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Introduction and objective

• To identify future evidence pricing and access challenges, and solutions for overcoming these, highlighting the

systemic, stakeholder, and organisational changes required.

Methodology

• Using the ISPOR top 10 HEOR trends • Scope covered complete lifecycle



Conclusions

- To overcome challenges, **changes** will be required in 5 areas⁴:
- Systemic changes (Information Technology (IT) Systems, Regulatory and Health Technology Assessment Systems, Coding Payment and Funding Systems, Ethical and Legal Systems}
- HEOR), Market Access, Market / Business insight)
- 4. Strategic collaboration between manufacturers and data providers to access data
- 5. Integration of data from multiple sources (Prescription data, Electronic medical records (EMRs), Health



2022/3 as catalyst, a 2-cycle delphi approach based on broad expert opinion (n=41) was used to determine the key evidence pricing and access challenges over the period 2023-30.

pathway, evolution of digital health and genomic insight, and consideration of value attribution. Cluster analysis was used to structure the output.

For each cluster, solutions to overcoming the challenges were identified.



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• The majority of the 30 specific challenges were applicable to most therapy areas.

Results

• The 30 challenges involved multiple stakeholders and capability requirements.

Sequencing Affordability **Evidence** Personalisation 30 specific Value Attribution Assessment challenges were identified that were Pricing Portfolio Optimisation "clustered" into 10 archetypes Pace of Change Differentiatior



- 2. Stakeholder changes (Physicians, Patients, Payers, Manufacturers)
- 3. Organisational changes (Commercial, Evidence Development (R&D and





Solutions

Evidence, pricing and access will need to be re-engineered to address four factors

Senerative AI and Big Dat re major future disruptors, even in R&D. Medical Delivery and Market Access

Science, Technology

& Al



Multi-Stakeholde Value Attribution

shift in focus from the sessment of a drug or alue of healthcare sease prevention and isease management.

Multi-Source Data Integration

egulatory and Health echnology Assessments asingly embracing da nd evidence beyond ditional randomised ontrolled trials

Fundamental changes will be required to overcome the evidence availability, accessibility, and acceptability challenge

R&D and HEO gulatory & HTA Ethical and Legal Stakeholder Change Requirement = need for new integration collaboration or policy change

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Multi-Component

Disease Managemen

al-world/real-time data

ether with biomarker-

ormed precision

New methodologies

Modified Porter Analysis

STEEPLE frameworks Delphi Method

Multiple Data Source

• The research indicated that the challenges can rarely be solved in isolation, in a single company or departmental silo, or based on single data sources.

Collaboration and access to multiple 'integratable' data sources will be critical success factors.

• The largest cluster (7/30) comprised personalisation challenges (digital and molecular diagnostics / biomarkers linked to therapeutic).

These challenges must be addressed if the promises of improved economic, clinical and humanistic outcomes are to be delivered^{1 & 2}.

Three key challenges need to be addressed for digital health to be successful



The second challenge: evidence rement and attribution of co-dependent value between evelopers of the different disease management components



The third challenge: value Value will be attributed and assessed in different ways MIP paradigm) Value segmentation based on 3 outcome types: economic, clinical & humanistic Value perception based on 3 stakeholder groups: patient, payer & physician Value attribution, informing value-based reimbursement allocation

will need to be applie to help decision-make understand drivers of change

Non-comparative dat plus modelling solution will increasingly be required

Financial engineering will be needed to address the challenge of gene, and other therapies, with high price density

New payer-types will emerge, and funding flows will need to change to relieve financial

| ed ers f | How the forces of competition, power of buyers/suppliers & threat of substitutes influence decisions & outcomes. | Social, Technological, Economic, Environmental, Political, Legal & Ethical considerations | A structured approach to expert opinion, achieving consensus & highlighting areas of misalignment | How data & insight from many sources can be merged to inform decision-making |
|----------------|--|---|---|---|
| | Behavioural Science & Prospect Theory | System Dynamics | War Gaming / Competitive Simulation | Stakeholder Tracking |
| | How behaviours influence decisions & outcomes, and how experience, uncertainty & risk change preferences | Understanding the dynamic behaviour of complex systems & the relationships between the component parts | Experiential methodology to guide decision making in a dynamic competitive rapidly changing environment | Monitoring how differentiation & investment impacts multi- stakeholder perceptions, behaviours & outcomes |
| ta, ons, | RCTs aren't always ethical, feasible, or practical | Uncontrolled studies are acceptable to Regulators (less so to HTA) | Non-comparative studies may provide the "best available" evidence | Iterative modelling fills the gap |
| | Detecting statistically significant differences between treatment arms will be hard due to low n-numbers – for example in rare genetic disorders. There are also often no established comparator treatments | When change in a condition can clearly be attributable to the therapy, prognosis bleak, and there is no acceptable control arm (FDA, 2007). The endpoint should, however, be 'hard/objective' | In the clinical trial setting: e.g. single-arm trials, case series, and case reports In the 'real-world' setting: registry studies (often necessary to provide data for outcome-based managed entry agreements and some observational designs | Comparison of single-arm trial with an artificial comparator arm built out of real-world data based on modelling, has been used in regulatory submissions (FDA/EMA) and health technology assessments |
| 9 | Gene therapy presents specific challenges… | cost is the biggest concern | and uncertainty around long-term benefit | leading to problems of defining value |
| es | Cost and affordability Funding flows Uncertainty: absence of data around long-term benefit Value definition | The cost of these therapies can be extremely expensive. Budget impact could be amplified depending on the size of the patient population. A further challenge is the timing of the cost. The fact is that all or most of the costs are up-front, not borne over time, as with chronic treatment | The pathway to approval of gene therapies (especially if expedited) may yield shorter- term data on efficacy than is needed to prove long-term benefit. This results in great uncertainty around how long the therapeutic benefit will last and whether a single dose will be sufficient to provide a lifetime cure. This impacts payer willingness to pay and ability to pay | Payers may have to incorporate measures of value to patients, the healthcare system, and society in the standard value assessments. Additional metrics include: disease severity, age of onset, lifetime burden of the illness and informal care elements, such as returning to work or study, increases in productivity and reductions in burden of care |
| | | | | |

Payment options for gene therapies

Traditional financial mechanisms to pay for pharmaceuticals are not adequate for gene therapies. Alternative payment models, more common to the financial services sector, will be increasingly adopted:





pressures

Re-Insurance Supplier Credit Mortgage / Loan Direct Payment **Risk Pools**

Any one, or combination, of these models have the potential to incentivise payers to invest in a gene therapy that may produce a better health outcome and lower cost over time, as opposed to paying for a competing product that is administered, with higher long-term costs – or even with a larger one-time/upfront costs for a curative therapy.

Eight barriers to molecular diagnostics and five to therapy use will need to be removed for biomarker driven healthcare to be successful

| Barriers to molecular | diagnostic use may include: | Barriers to therapy use may include: |
|---|---|--|
| FundingAccess to and availate | ability of testing | Affordability, access and availability: of drugs, diagnostic testing, reimbursement, and of data/evidence |
| Testing methods and turnaround time | process: difficulty of obtaining sample, complexity, and | Technology Assessments and implementation, and time lag in adopting technology |
| Test performance: w accurate or ambiguo | ill the test be (or be perceived to be) insufficiently us? | Preference: influenced by context, personal experience, and outcomes or earlier treatment(s) |
| Population selected drug's indication? | for testing: will the population tested be as broad as the | · Policy & priorities |
| · Physician's adoption | of the test proposition | Power: Physician vs. payer, HTA vs. medical society, and treatment guideline perspectives differing from patients' perspectives. |
| Patient demand for t | esting, and willingness to be tested | |
| Conversion rate: will test result? | physicians prescribe other drugs despite a "positive" | |



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