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Consult the Data
Precision Analytics for Biomedicine

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Big Data in Medicine

- Today across the world, enormous transformations are taking place in health care.
  - What has become very clear… is that the greatest force behind these trends is data.
  - "We're bringing together diverse kinds of data that we haven't had available to us before that will help us address questions about care and about the determinants of health."
- Data is growing— and flowing—across our health care system faster than ever before.
- New technologies and industry players are taking medical knowledge from a human scale to a digital scale.

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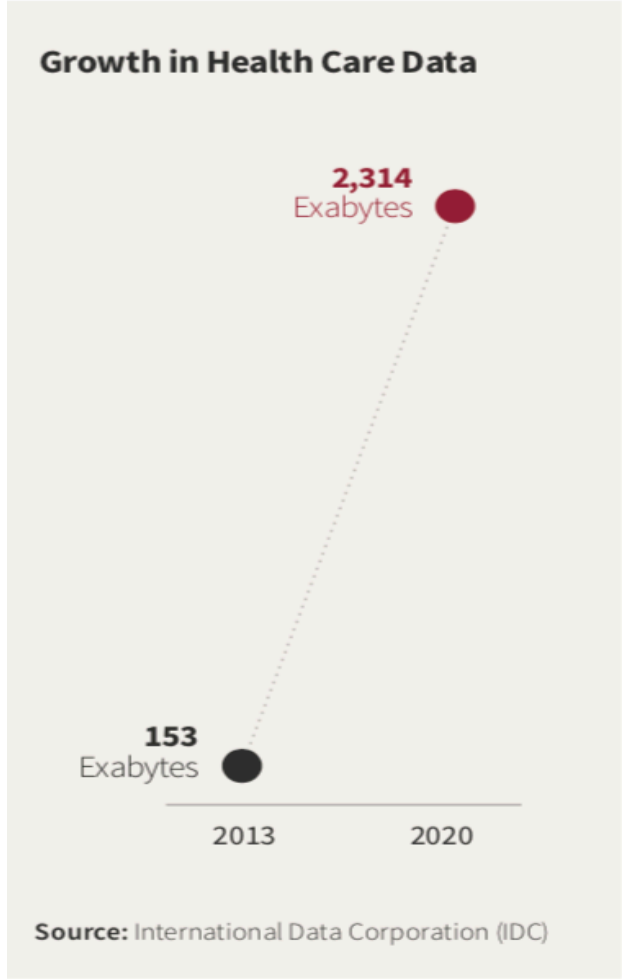
Emerging Big Data Biosphere



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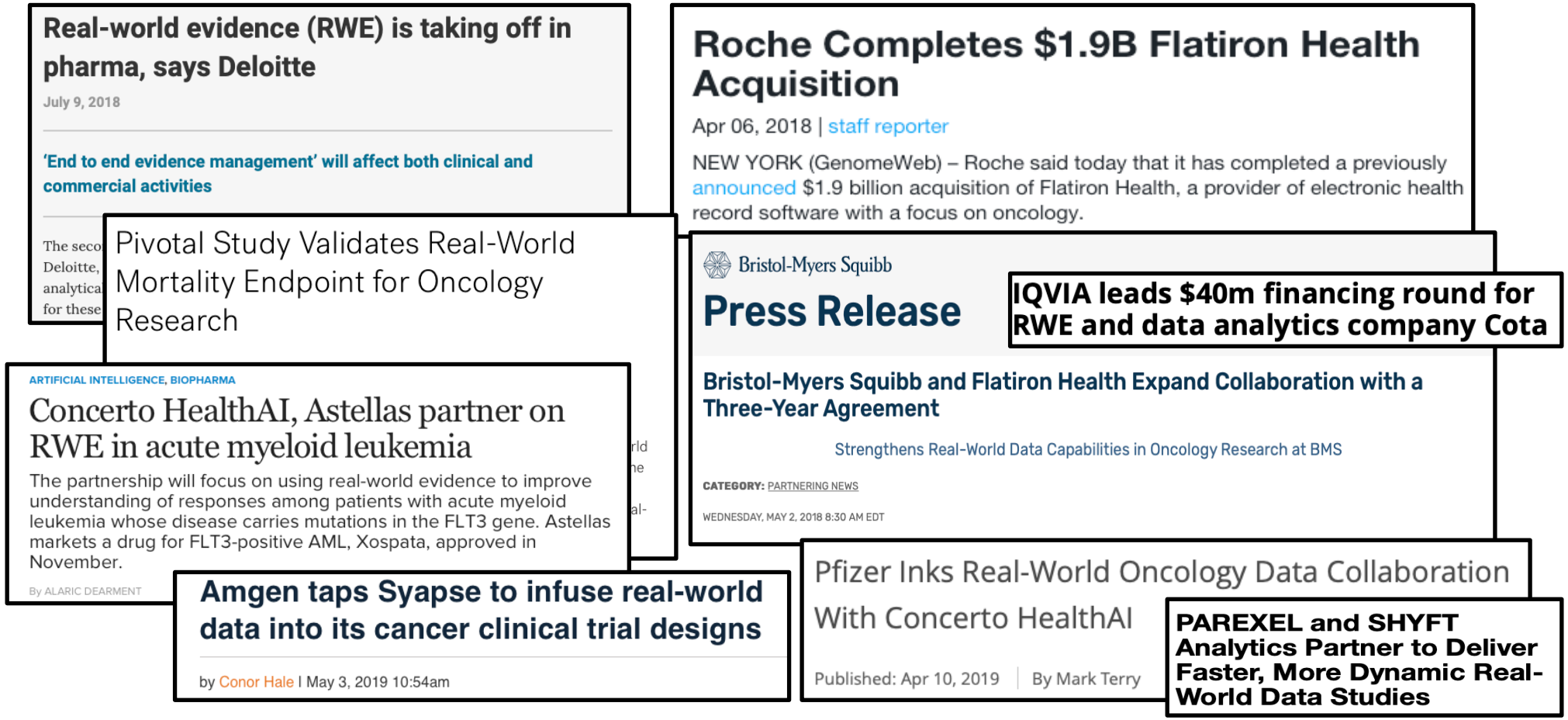
Transforming Industry



Regulators – Advocacy for Real-World Evidence

Submitting Documents Using Real-World Data and Real-World Evidence to FDA for Drugs and Biologics

Guidance for Industry

DRAFT GUIDANCE

Accelerating development of scientific evidence for medical products within the existing US regulatory framework

Rachel E. Sherman[1], Kathleen M. Davies[1], Melissa A. Robb[1], Robert M. Califf[1,2]

Growing access to diverse 'real-world' data sources is enabling persistent evidence gaps about the optimal use of medical prod Here, we argue that contrary to widespread impressions, existin sufficient flexibility to accommodate the emerging tools and met

Framework for FDA's Real-World Evidence Program

Real World Evidence

How FDA, Pfizer, and Flatiron Health did it

Approval of Ibrance for men affords a glance at use of real world data

By Paul Goldberg

d a role in FDA's recent decision to expand the indications for Pfizer's drug to include men.

Pivotal Study Validates Real-World Mortality Endpoint for Oncology Research

EW YORK, NY, May 14, 2018

Today, Flatiron Health, a market leader in the curation of regulatory-grade real-world data for cancer research and real-world evidence (RWE) generation, announced the publication of a pivotal study validating the quality of Flatiron's mortality endpoint and illustrating the potential impact for research conducted with contemporary real-world datasets.

FDA cancer office taps Syapse for real-world evidence development

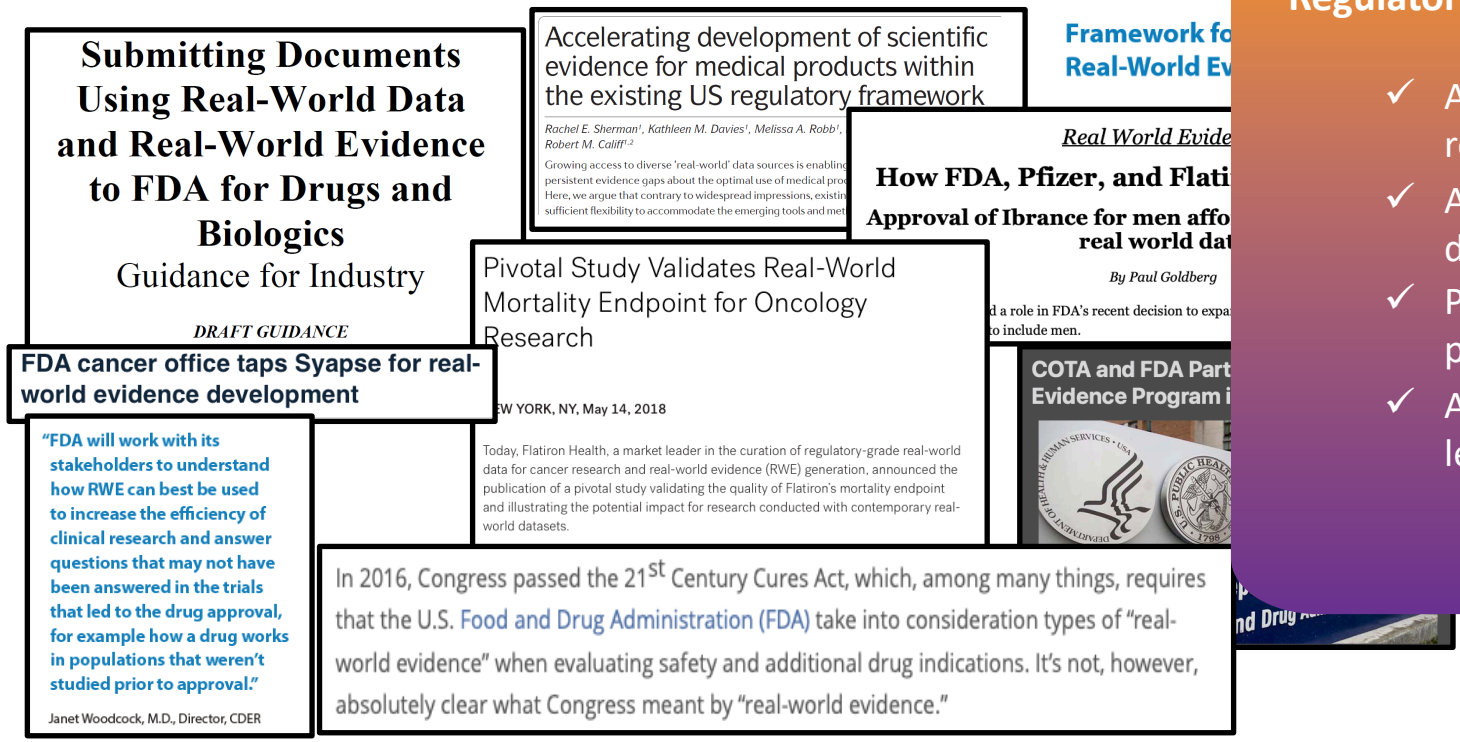
"FDA will work with its stakeholders to understand how RWE can best be used to increase the efficiency of clinical research and answer questions that may not have been answered in the trials that led to the drug approval, for example how a drug works in populations that weren't studied prior to approval."

Janet Woodcock, M.D., Director, CDER

COTA and FDA Partner on Real-World Evidence Program in Breast Cancer

In 2016, Congress passed the 21st Century Cures Act, which, among many things, requires that the U.S. Food and Drug Administration (FDA) take into consideration types of "real-world evidence" when evaluating safety and additional drug indications. It's not, however, absolutely clear what Congress meant by "real-world evidence."

Regulators – Advocacy for Design Innovation



Regulatory landscape has evolved

- ✓ Approvals small trials w/ refractory subpopulations
- ✓ Advocacy for innovations in design related to precision med
- ✓ Pathway for rapid expansion of phase 1 with seamless design
- ✓ Advocacy for data sharing and leveraging external evidence

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Precision Analytics is not limited to Real World Data

cdisc

- ✓ Unlock external databases for decision-making
- ✓ Characterization of heterogeneity (clarify the playing field; identify who can be averaged?)
- ✓ Counter-factual reasoning (who did we actually enroll? to what extent did we beat historical expectations?)
- ✓ Project future success of confirmatory trials of various design (how much response to demonstrate OS?)
- ✓ Enables SIMPLE sequential trials devised with appropriate eligibility, stratification, and prospective avenues for expansion (guided by Bayesian exchangeability)

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Big Data in Medicine

- Data doesn't do you any good until you can turn it into information, and that is really our challenge.
- ... there's a whole new set of jobs emerging around a health care tech skillset that is very different than it was even just 5 years ago.

- This health care democratization is characterized by two major factors: the distribution of data and the ability to generate and apply insights at scale.

- The biggest problem is that our data are not prepared in a way that allows us to even make sense of it. Once the data are readily analyzable, frankly, the majority of the critical clinical questions can be addressed.

  - Amy Abernethy, former Chief Medical Officer/Chief Scientific Officer & SVP Oncology, FlatironHealth; current Principal Deputy Commissioner for Food and Drugs

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Experts in Industry Agree

AI in Biopharma Slowed by Challenges Involving Data, Corporate Culture

By **Alex Philippidis** - May 15, 2019

As companies address AI bottlenecks, Durvasula of Eli Lilly said, they will be best able to integrate the technologies into their drug discovery and development efforts.

"My hope is that in the next decade, we're going to shift to a compute-first research environment, a model-first research environment, rather than run as many of these experiments as you can, and then do the modeling and figure out what the heck just happened," Durvasula said. "It's got to be a compute-first or a model-first research environment. That requires focusing everybody in the room with all their skills, and with all the multi-domain skills even, on the common purpose, the common scientific question."

AI in drug discovery is overhyped: examples from AstraZeneca, Harvard, Stanford and Insilico Medicine

In this craze, lots of pharma/biotech companies and investors wonder whether they should jump on the bandwagon in 2018, or wait and see.

Not so elementary, Watson: the roadblocks for AI in pharma

By **Chris Lo**

SHARE

Machine learning systems such as IBM's Watson can fail if the data that 'teaches' them isn't carefully curated. Credit: IBM

RECOMMENDED COMPAN

KENX **Validation Univers**

Knowledge Exchange (KENX) has announc their Validation Univ

ABINGDON **Abingdon Health**

Abingdon Health is a innovative, high-volu mHealth and point-o (POC)...

The AI hype bubble

The attractiveness of the proposition has been borne out in the stacks of pharma and biotech investment that has been flowing towards AI drug discovery tech and machine learning-focused start-ups in the last few years. From Merck's AI partnerships with Numerate and Atomwise to GlaxoSmithKline's $43m collaboration with Exscientia and the rise of AI-centric scientific innovators such as BenevolentAI, pharma AI has become a lucrative business, even before substantial evidence of its impact on drug discovery has been fully explored.

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Panel Identifies Hurdles to Wider AI Use in Diagnostic, Rx Development

By **Alex Philippidis** - May 15, 2019

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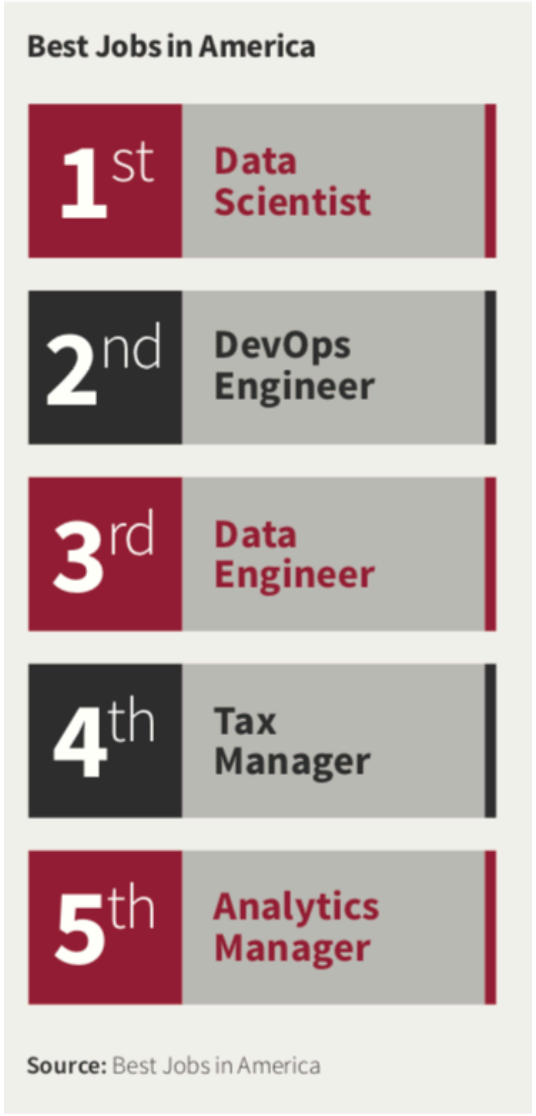
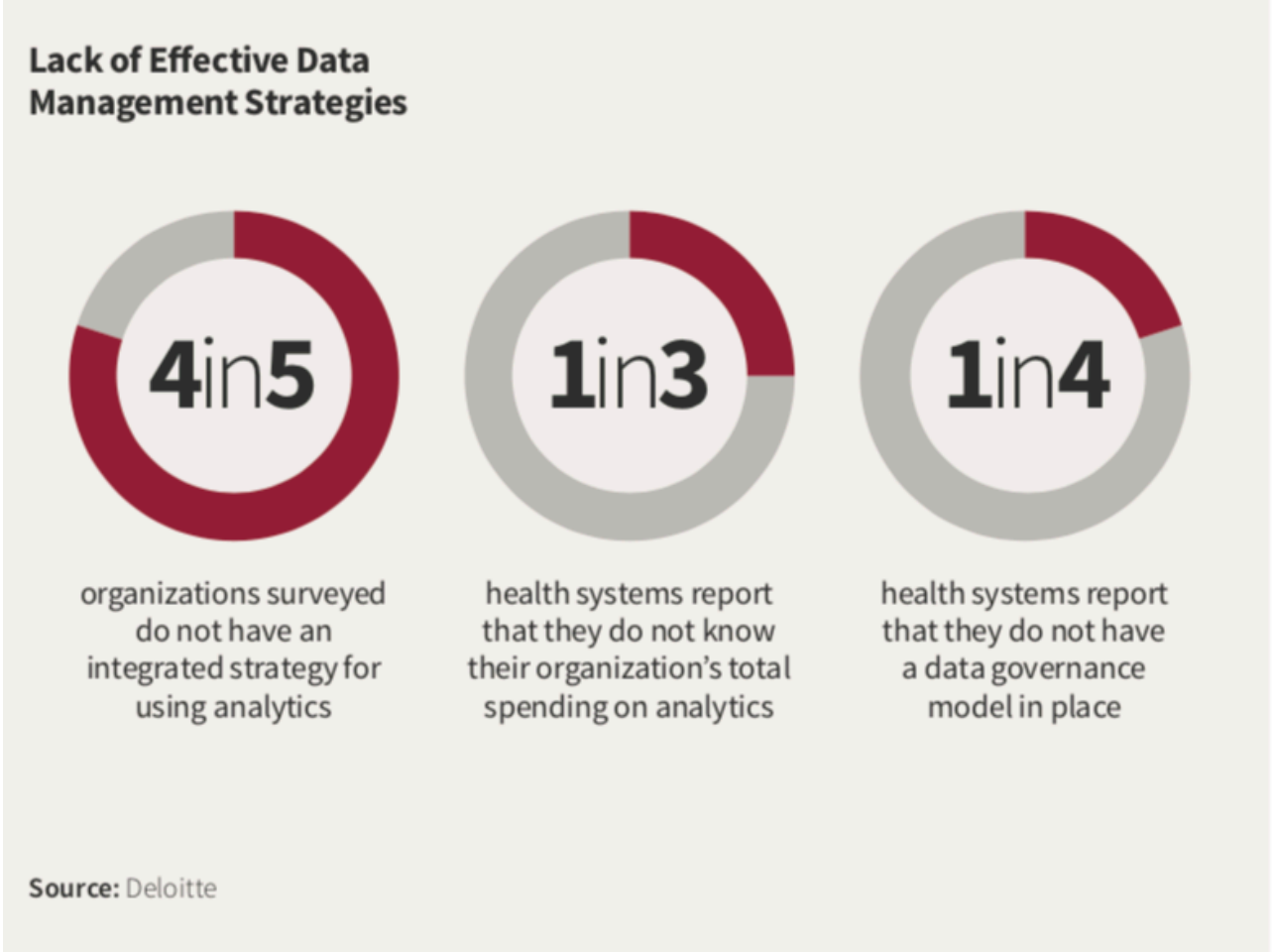
Expertise Is Key To Avoid Overhyped Claims





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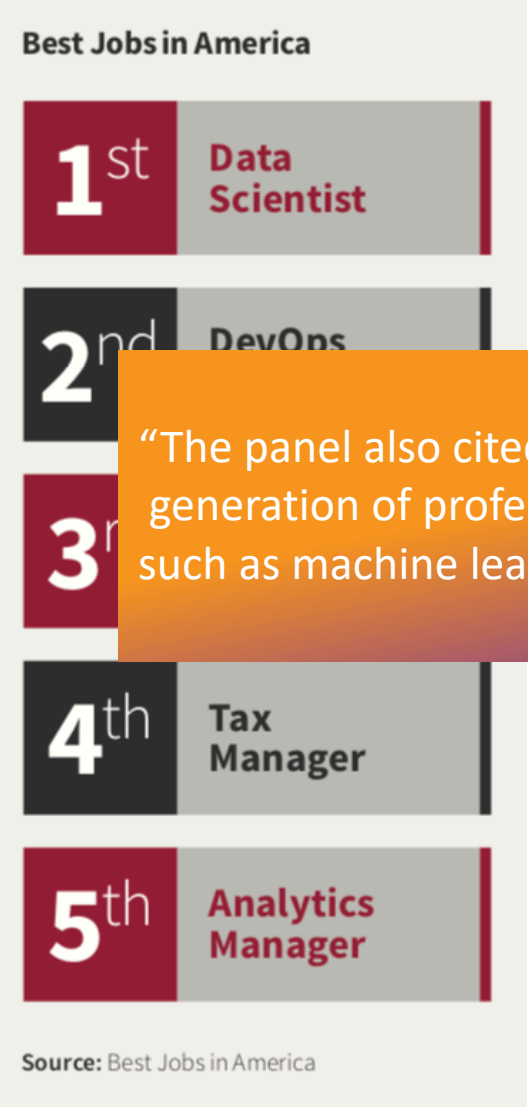
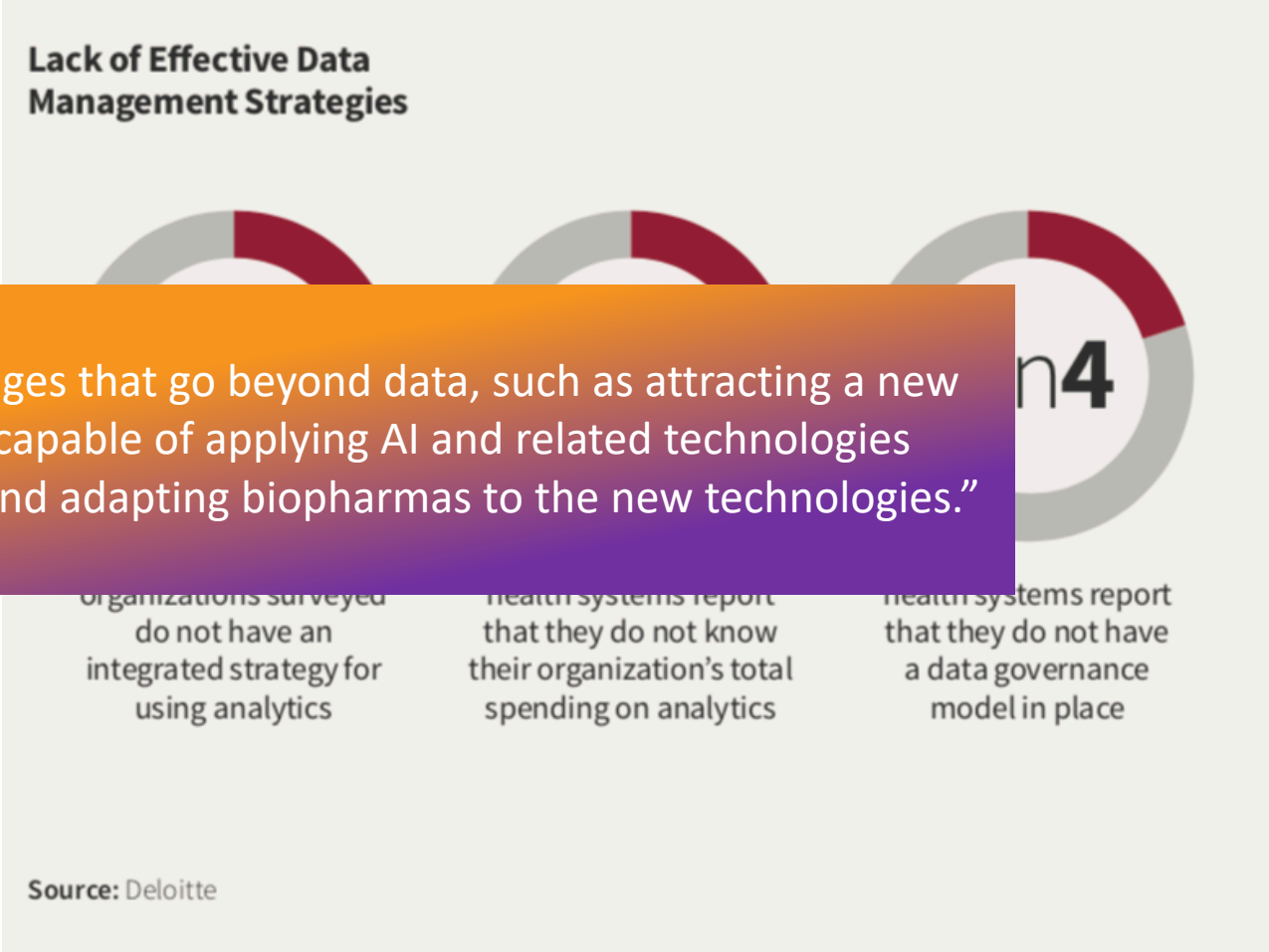
Expertise Is Key To Avoid Overhyped Claims





"The panel also cited challenges that go beyond data, such as attracting a new generation of professionals capable of applying AI and related technologies such as machine learning—and adapting biopharmas to the new technologies."

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Our Clients Confirm

Purchased but not used

Collected but not fully utilized

- ✓ Unlock external databases for decision-making
- ✓ Characterization of heterogeneity (clarify the playing field; identify who can be averaged?)
- ✓ Counter-factual reasoning (who did we actually enroll? to what extent did we beat historical expectations?)
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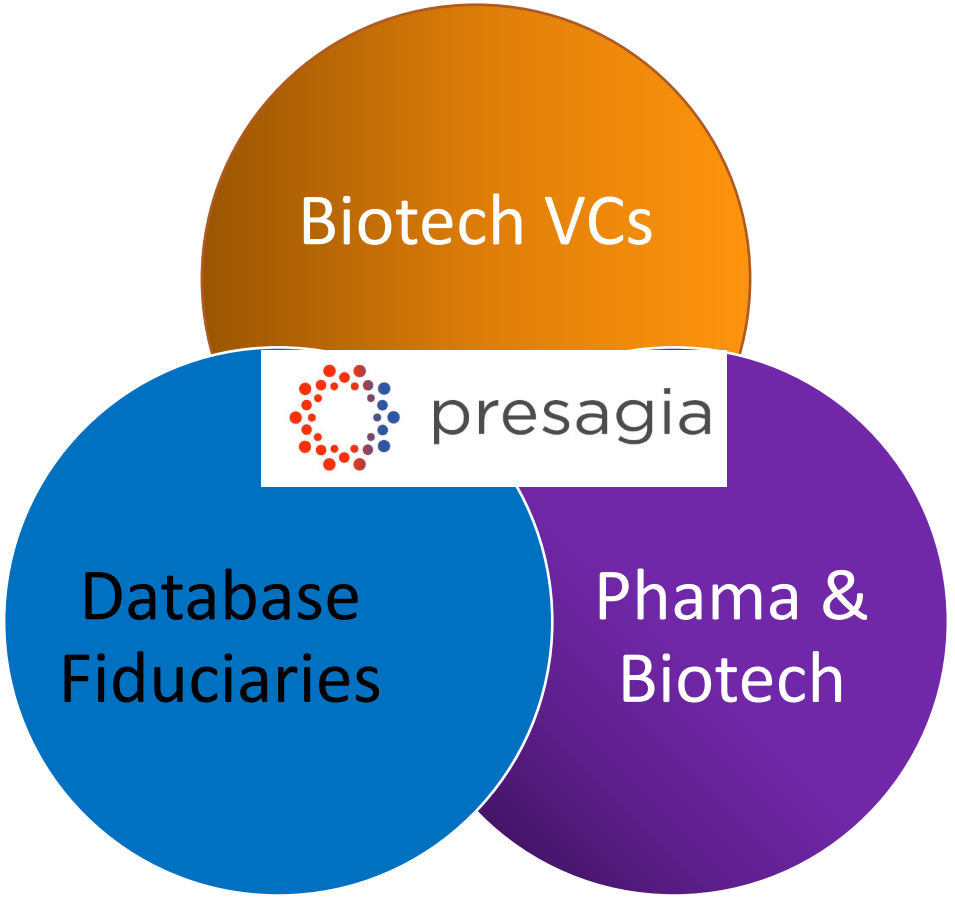
Consult the Data

- **Database Fiduciaries**
  - have asked that we help them monetize
- **Phama & Biotech**
  - Find Data
  - Make use of data
  - Understand data
- **Biotech Venture Capital**
  - Find data to evaluate assets



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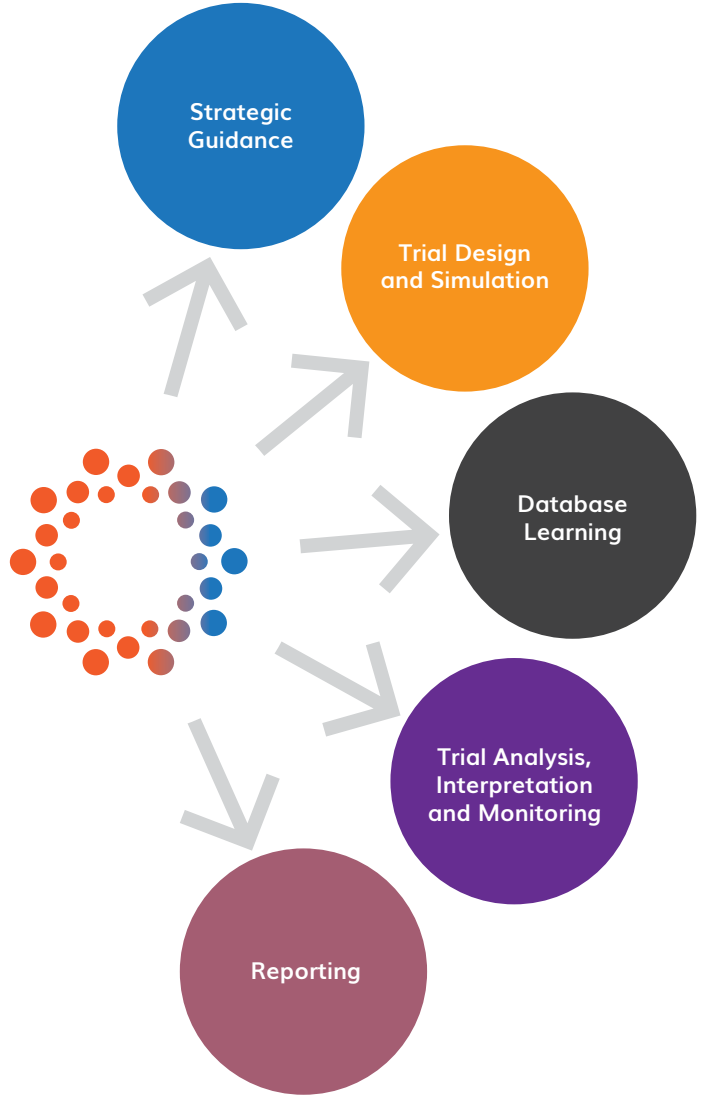
Decision Sciences + Precision Analytics

- Do you use data to make decisions?
- If yes, do you have any data?
- If no, do you know where to get data?
- If you have data, what are you doing with it?



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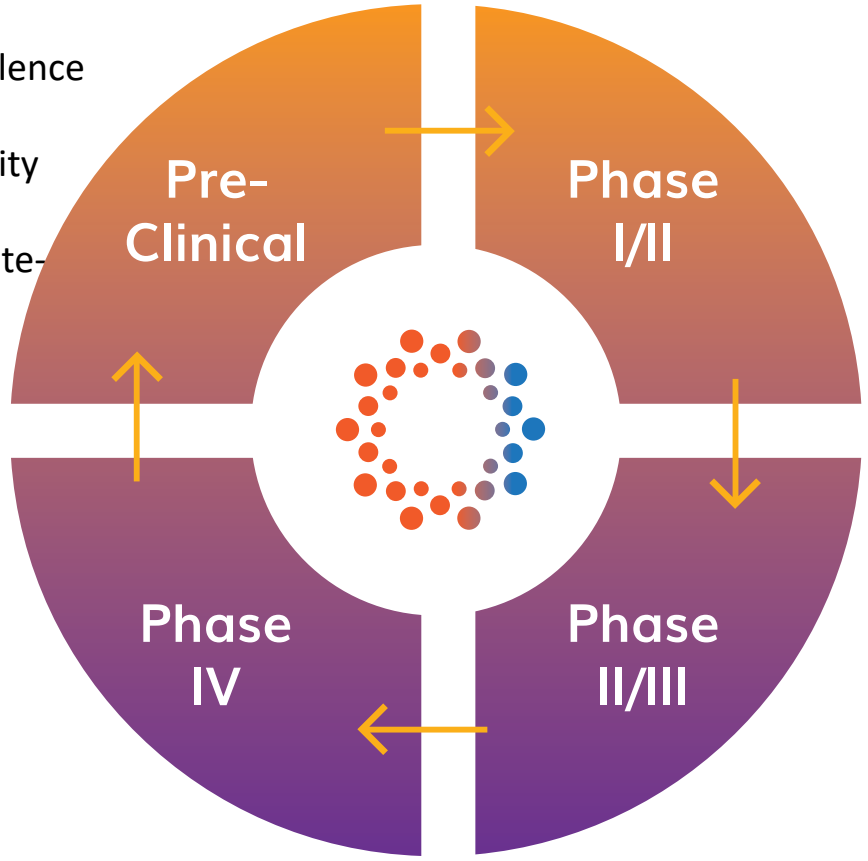
Refined Drug Development Paradigm Aided by Analytics

- Real-world and Historical Evidence
- Estimate Drug Target Prevalence
- Subpopulation Heterogeneity
- Historical and RWE Surrogate-Survival Mediation



- Optimize expansion cohort design, strata, eligibility, and monitoring plan
- Precision Analytics characterize safety profiles
- Quantify extent of trial risk in phase II
- Evaluate histological heterogeneity

- Optimize subsequent development programs
- Discern optimal “patient selection” in relation to competitors
- Surrogate endpoint re-assessment and refinement
- Network meta-analyses

- Formalize Phase III Go/No-Go
- Precision Analytics characterize responder profiles
- Simulate Phase III Survival comparison
- Label expansion strategy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Leadership
Multidomain expertise is the key

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Leadership: Diverse Background

- **Rick Landin, PhD:** President & CEO, 25+ years as a statistician in the pharmaceutical / biotechnology industry. Extensive experience in leading cross-functional and multi-corporation through the **drug approval process**.

- **Mike Kane, PhD**: Chief Data Scientist, Assistant Professor of Biostatistics at Yale University. Expertise in **Big Data and machine learning** with focuses on applications in clinical trials and population-scale human mobility.

- **Adam Sharp, BS**: Chief Financial Officer, Chief Financial Officer, 20+ years as a statistical programming consultant in the pharmaceutical industry. Chief Executive Officer of SimulStat Incorporated. Experience providing **efficient, scalable operational and financial systems** to support growth and profitability.

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Leadership: Diverse Background

- **Brian Hobbs, PhD:** Scientific Advisor, Associate Staff and Section Head of Cancer Biostatistics, Cleveland Clinic. Serves as Co-Director of the Biostatistics and Bioinformatics Core for the Case Comprehensive Cancer Center. Former tenured Associate Professor MD Anderson. Expertise in **Bayesian inference, subtyping, prediction, and trial design as well as cancer radiomics**.

- **Jim Welsh, MD:** Scientific Advisor, Head of Immuno Radiation, and Tenured Physician Scientist and faculty member at The University of Texas MD Anderson Cancer Center. **Founder of OncoResponse and Molecular Match**.

- **David Hong, MD**: Scientific Advisor, Associate Professor and Deputy Chair in the Department of Investigational Cancer Therapeutics (Phase I Program) at MD Anderson Cancer Center. **Extensive expertise in drug development**. Research endeavors focus on tissue agnostic therapies and combining targeted therapies with immunotherapies.


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