



# **Integrated Evidence Generation Planning**

**Leveraging AI and Real-World Evidence to Advance Science**

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**Foundations of planning, integrated approaches, and adaptive frameworks for meaningful evidence across stakeholder needs**

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**Investigator-initiated studies, execution processes, and impact assessment methodologies for evidence generation success**

## **03. Redefining Evidence Generation**

**AI-Enabled Approaches for the New Era**

## **Disclaimer**

**Global Luminary: United Nations (WPHF)**

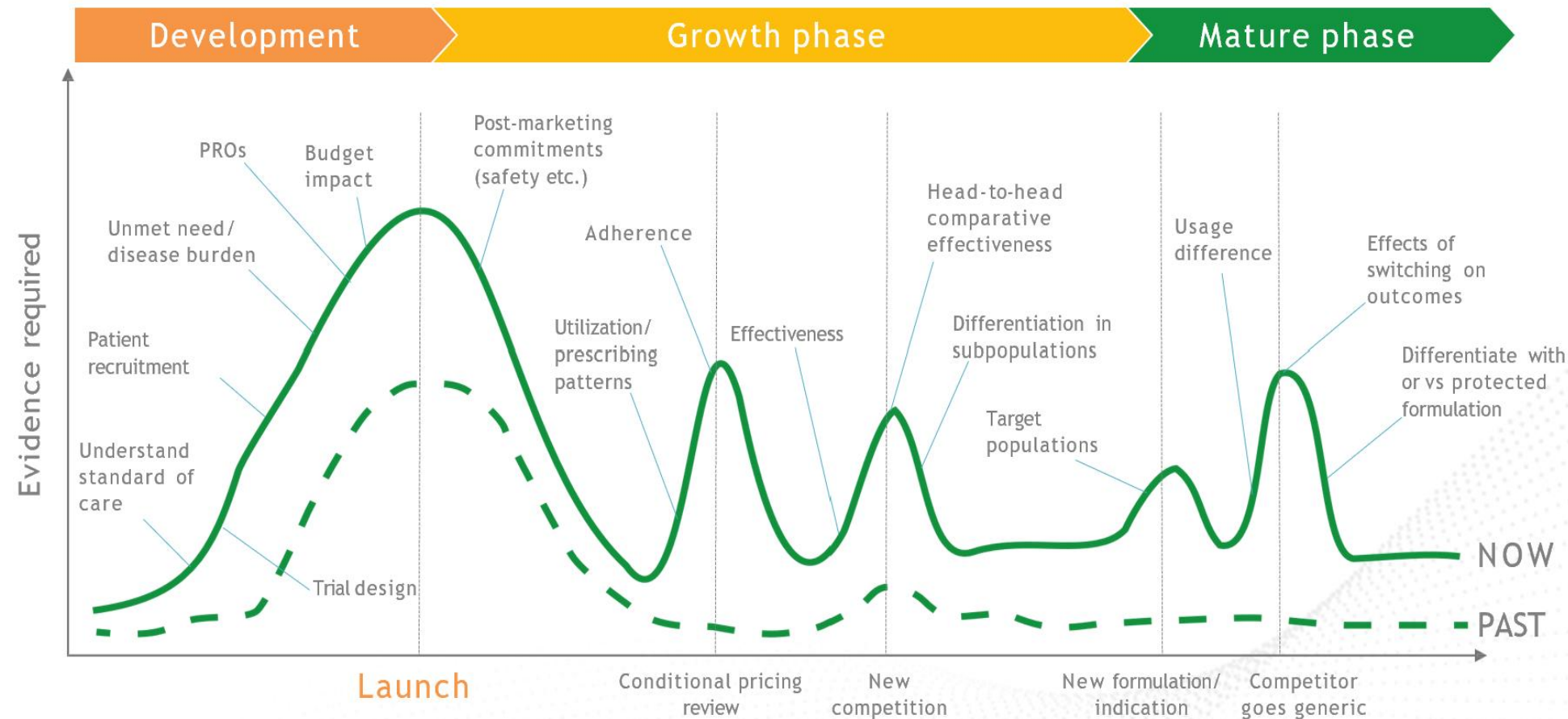
**Fides Member: WHO**

**Board Member: Arogya World, Indo-US Rare, Jiti Foundation**

**Strategic Advisor: Vor Bio, Alumis Inc., Passkey Therapeutics**

***The viewpoints presented are my personal views and  
interpretations***

Evidence planning should consider the entire product journey, and reflect evolving stakeholder needs throughout its lifecycle

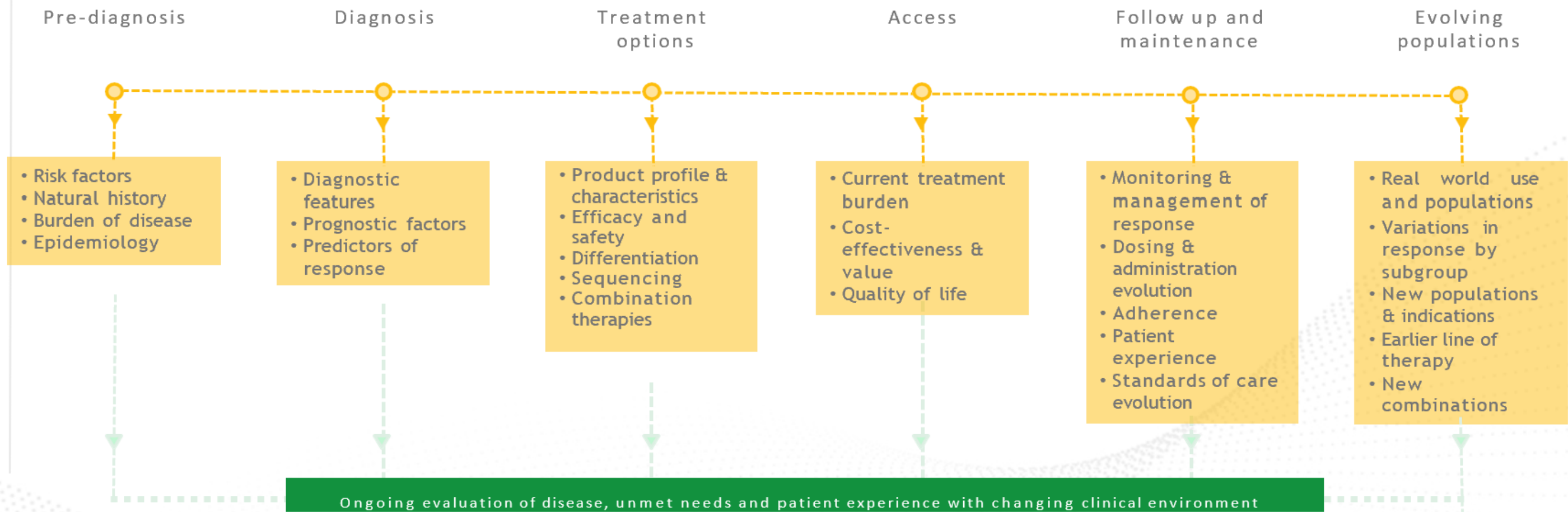


Adapted from EMA Adaptive Pathways Pilot Presentation at STAMP;  
[http://www.ema.europa.eu/docs/en\\_GB/document\\_library/Presentation/2015/11/WC500196727.pdf](http://www.ema.europa.eu/docs/en_GB/document_library/Presentation/2015/11/WC500196727.pdf)

Medical Affairs Professional Society (MAPS)® 2025

Ideally, planning should start ~3 years before data are expected to be needed

# A holistic view is needed to ensure that planning activities reflect the widest needs of stakeholders



Adapted from Integrated evidence generation: A paradigm shift in biopharma; McKinsey report, 2021

# Integrated Evidence Plans Maximize Value Across Stakeholders



## Regulators

- Expedited approval processes require consideration of evidence beyond RCTs

## Payers

- Varying regional and local requirements for successful reimbursement and access
- Need cost-effectiveness, budget impact, formulary decisions

## Healthcare Professionals

- Increasing treatment variety furthers need for evidence supporting treatment decision-making
- Guidance for patient selection, sequencing outcome optimization

## Patients

- Understanding how treatments enhance quality of life for shared decision-making

## Internal Teams

Strategic alignment across medical, market access, and commercial functions



# **Implementation Framework: From Strategy to Execution and Measurement**

# Investigator-Initiated Studies Generate Real-World Evidence



## Strategic Benefits

IIS has advanced knowledge of patient populations and management strategies important to clinical practice.

Benefits include:

- Expanding product knowledge in real-world settings beyond RCTs
- Gathering additional safety data and insights into management processes
- Exploring therapeutic area knowledge
- Investigating new indications or populations not in clinical trials

## Critical Success Factors

- Clear internal and external roles and responsibilities established
- Consistent communication channels
- Standardized processes for data sharing, reporting and storage
- Consistent review processes and risk mitigation approaches
- Building appropriate tools to support execution throughout the study lifecycle.

# IIS Partnership Process: From Request to Publication

## Request Initiation

Investigator submits protocol synopsis or full protocol, budget, clinical credentials documenting study experience, and nature of support requested including medicinal products, material support or financial support.

## Agreement & Setup

Execute agreements with all parties including investigator's institution. Establish communication frequency and format. Define roles for study conduct including safety reporting, ethics approvals, public registry, regulatory and legal agreements, drug supply mechanisms, data ownership, and publication plans. Set up oversight measures.

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## Company Review & Approval

Assessment of ICH-GCP compliance and applicable regulations, medical and scientific value of proposed study, alignment with global and local medical strategy, and quality risk assessment based on risk level and study design.

## Execution & Close-out

Adequate two-way communication with clear AE reporting process. Monitor progress against contract with protocol amendments as needed. Upon completion, ensure all safety activities complete, statistical analyses finalized, close-out processes executed, final deliverables reviewed, and CSR completed for publication.

# Measuring Impact: From Outputs to Strategic Change

Success measurement considers not only evidence creation but wider processes, partnerships, and strategic outcomes through the results chain framework



**OUTPUT**

## Output: Delivery

Volume and quantity of activity delivered. Measures include study completion on time, evidence availability when needed, and productivity metrics



**OUTCOMES**

## Outcome: Quality Results

Results of activities delivered including measures of quality. Updated HCP education incorporating evidence, stakeholder engagement quality, review turnaround times, education resource update speed.



**IMPACT**

## Impact: Long-term Change

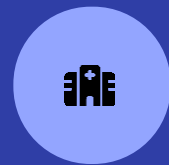
Wider strategic change outcomes contribute to. Enhanced therapy sequencing improving patient outcomes, guideline or formulary changes, practice pattern shifts, sentiment and behavior analysis across stakeholders.

*Potential success measures include the willingness of internal and external partners to engage in collaboration, internal feedback on the IEP development process and stakeholder feedback regarding evidence value demonstrating meaningful change*

# **Redefining Evidence Generation: Is AI the answer?**

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# Traditional Evidence Generation Challenges



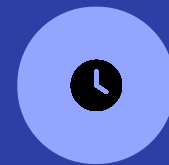
## High Site and Patient Burden

Traditional registries face low completion rates in long-term follow-up



## Representative Population Gaps

Difficulty recruiting diverse, representative populations



## Long-term Follow-up Requirements

Cell and gene therapies, immune-modulating drugs require extended follow-up for safety risks



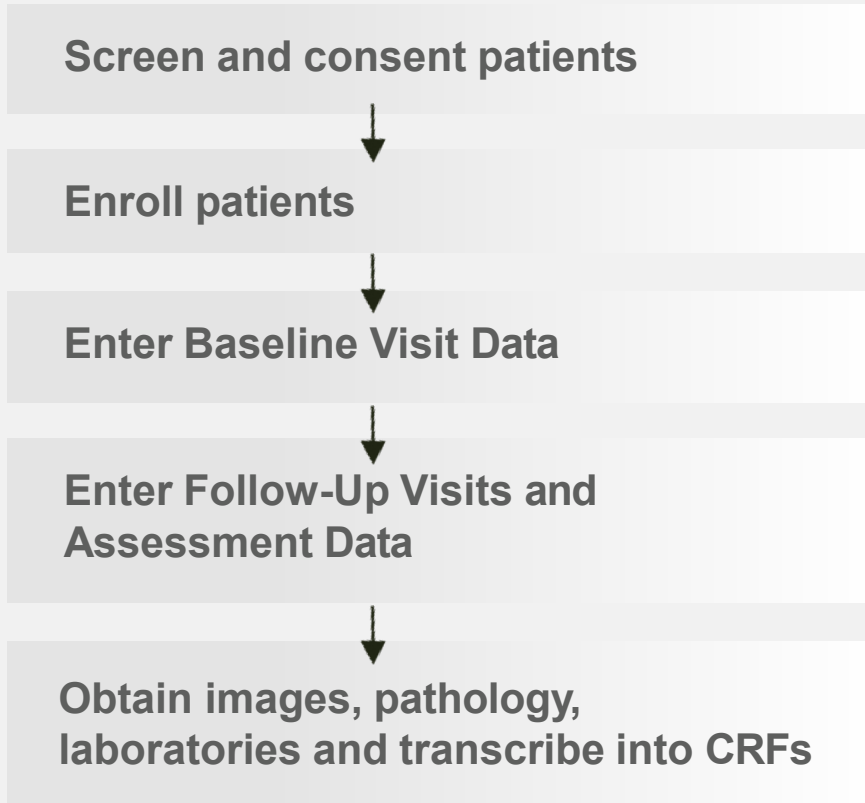
## Novel Therapies Complexity

New molecular entities and mechanisms of action need ongoing evidence for treatment sustainability and emerging safety concerns

# Traditional Data Collection



# “Automated” Data Collection



**Passive data from EHR retrieval accounts for most of the data** including encounters, notes, labs, pharmacy, ancillary systems, images, pathology reports, tumor registry and outcomes as available



**Active data from consented patients** including ePROs & eCROs and specialized assessments not routinely collected (similar to traditional approach)



**Linked Data:** Medical and Pharmacy Claims, SDoH, Mortality, etc.

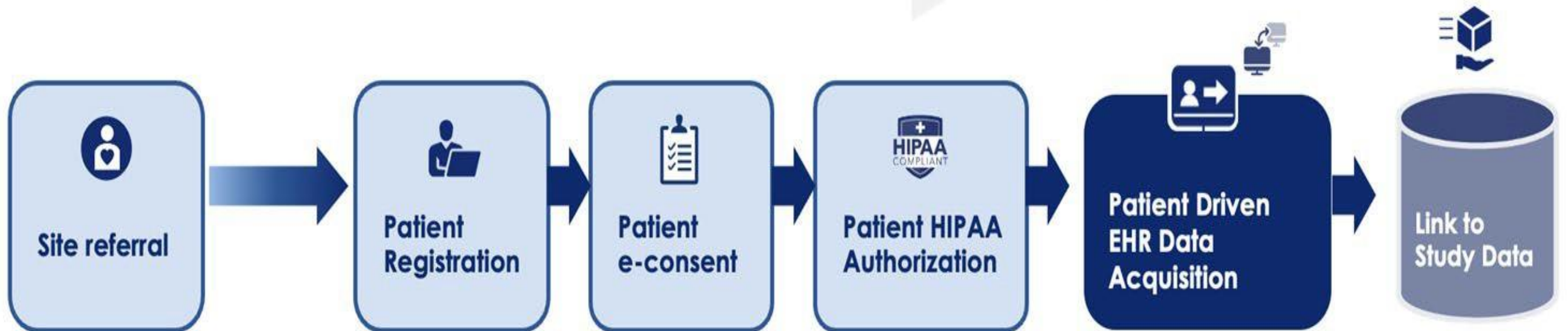
## Virtual Site Workflow

Sites provided with QR code to distribute to eligible patients

Patient or caregiver registers, enrolls, and consents to the study

Site team retrieves patient's EHR via their digital portal or network request

Deliver as validated real-world dataset





# AI-Powered Hybrid Data Collection Approach

## Active Data Collection

**Patients are consented for ePROs and eCROs following procedures similar to traditional registry protocols**

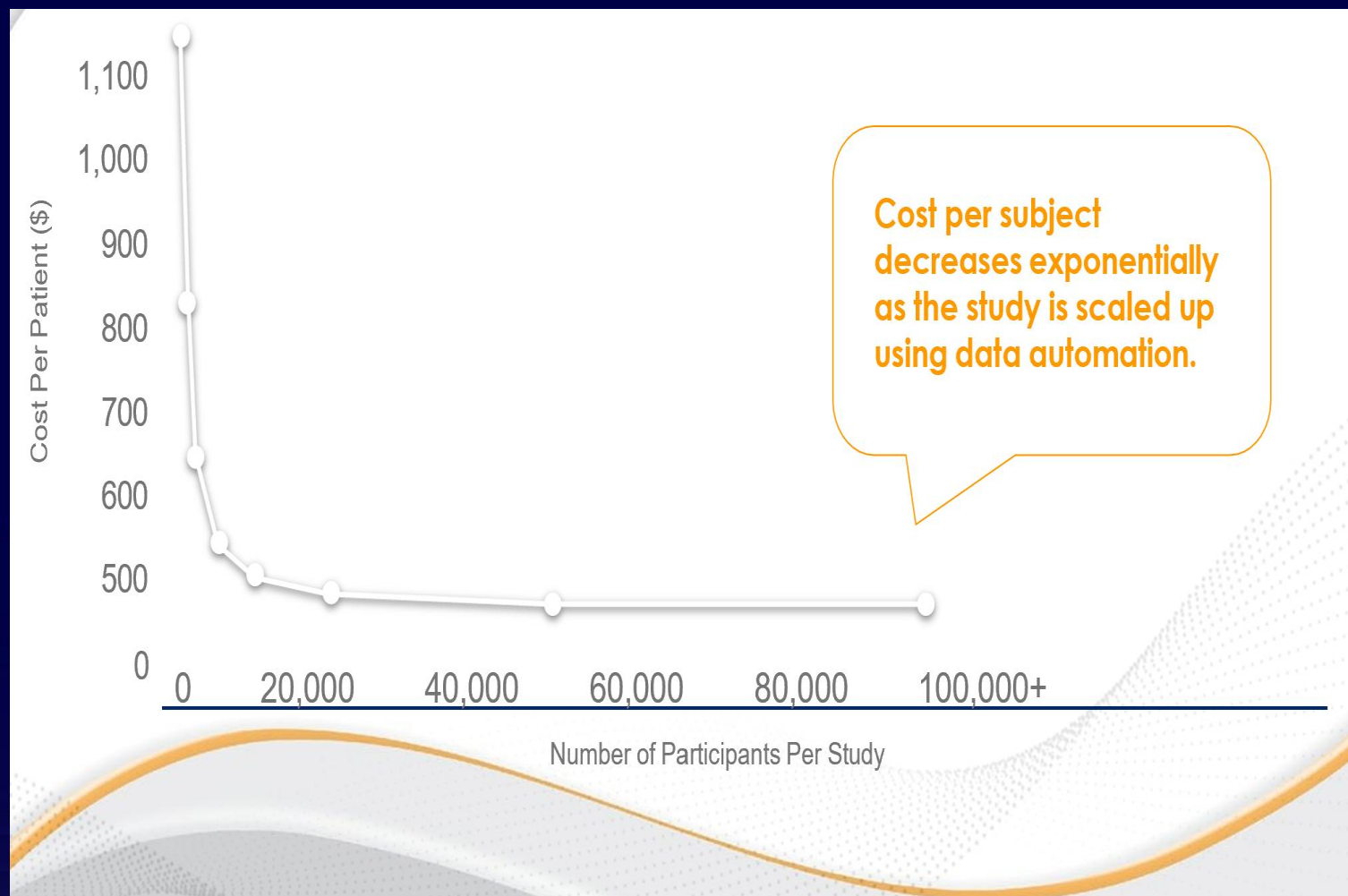
## Passive Automated EHR Extraction

**Agentic AI connects to site EHRs to extract all relevant elements including encounters, clinical notes, laboratory results, imaging reports etc in an automated fashion**

## AI-Driven Unstructured Data Processing

**Medical language processing extracts information from free text and estimates clinical endpoints such as eSLEDAI, eCDAI, ePHQ-9, and eEDSS, with rigorous validation performed by clinicians**

# What About The Cost?



# What About Regulatory?

- Postapproval Methods to Capture Safety and Efficacy Data for Cell and Gene Therapy Products (Draft Guidance for Industry)
- Use of Electronic Health Record Data in Clinical Investigations (Guidance for Industry)
- Considerations for the Use of Real-World Data and Real-World Evidence to Support Regulatory Decision-Making for Drug and Biological Products (Guidance for Industry)
- Real-World Data: Assessing Electronic Health Records and Medical Claims Data To Support Regulatory Decision-Making for Drug and Biological Products (Draft Guidance)
- Data Standards for Drug and Biological Product Submissions Containing Real-World Data (Guidance for Industry)
- Real-World Data: Assessing Registries to Support Regulatory Decision-Making for Drug and Biological Products (Guidance for Industry)
- Guidance for Industry: Electronic Source Data in Clinical Investigations
- Real-World Evidence: Considerations Regarding Non-Interventional Studies for Drug and Biological Products (Draft Guidance)

“The use of AI, including to facilitate data collection... are really transforming the way drugs are developed and used.”

M. Khair El Zarrad, Ph.D., M.P.H, Director, Office of Medical Policy, CDER | FDA, 2024



<b>~600,000 Patients</b>	<b>~10 Sites</b>	<b>Site NPS: 87.5</b>	<b>3 Years Follow-Up</b>
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Case study from OM1, Inc.

## Challenges

To evaluate the clinical performance of an approved testing device for cancer screening using RWD

- Multi-center retrospective
- Required unstructured data extraction including test results with comparator, biopsy details, pathology

## Solution

- Largest, representative cohort of patients receiving test with comparator cohort submitted to FDA
- Careful matching of sites as well as patients to ensure comparability.
- Extensive validation package
- Traceable, Auditable, Transparent
- Determined “Relevant and Reliable”

# AI is here to stay....



Healthcare IT news

AI-driven diagnostics outperform traditional methods in early disease detection.

Dec 14, 2025



Regulatory focus

FDA Advisory Committee Advances Final Guidance on AI/ML in Mental Health Applications

Nov 06, 2025



Techcrunch

Smart glasses & wearables: the next frontier for AI-augmented reality.

June 10, 2025



Harvard business review

The future of work is human-centric but AI-integrated: adapting to the shift.

Jan 05, 2026



Nature medicine

New AI models predict chronic disease progression with 95% accuracy.

Jul 01, 2025



AI insider

Alibaba unveils new AI model Tongyi Qianwen to automate complex tasks.

Jul 04, 2025

**THE FUTURE IS  
NOT AI VS. HUMANS—  
IT' AI + HUMANS**



# Q&A

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