Quantifying What Matters to Patients

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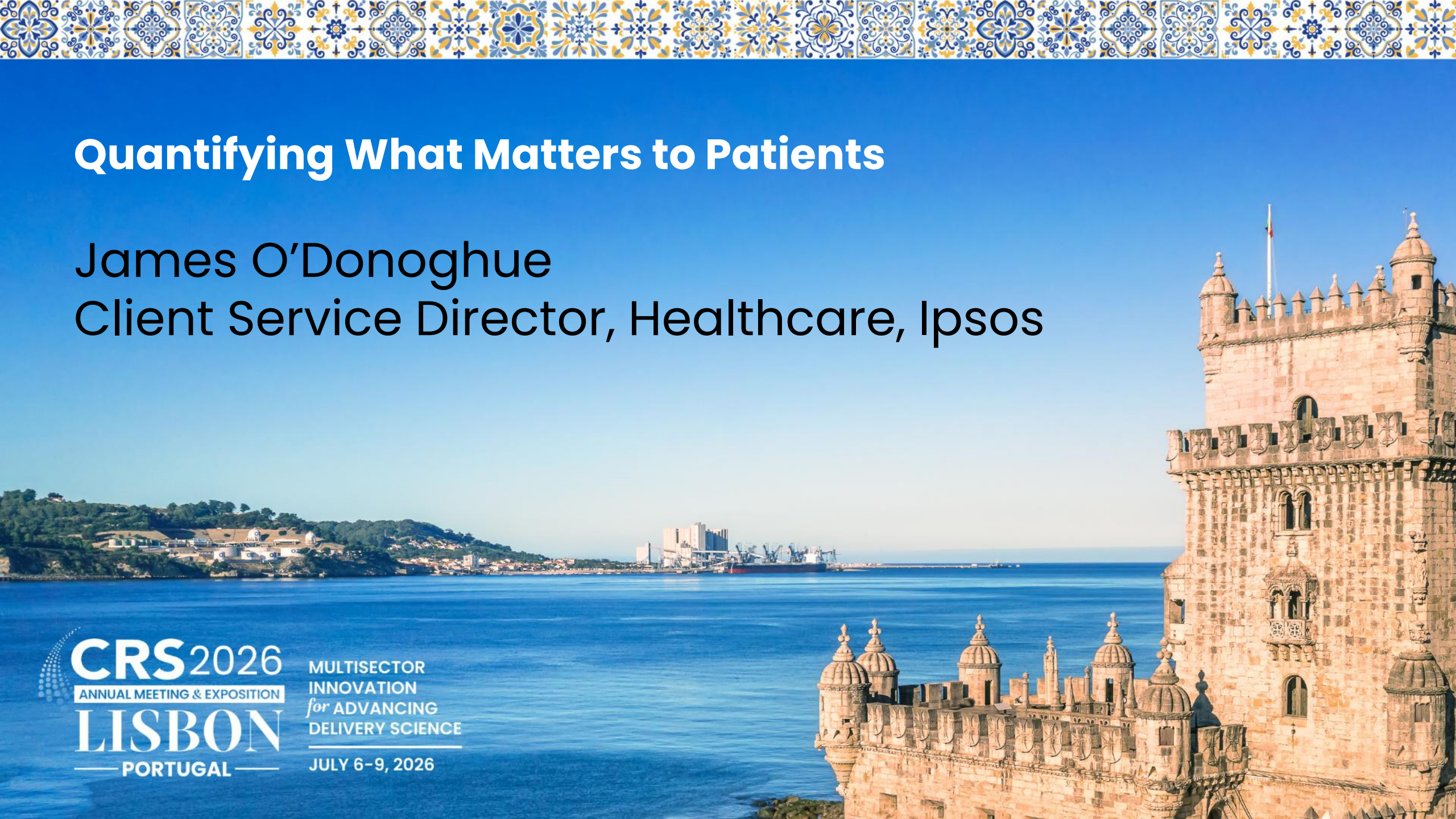

Why Generate Primary Evidence?

- ❑ Inform device design by prioritising attributes and features that address unmet needs
- ❑ Quantify the added value of features within real-world context
- ❑ Identify high-need patient segments to maximise utility
- ❑ Support messaging and value proposition development for launch readiness
- ❑ **Avoid late-stage pitfalls (e.g. over-investment in low-impact or “gimmicky” features that do not improve adherence or quality of life)**

Evidence Approaches in Practice

1. Market Research

- Commercial
- Decision-focused
- Flexible design and timelines

2. Real-world evidence

- Non-interventional studies (prospective or retrospective)
- Increasingly used in HTA and submissions
- Protocol-driven; ethics and governance required

3. Secondary data

- Existing data sources (e.g. real-world data, databases, claims)
- Often syndicated
- Large-scale and longitudinal
- Limited control over variables and level of detail

Selection will be driven by key decisions affected

- Approaches are often combined
- Factoring in: Stage of development, evidence requirements (e.g. regulatory, HTA, commercial), timelines and resources

From Questions to Evidence

- ❑ Define key decisions rather than broad exploratory question
- ❑ Structure attributes into clearly differentiated and manageable sets
- ❑ Design around meaningful end-benefits (e.g. simplicity, usability, dose-confirmation)
- ❑ Apply appropriate methods, e.g. to capture trade-offs while remaining intuitive for respondents (e.g. Best-Worst scaling: conjoint techniques, Max Diff exercises) include randomization, filtering & routing, qualitative / explorative ethnographies / piloting

- ✓ Estimate market opportunity and distinguish must-haves vs optional features
- ✓ Avoid reliance on untested assumption: Build evidence early & reduce launch risks

Case study_1

Patient preferences for injection devices

Situation

- Pharma company considering future device & digital innovation strategy
- Define which injection device features truly add value to patients
- Challenge: wide range of possible “smart” innovations vs unclear priorities

Approach

- Quantitative multi-country, online surveys with patients & caregivers who self-inject at home, indication-agnostic
- Over 600 (4 distinct groups)
- Best-Worst Scaling (max diff) exercise across device & digital features
  - I. Core device attributes, e.g. easy handling, dose-confirmation, temperature-excursion tracking
  - II. Connected / “smart” features, e.g. individualized reminders, connectivity

Outcome

- Publication
- Quantified hierarchy of feature importance
- Core functional attributes to prioritize
- Guidance on indication, patient population unmet needs & pain points
- Define role of “smart” features - supportive, not primary drivers of use
- Fed into practical guide to steer device development & innovation prioritisation

Case study_2

Chronic Spontaneous Urticaria patient preferences regarding biologics

Situation

- New injectable therapy in development for CSU
- Competes with antihistamines, DMARDs, biologics
- Need to understand relative importance of treatment attributes

Approach

- Sequential mixed-method study:
- CSU patients only
  - Qualitative interviews for attribute identification & disease burden insights
  - Quantitative choice-based experiment (CBE) Multi-country surveys
- n=10 interviews / country, n=150 online surveys / country

Outcome

- Quantified importance of treatment attributes
- Identified patient-relevant trade-offs
- Cross-country comparison of preferences
- Scenario testing vs existing therapies
- Outputs informed:
  I. Clinical development priorities
  II. Patient-relevant endpoints
  III. Value communication

Case study_3

Ambulatory infusion-pump design & market definition

Situation

- Manufacturer preparing next-generation infusion pump and considering new innovative features (slim, multi-use, smart features)
- Broad, indication-agnostic opportunity (post-acute care stay, ambulatory infusion)
- Optimize device design and define target use-case

Approach

- Large-scale quantitative study
- 8 x countries 1360 online surveys
  - 855 Physicians, Nurses, & Hospital Pharmacists
  - 500 Patients & caregivers
- Extended survey length (30 to 40 minutes)
- Conjoint Exercise + MaxDiff:
  - Core device attributes
  - General features & end-benefits
- Focused on trade-offs and preference hierarchy

Outcome

- Design optimisation of a new mechanical infusion pump
- Compact, lightweight, robust, Long battery life (full infusion cycle), High usability (simple setup, minimal programming)
- Key drivers of preference: Mobility & independence, Safety (e.g. air bubble, anti-kink), Ease of use
- Clarified role of connectivity: work-flow & documentation not patient-facing autonomy, quantified willingness to pay for advanced ‘smart’ features
- Target use-case: extended ambulatory IV therapies involving variable, multi-phase infusion regimens with programmable flow and EPR integration