Welcome from our hosts

Genesis Research provides agile, tech-enabled HEOR and RWE solutions to life sciences companies, providing them with a better way to develop impactful evidence and optimize market access.

Market Access Transformation specializes in developing agile technology platforms that enable healthcare manufacturers to gather insights and assess the commercial viability of their products.

In May 2022, MAT became part of Genesis Research

Our speakers

Priti Jhingran, PhD
VP, Evidence Strategy
Genesis Research

With over two decades in the pharma industry, Priti Jhingran has focused on understanding evidence needs and delivering tools/solutions for access decision makers/HTAs. She has led multiple enterprise level initiatives; launched 15+ products; and developed diverse teams of scientists dedicated to the generation, dissemination, and communication of evidence.

Tijana Ignjatovic, PhD
Director, Operations Team
Market Access Transformation

Tijana Ignjatovic has over 15-years of consultancy experience within market access, having conducted over 100 pieces of research across a range of therapy areas during her time at MAT. This experience has given Tijana in-depth knowledge on how to optimize research methodologies to meet the strategic intent of payer research and provide actionable recommendations.

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Payer Insights and Evidence: Meeting the Challenges of the New Evidence Paradigm

Priti Jhingran, PhD
VP Evidence Strategy, Genesis Research

Tijana Ignjatovic PhD
Director Market Access Operations, Market Access Transformation

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Today’s focus

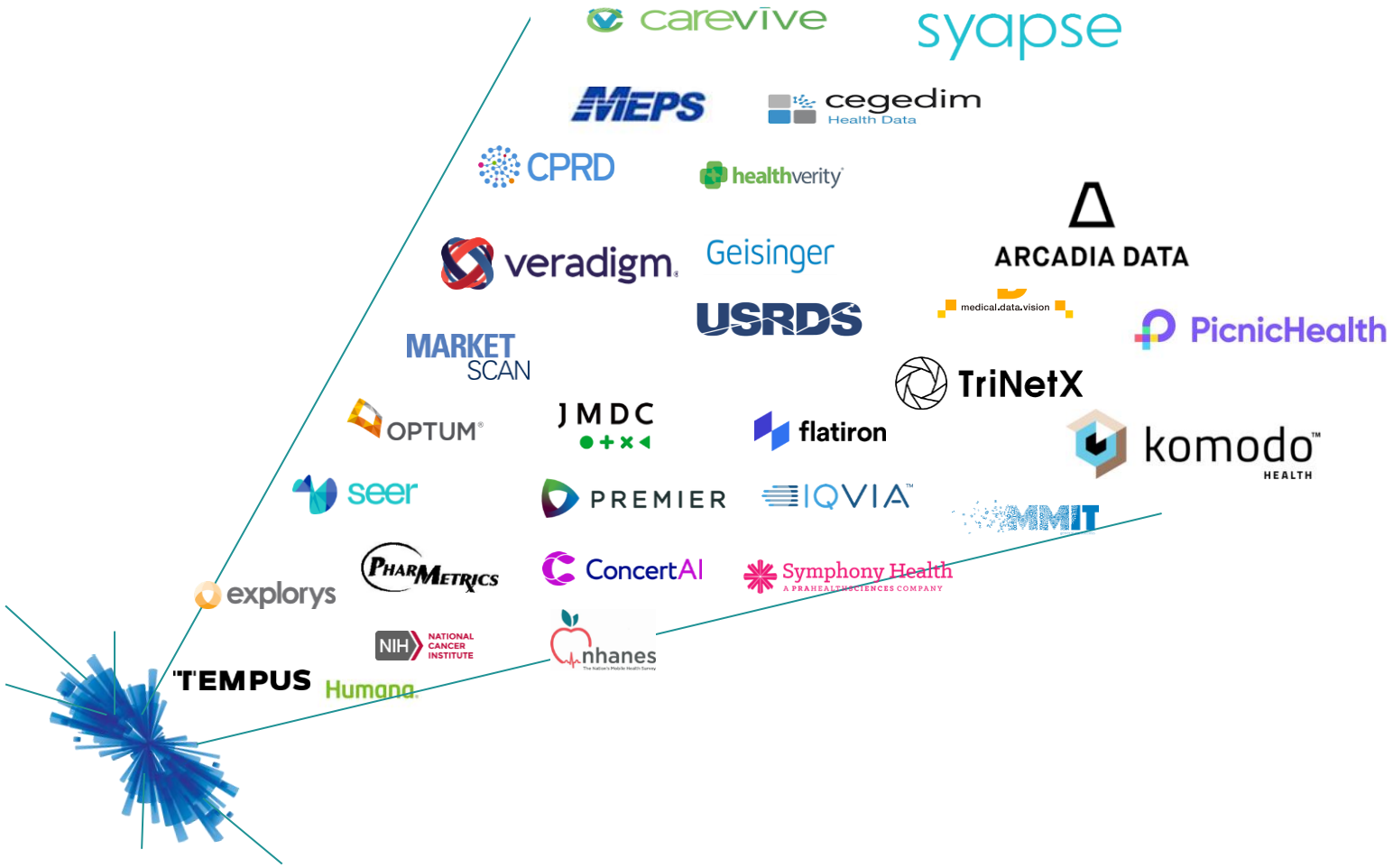
Evidence and **insights** are critical to life science companies (LSC) drug development and commercialization efforts and can accelerate access to better care by successfully meeting the evidence needs of regulators and health technology assessments (HTAs)/payers.

Agenda

1. Trends Creating a New Paradigm
2. Insights Informing Evidence Strategy
3. Meeting the Challenges of New Evidence Paradigm: Case Studies
4. Live Q&A
5. Closing Remarks

Trends Creating a New Paradigm

Novel data sources and data integration methodologies have expanded the availability of innovative, rigorous, fit-for-purpose evidence and creating a new paradigm



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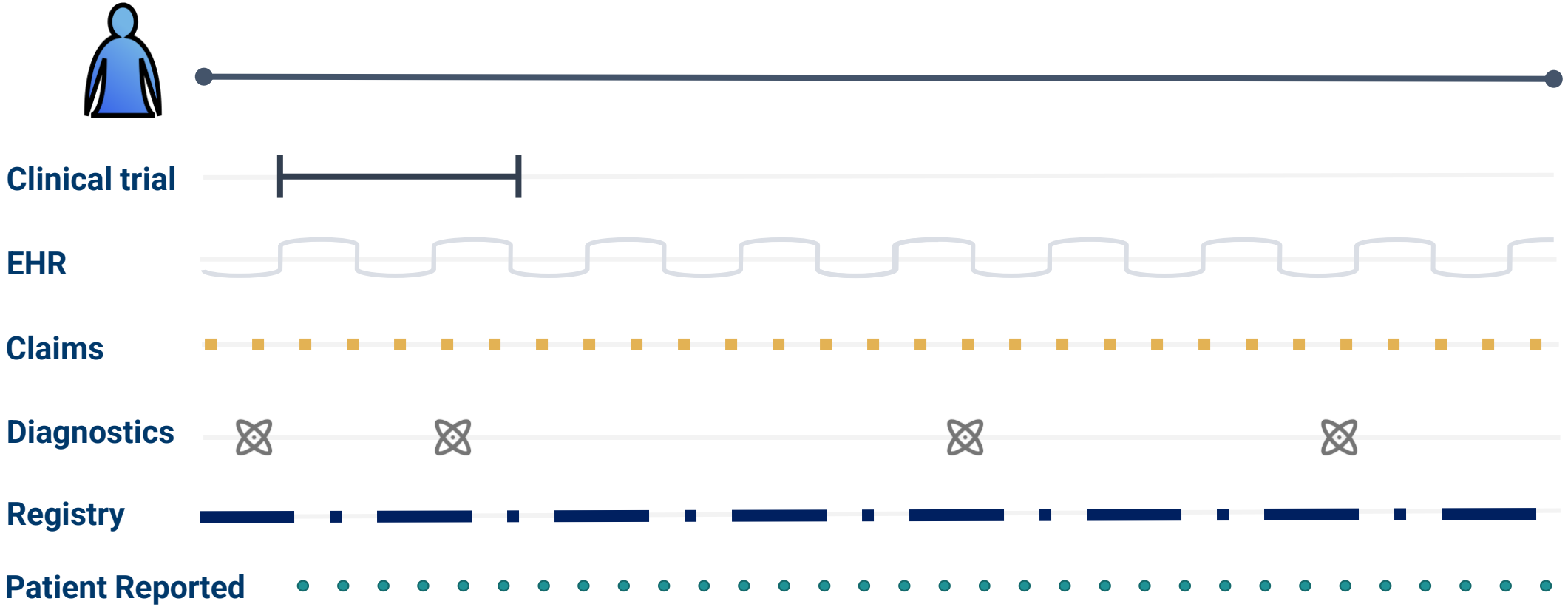
New evidence paradigm provide opportunities to address evidence gaps



EHR, Electronic Health Records

There is increased adoption of evidence to support decision-making across the product lifecycle

Increased adoption of real-world evidence (RWE)

- Pre- and post-approval phases[1]
- Augment with clinical trials in regulatory and reimbursement processes[2,3,4,5,6,7]

RWE are used to

- Inform RCTs design elements
- Contextualize and strengthen regulatory label
- Ensure stakeholders understand patient needs
- Reduce time to market

[1]FDA 2022a; [2]Dagenais 2022; [3]FDA 2018; [4]Arlett 2021; [5]NICE 2022a; [6]ICER 2020; [7]Schad 2022.

FDA and EMA are taking active steps towards practical use of RWE

FDA

In September 2022, the FDA released guidance to encourage use of real-world evidence in **regulatory decision making**[1]

Guidance outlines considerations for using electronic medical records, medical claims, disease registries while complying with FDA-supported data standards

EMA

The EMA is establishing a coordination center, **DARWIN EU** to deliver real-world evidence on disease, populations, and medicine uses across Europe[2]

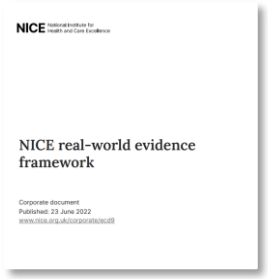
DARWIN EU will establish and expand a catalogue of observational data sources for use in medicines regulation

DARWIN EU, Data Analysis and Real-World Interrogation Network; FDA, Food and Drug Administration; EMA, European Medicines Agency; [1]FDA 2022b; [2]EMA 2020.

HTA Frameworks encourage use of RWE for many use cases

NICE

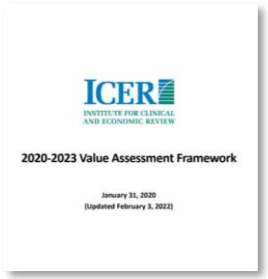
NICE has developed an RWE framework to describe best practices for utilizing RWE to improve guidance[1]



The NICE framework encourages use of high-quality, relevant RWE to reduce uncertainties and address data gaps

ICER

ICER encourages the use of RWE to address knowledge gaps in their clinical development programs[2]



ICER views RWE as an effective method to supplement the existing evidence package and provide a comprehensive view of a therapy's comparative and cost-effectiveness

ICER, Institute for Clinical and Economic Review; NICE, National Institution for Health and Care Excellence [1]NICE 2022a; [2]ICER 2020.

Bringing products of value to the market is critical for optimal access

Regulators and payers/HTAs want to better understand

Populations with unmet need

Relevant comparator in populations of unmet need

Meaningful endpoints

HTAs offer opportunities to seek advice

Countries offering early advice

NICE National Institute for Health and Care Excellence

AIFA

Gemeinsamer Bundesausschuss

HAS HAUTE AUTORITÉ DE SANTÉ

agencia española de medicamentos y productos sanitarios

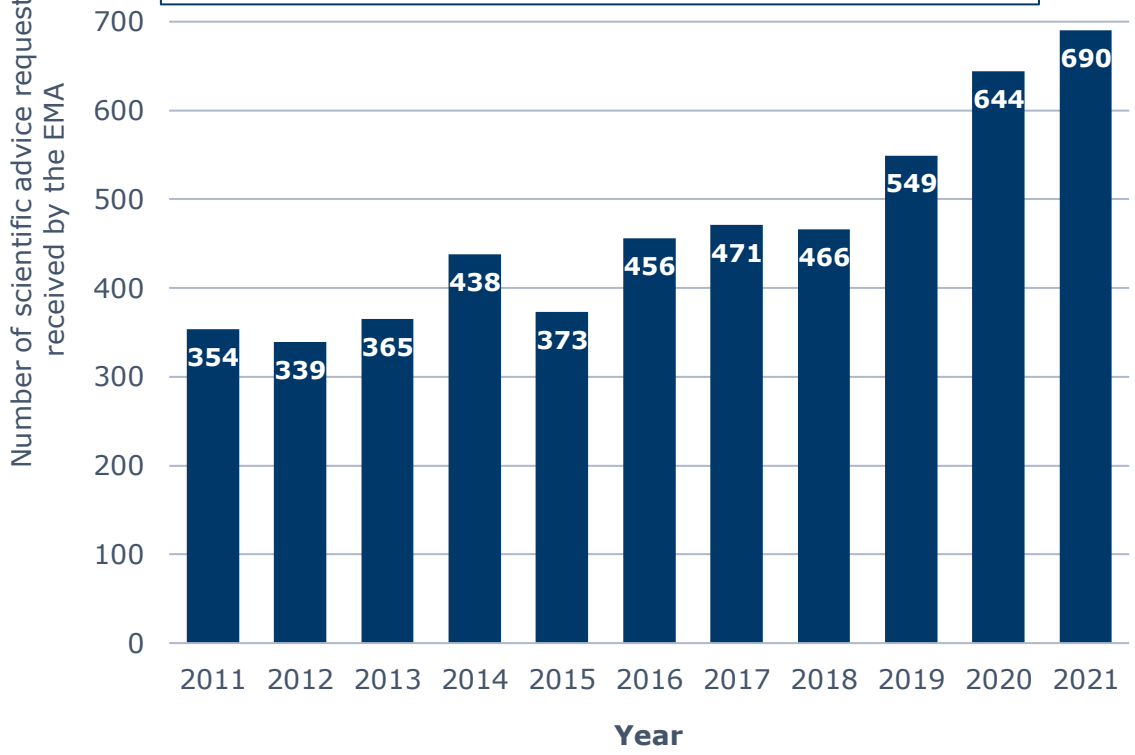
Clinical trial program advice[1]

- Trial population
- Position in treatment pathway
- Comparators
- Outcomes, including acceptability of endpoints
- Patient reported outcomes

Economic evaluation advice[1]

- Economic model (design and approach)
- Data sources and extrapolation
- Resource use and costs
- Utility values

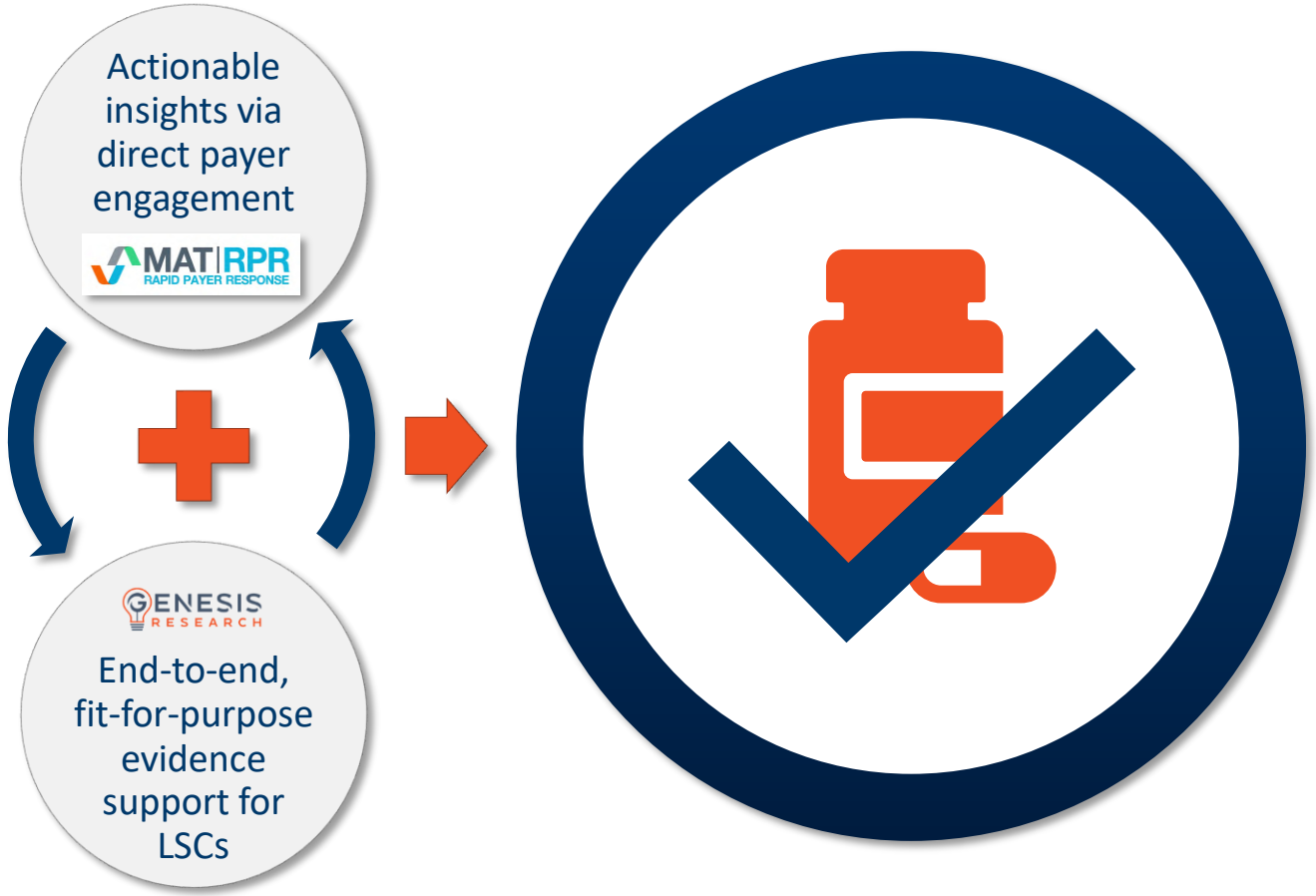
Trend of increasing payer advice by the EMA since 2011[2-4]



[1]NICE 2022b; [2]EMA 2021; [3]EMA 2017; [4]EMA 2013.

Insights Informing Evidence Strategy

The integration of insights and evidence facilitates efficiency across the product lifecycle



Iterative process of

insights & evidence generation

maximizes likelihood of demonstrating value of a product

Insights & evidence generation activities are needed across the product lifecycle

EARLY SUPPORT (PHASE I/II)

PHASE III/LAUNCH READINESS

PHASE IV

Insights (Global & Local): Timely payer insights can inform a relevant evidence package and development strategy

Evidence (Global & Local): Evidence strategy and generation across the value chain, informed by insights, facilitates optimal access at launch/post launch

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Meeting the Challenge of the New Evidence Paradigm: Case Studies

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Case Study 1: Rapid payer feedback on evolving use of RWE in initial decisions and reassessments in oncology (1/2)

CHALLENGE

PHASE I/II → PHASE III → PHASE IV

Large LSC client requested MAT support for gathering payer insights on future use of RWE

- Above brand, oncology-focused
- Assess current situation and explore future RWE use
- Internal educational purposes
- Pulse check on fast evolving situation to refine evidence generation plans and grow internal capabilities to meet the payer expectations

APPROACH

MAT conducted a survey with payers across five markets with the following objectives:

- Assess the role of RWE in the initial assessment and post-launch reassessment of oncology drugs
- Determine the impact of RWE in product pricing and market access (P&MA) outcomes
- Investigate the future potential for RWE in oncology drug assessment and reassessment

Scope markets					
	10	5	5	5	5

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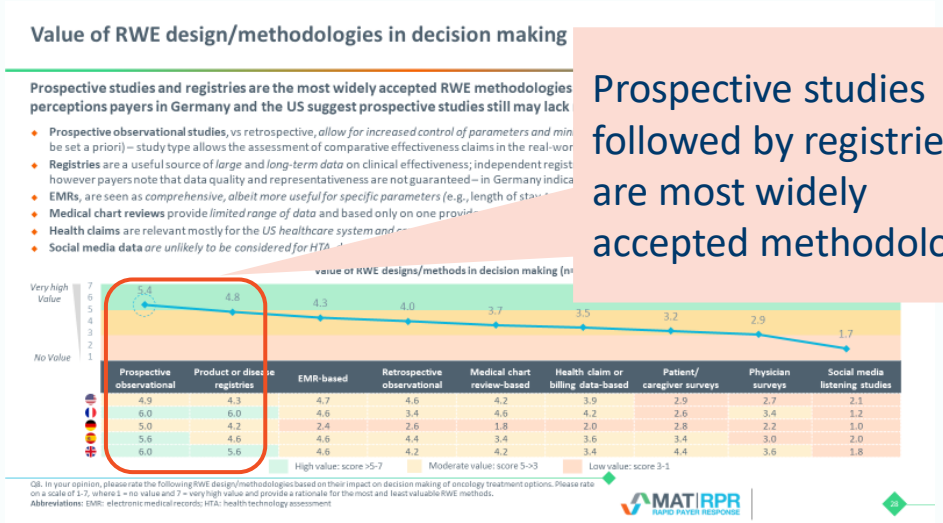
Case Study 1: Rapid payer feedback on evolving use of RWE in initial decisions and reassessments in oncology (2/2)

OUTCOME

- RWE are used at ***initial assessment*** to define the treatment landscape and clinical profile of relevant comparators
- In ***re-assessments*** RWE are confirmatory for the efficacy, safety and level of utilization in clinical practice of the marketed oncology therapy.



Prospective studies followed by registries are most widely accepted methodologies

P&MA outcomes

Spain is leading in usage of RWE in coverage decisions, while NET price can be adjusted with RWE in France, Spain, and the UK

Recommendations

- ❑ Methodological rigor
- ❑ Data sources: robust and local
- ❑ Adherence to guidance and external validation
- ❑ Increased payer perception and acceptance

Case Study 2: Rapid payer feedback on registration trial design focuses HTA Scientific Advice ahead of protocol lock (1/2)

CHALLENGE

PHASE I/II → PHASE III → PHASE IV

Large LSC client requested MAT support for gathering payer insights for their registration trial in a new patient population

- Phase II asset was an being investigated in multiple oncology indications, including non-small cell lung cancer (NSCLC)
- Proposed study design for NSCLC was complex with multiple combinations of therapies, several patient subpopulations, and subgroup analyses
- Critical deadline for HTA advice meeting for registration trial design discussions

APPROACH

MAT conducted a survey with payers across seven markets with the following objectives:

- Detailed vetting of the trial design elements
- Perception of the TPP and price potential upon indication expansion
- Exploration of impact of competing agents' trial designs

Scope markets

15	5	5	5	5	3	5

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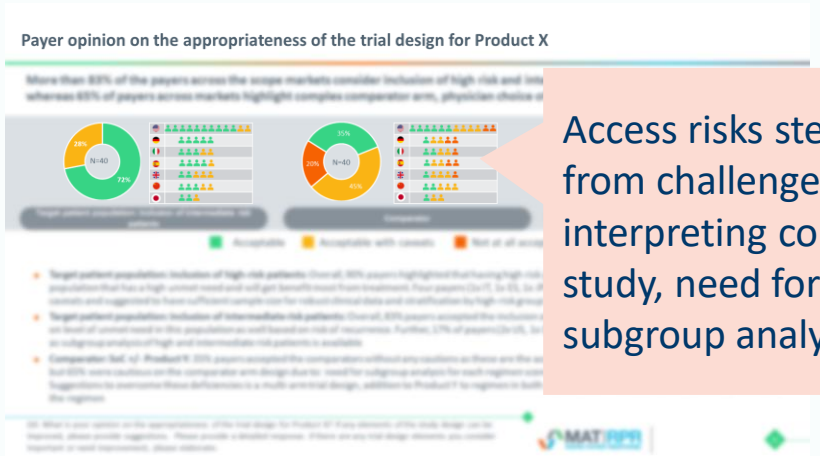
Case Study 2: Rapid payer feedback on registration trial design focuses HTA Scientific Advice ahead of protocol lock (2/2)

OUTCOME



Access risks stemming from challenges in interpreting complex study, need for multiple subgroup analyses, ITCs

Case Study 2: Rapid payer feedback on registration trial design focuses HTA Scientific Advice ahead of protocol lock (2/2)

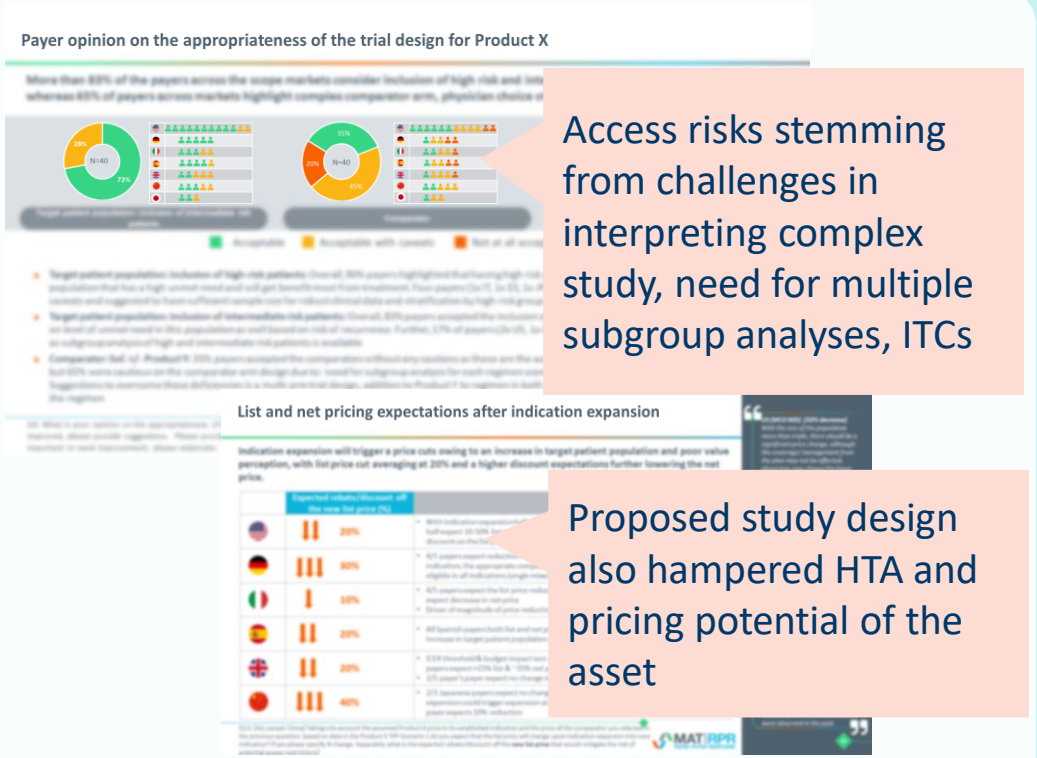
OUTCOME

Access risks stemming from challenges in interpreting complex study, need for multiple subgroup analyses, ITCs

Proposed study design also hampered HTA and pricing potential of the asset

Case Study 2: Rapid payer feedback on registration trial design focuses HTA Scientific Advice ahead of protocol lock (2/2)

OUTCOME



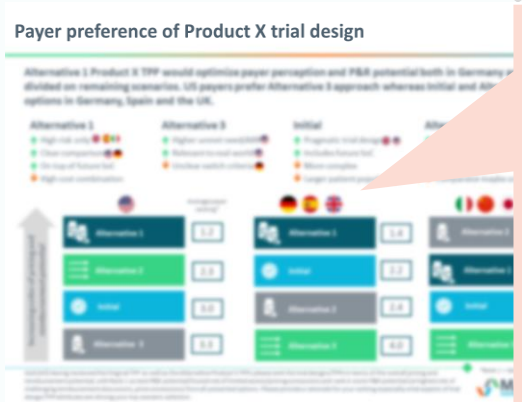
Access risks stemming from challenges in interpreting complex study, need for multiple subgroup analyses, ITCs

Proposed study design also hampered HTA and pricing potential of the asset

Follow up survey objectives

- **MAT proposed three alternative study designs based on payer feedback,** which were tested with payers

Follow-up survey was fielded within 2 weeks of first readout



MAT identified preferred study design for each scope market allowing the client to select approach that maximizes P&MA potential and focus on during HTA advice

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Case Study 3: Rapid and time-sensitive integration of payer/HTA evidence needs leads to adjustments in evidence strategy, including Phase III program (1/2)

CHALLENGE

PHASE I/II | PHASE III | PHASE IV

Clinical-stage LSC requested support for their assets with focus on

- Rapid and time-sensitive integration of payer/HTA considerations into Phase III program
- Comprehensive HEOR/RWE plan

LSC had multiple assets in oncology on a proprietary platform

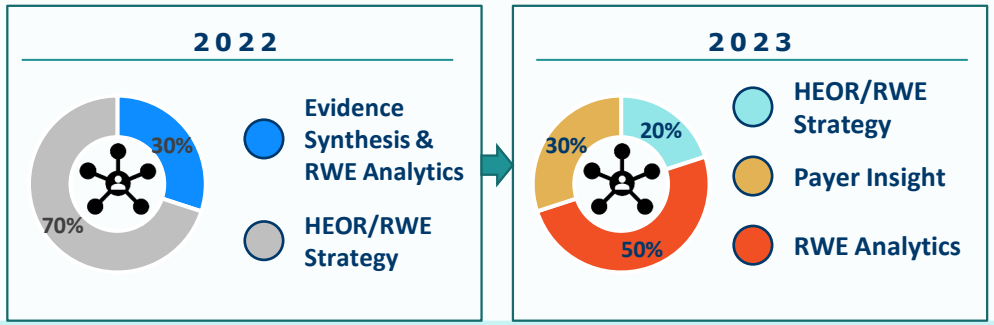
- Asset X: Alliance with another company
- Asset Y: Independent development and commercialization

Case Study 3: Rapid and time-sensitive integration of payer/HTA evidence needs leads to adjustments in evidence strategy, including Phase III program (2/2)

Approach

- Rapid integration into client's cross-functional team
- Deployment of evidence synthesis, HTA analog analysis, & strategic insights to shape phase III program.
- Development of short and long-term strategic plan and its execution

Dedicated Team Composition



Outcome

- **Phase III program was updated in terms of:**
  - Target population definition
  - Structured hierarchical endpoint including incorporation of select PROs
  - Within trial HCRU analysis
- **HEOR/RWE/Insight plan currently being deployed and includes:**
  - Development/testing of payer value proposition leveraging MAT Rapid Payer Response™ platform
  - Planning for early scientific advice
  - Multi-prong, multi-year RWD plan
  - Early economic model

Case Study 4: Comprehensive evidence generation support leads to a better understanding of a disease not well understood (1/2)

CHALLENGE

PHASE I/II → PHASE III → PHASE IV

- Mid-size LSC requested tactical support for a small molecule, first-in-class nephrology treatment that was granted orphan drug designation, i.e., FSGS
  - Evidence needs included
    - Unmet need and burden of illness
    - Prevalence
    - Healthcare utilization and cost
    - Development of patient identification algorithms

FSGS, Focal segmental glomerulosclerosis

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Case Study 4: Comprehensive evidence generation support leads to a better understanding of a disease not well understood(2/2)

Approach

GR conducted a study evaluating the prevalence of FSGS and the impact of proteinuria on BOI and HCRU utilizing linked claims and EHR

- Study protocol and analysis plan utilizing Optum Market Clarity were developed
- Patient identification algorithms used to accurate identify patients utilized data from both claims and EHR.

Published Abstract at ISPOR US 2022: Link

Dedicated Team Composition

Outcome

- **Delivered a comprehensive evidence package supported by actionable insights**
  - Novel patient identification algorithms
  - Robust value story supported by peer-reviewed scientific evidence
  - Inputs for patient funnel, forecasting model, early economic model, etc.

BOI = Burden of illness; HCRU = Healthcare resource utilization

Summary of key points

1. In an evolving healthcare environment, LSCs should take a lifecycle approach to insight and evidence generation that begins in early development
2. An iterative approach to insight and evidence generation can increase the efficiency and effectiveness of development and commercialization activities, leading to reduced time to optimal market
3. Agile partnerships powered by processes and technology improvements can accelerate and optimize insight and evidence generation