Democratizing Real-world Patient Centered Outcomes Research

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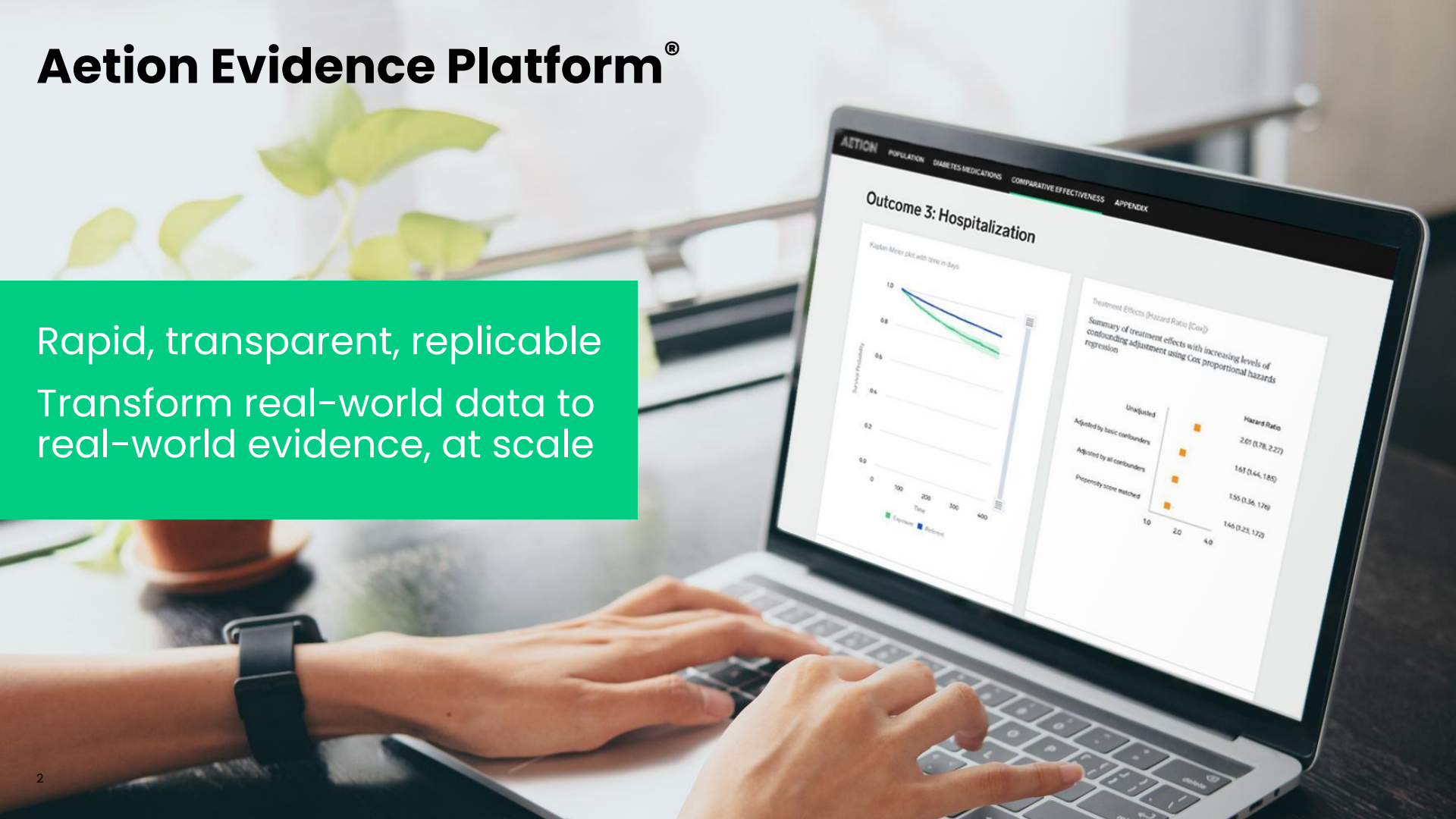
May 2021

Aetion Evidence Platform®

Rapid, transparent, replicable

Transform real-world data to real-world evidence, at scale



Nirosha Mahendraratnam Lederer, PhD
Director, RWE Strategy

- At Aetion, develops strategies for optimizing real-world evidence generation across the product life-cycle to advance regulatory, payer, and provider decision-making
- Led RWE portfolio at the Duke-Margolis Center for Health Policy, including RWE Collaborative
- Previously was a Patient Outcomes SME at the US FDA Oncology Center of Excellence and Manager of HEOR at Avalere Health
- PhD in Health Outcomes and Policy from the UNC Eshelman School of Pharmacy, MSPH from the Johns Hopkins Bloomberg School of Public Health, BA Public Health from the Johns Hopkins University

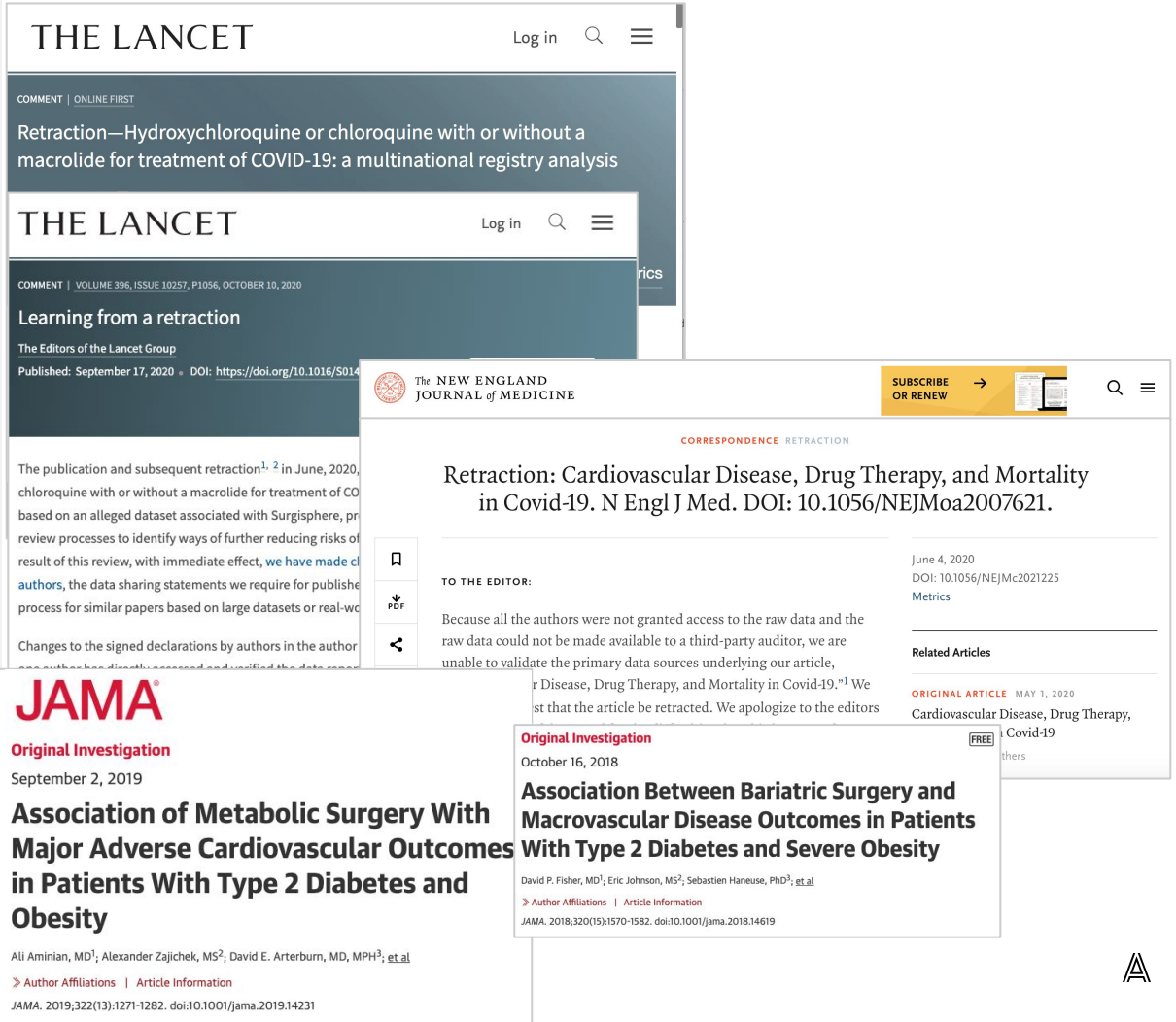
Different types of RWD have different purposes

	Data type				
	Medical + pharmacy claims	**Hospital chargemaster**	**EMR**	**Labs**	**Patient-generated Health Data**
Original purpose of RWD	Collected for billing	Collected for billing	Collected for patient care	Collected for patient care	Collected for patient care/well-being
Source of RWD	Closed and open claims coming from clearing houses or provider revenue cycle management systems	A comprehensive list of a hospital's products or procedures and services	EMR system vendor or data providers	Clinical laboratories	Self-reported mobile health apps Sensors Wearables
Key information captured *(examples)*	Diagnosis codes, National Drug Codes, and procedure codes; enrollment files in closed claims for continuous observability	Visit-level diagnoses, procedures, and specific drug/product revenue codes and discharge status by day of stay or OP visit	Medical record information detailed for the appointment, strong demographic information	Test order, the results, and interpretation if required; outpatient information only	Patient- reported outcomes, physical function, mental well-being, daily activities
Inpatient (IP) versus outpatient data (OP)	IP acute and subacute stays; OP physician and facility services claims	IP and OP information	OP information	OP information only	Select IP/OP information for trial participants

Linkage across sources

Data Collection vs. Data Accessibility

Data, study quality, and transparency are paramount to build trust with decision-makers and the public



Study considerations: Data Fitness and Principled Epidemiology

Ensure data is fit-for-purpose

- Identify minimum criteria needed (e.g., sample size, availability of key variables)
- Identify all possible data sources
- Narrow data sources using min. criteria
- Conduct data feasibility assessment of candidate data sources; rank data
- Consider operational considerations (timeliness, budget)

Ensure principled epidemiology

- Articulate research question
- Describe study elements
  - study population
  - treatment and comparator groups
  - outcomes
  - length of follow-up
- Conceptualize a target trial
- Draw causal diagram for treatment-outcome relationship
- New user design
- Address potential biases and confounding

Real-world PCOR toolbox can organize existing tools to facilitate their adoption

Jaksa A. et al. Organized Structure of RWE Best Practices: Moving from Fragmented Recommendations to Comprehensive Guidance *Journal of Comparative Effectiveness Research. June 2021.*

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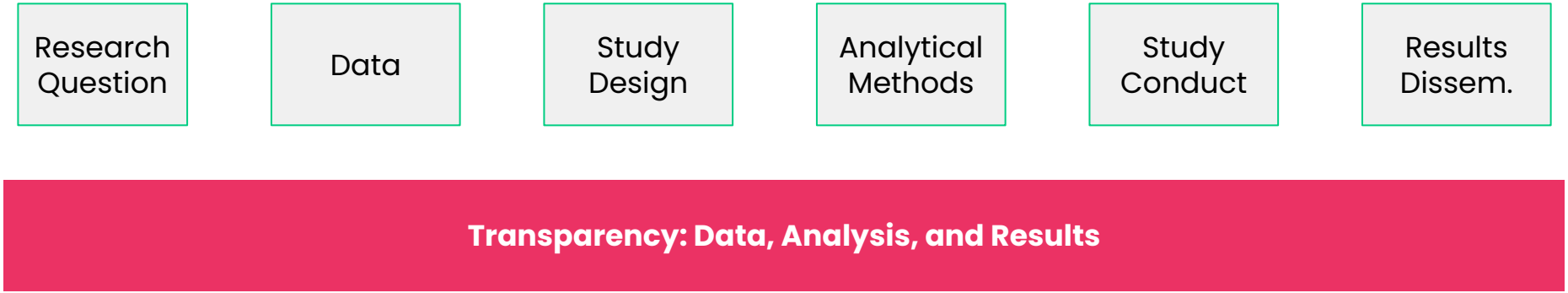
Transparency is essential for building credibility to advance the science of real-world PCOR



Jaksa A. et al. Organized Structure of RWE Best Practices: Moving from Fragmented Recommendations to Comprehensive Guidance *Journal of Comparative Effectiveness Research. June 2021.*

Democratizing PCOR by making high quality data and methods accessible to all researchers

- **Collect and make available relevant RWD**
  - RWD quality starts at point-of-entry
  - Requiring a minimum set of data elements can enable linkages and more meaningful research
  - Today's primary data is tomorrow's secondary data
- **Enable high quality research generation through the adoption of existing research tools through a real-world PCOR toolbox**
  - Assess the landscape of available tools, and map them to the research process to reduce redundancies and avoid duplicating work
  - Identify outstanding gaps and invest in tools to address those gaps
- **Build and incentivize a culture of transparency and trust to advance the credibility and science of PCOR**
  - Science is not proprietary
  - Cross-disciplinary collaboration is essential for high-quality research

Questions?

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Special acknowledgements to Alecia Slade Clary, Drew Roberts, Emily Rubinstein, Liz DeMatteis, Morgan Romine, Stacie Dusetzina, & Wendy Turenne