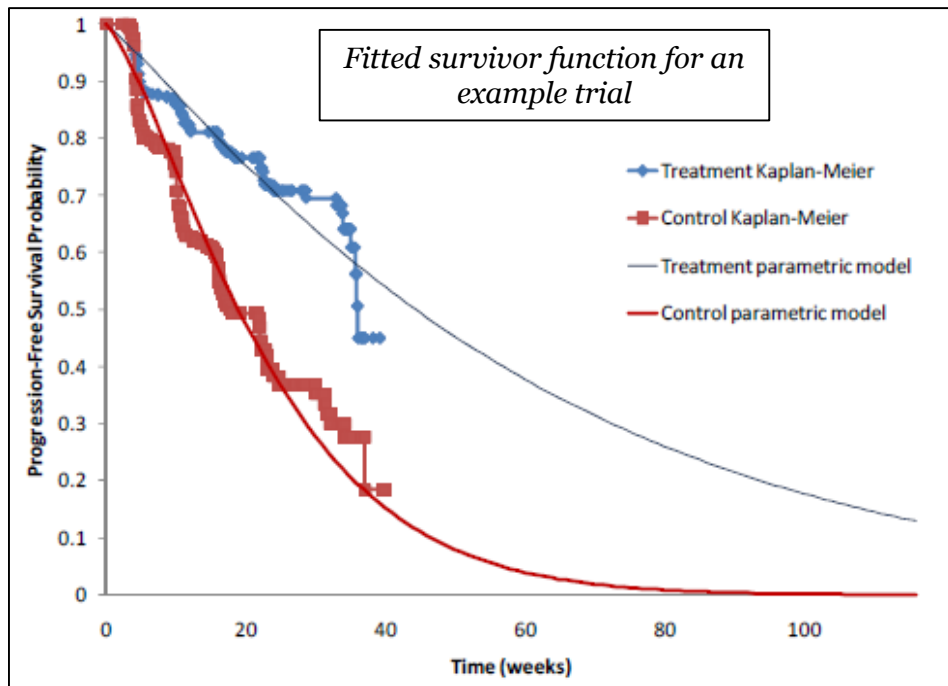
** Over five years

^ Maximum number of patients in years one through five (as annuity payments are split over five years)

Case study 6: We apply regression analysis to bridge the evidence gap between short-term trial data and long-term value claims



- We apply the methods provided by NICE's Decision Support Unit*
- Specified parametric models are fitted
 - Exponential, Weibull, Gompertz, log-logistic, log normal, generalised Gamma
- Optimal model selected based on statistical considerations and external validity



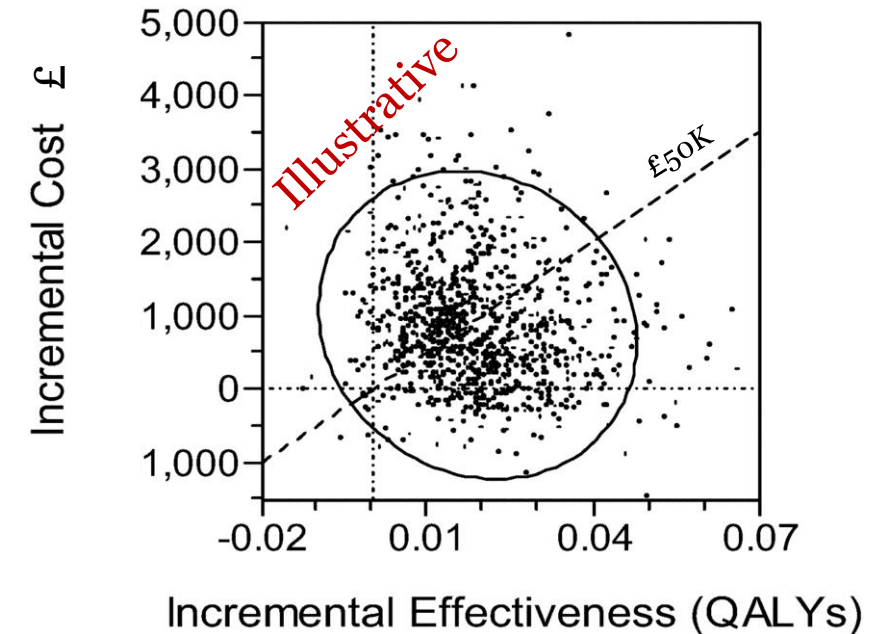
- Sensitivity analysis is undertaken using alternative plausible models
- The resulting degree of uncertainty depends on:
 - The relative length of the extrapolated period vs. the observation period
 - The ability to validate extrapolated data on the basis of biological plausibility, predictive surrogate markers, clinical expert opinion etc.

*NICE Decision Support Unit Technical Support Document 14: Survival analysis for economic evaluations alongside clinical trials – extrapolation with patient-level data, March 2013

Case study 7: We quantify uncertainty metrics to support the cost-effective price determination



1. Probability of not exceeding the ICER threshold (based on probabilistic sensitivity analysis (PSA); % iterations \leq ICER threshold)
 - No defined threshold: ~70% probability of being CE is considered of low uncertainty
2. Incremental Net Health Effect (NHE) expressed in monetary or QALY terms; it is the mean value across all iterations i.e.

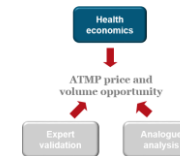


$$\text{Incremental NHE} = [(\text{Incremental Effectiveness}) \times (\text{ICER threshold})] - [\text{Incremental Costs}]$$

The NHE should be positive for adoption; the greater, the more likely

3. Incremental NHE at population level and over the technology time-horizon

Case study 8: Calculating the consequences of decision uncertainty is another way of addressing data uncertainty



Consequences of Decision Uncertainty (calculated according to the Expected Value of Perfect Information framework)

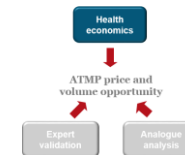
Illustrative

Scenarios (PSA iterations)	Treatment Net Health Effect (NHE) in terms of QALYs		Optimal Choice	Opportunity Loss when choosing B vs. A (in QALYs)
	<i>Treatment A</i>	<i>Treatment B</i>		
1	9	12	B	0
2	12	10	A	2
3	14	20	B	0
4	11	10	A	1
5	14	13	A	1
Mean value across all scenarios	12	13	B	0.8

Consequences of decision uncertainty at individual patient level

- This can be calculated at population and technology time-horizon level
- No defined threshold; relative magnitude in comparison to NHE is key

Case study 9: The uncertainty metrics can help us identify the optimal Managed Entry Agreement



Illustrative

Scenario	ICER	Incremental NHE QALY *	Probability Cost Effective	Consequences of decision uncertainty QALY *	Adoption potential
£100,000 one-off acquisition cost per patient	£50,000	-55	50%	300	Very low
10% discount	£45,000	200	65%	250	Low
Pay-for-performance: <i>payment only for patients with remission by day 30</i>	£40,000	250	70%	100	Possible
Lifetime leasing: <i>payment on a monthly basis as long as patient remains alive (£2,000 pcm)</i>	£35,000	1,000	99.5%	2	High

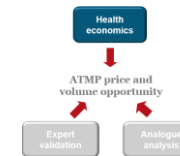
Maximise

Minimise

Pricing scheme that optimises uncertainty metrics

*Based on end-of-life ICER threshold: £50,000

Case study 10: We developed a framework for quantifying administrative cost of outcomes-based reimbursement (OBR)



- The methodological framework was developed with input from a Project Advisory Group of NHS stakeholders and representatives from NICE
- Respondents detailed the tasks and activities related to four implementation phases across various pricing schemes for a given therapy

Pricing scenario	Set-up	Intervention	Monitoring	Exit
Full upfront payment				
Annuity		<i>Cells to be populated through research</i>		
Rebate				
Other				

- The data was categorised by task, time required to complete the task, job band, and capital investment, before grouping by hospital departments
- Participants provided their respective job bands, and time resource was costed using the mid-point salary from the NHS Pay Scales
- The results were reported as cost (£s) and time; per implementation phase; total and incremental costs; direct and indirect costs
 - Direct costs: relating to ‘per patient activity’ (mainly variable costs)
 - Indirect costs: not patient number-sensitive, e.g. set-up costs and infrastructure that is required for the system to run (mainly fixed costs)

Case study 10: We applied this framework to the hypothetical CAR T-cell therapy assessed by NICE, using a staged payment OBR scheme over 10 years

- The scenario with OBR is associated with a less mature data set, while the scenario without OBR, the therapy is assumed to have more mature data
- The time horizon of the OBR scheme is assumed to be 10 years, and 50 new patients were assumed to be treated each year
- The focus of the analysis is the administrative burden (the therapy cost and associated patient management costs were excluded)

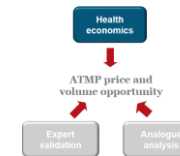
Incremental administrative burden of introducing a CAR T-cell therapy *with* an OBR

Comparator	Total (10 years)		Per year	
	£	Time	£	Time
SoC	£1,814	11 days	£181	1.1 days
CAR T-cell therapy w/o OBR	£2,403	15 days	£240	1.5 days

Key learnings:

- In the context of a gene therapy with a likely price tag in the hundreds of thousands of pounds, the incremental administrative cost per patient seem reasonable (i.e. £181 per patient per year compared to the SoC)
- Most of the additional administrative costs stem from the additional monitoring for OBR
 - The monitoring phase represent 87% of the additional cost, 58% of which are concentrated in year 1 (due to greater number of blood tests to address the uncertainty around safety and efficacy)
 - 56% of the additional costs are in the pharmacy department, due to the requirement for additional pharmacy personnel time and the higher salary band for this type of personnel

Case study 11: Assessing the appropriateness of the NHR for facilitating outcomes-based reimbursement (OBR)



- We used secondary and primary research to explore how the National Haemoglobinopathy Registry (NHR) can facilitate OBR in thalassaemia

Key findings:

Opportunities	Challenges
Many key data points recorded: <ul style="list-style-type: none">Blood transfusion frequencyIron chelation therapy givenMortalityNumber of hospital admissionsComplications and serious adverse events	Key data points currently not recorded (in NHR): <ul style="list-style-type: none">Blood haemoglobin levels (proxy for anaemia)Stem cell transplantation dataUnits of blood transfused
Completion rates ~90%	Annual reporting of data (meaning OBR schemes need to be on annual time horizons)
Future funding to incentivise higher completion rates	No official completion rate data
	Staff shortages are a barrier to complying with data entry
	Patient reluctance to grant NHR access to data

Key learnings

- The NHR in its current form only partially provides the framework needed to enable OBR
 - Not optimal for implementing patient-level reimbursement schemes, schemes that require more frequent than yearly readings and/or outcomes not currently recorded
- NHR can be a vehicle for cohort-level OBR with annual intervals based on e.g. transfusion independence, if:
 - The appropriate staff resource is provided
 - There is a successful drive towards ensuring all patients have their data recorded
- Being able to leverage NHR as the data collection infrastructure for OBR in thalassaemia depends on:
 - NHS England's current restructuring efforts being successful in increasing data entry compliance and improving patient consent rates to sharing data
 - Creation of additional data fields

<https://ct.catapult.org.uk/how-we-work/health-economics-and-market-access>

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