Emerging Opportunities: Real-World Evidence

The proliferation of real-world data drives startup opportunities and facilitates clinical trials

PitchBook is a Morningstar company providing the most comprehensive, most accurate, and hard-to-find data for professionals doing business in the private markets.

Credits & Contact

PitchBook Data, Inc.

John Gabbert Founder, CEO
Nizar Tarhuni Senior Director, Institutional Research & Editorial

Institutional Research Group

Analysis
KAIA COLBAN Analyst, Emerging Technology
kaia.colban@pitchbook.com
pbinstitutionalresearch@pitchbook.com

Publishing

Designed by **Drew Sanders**

Published on August 16, 2021

Key takeaways

- Real-world evidence (RWE) has the potential to be useful across the entire healthcare ecosystem, including for clinical trials, care provision, determining treatment outcomes, and post-care safety monitoring. However, impact potential is relative to the quality and quantity of data available for analysis.
- We have tracked 57 startups that we view as primarily focused on RWE business opportunities. Since 2019, these startups have raised $1.8 billion in VC and have an aggregate valuation of $16.0 billion (although valuations are not available for many of these companies).
- We forecast the RWE solutions market to grow at a 15% CAGR from $1.1 billion in 2020 to $2.9 billion in 2027.
- Key startups opportunities include building proprietary datasets, developing real-world data (RWD) analytical tools, and creating data standardization and management solutions.

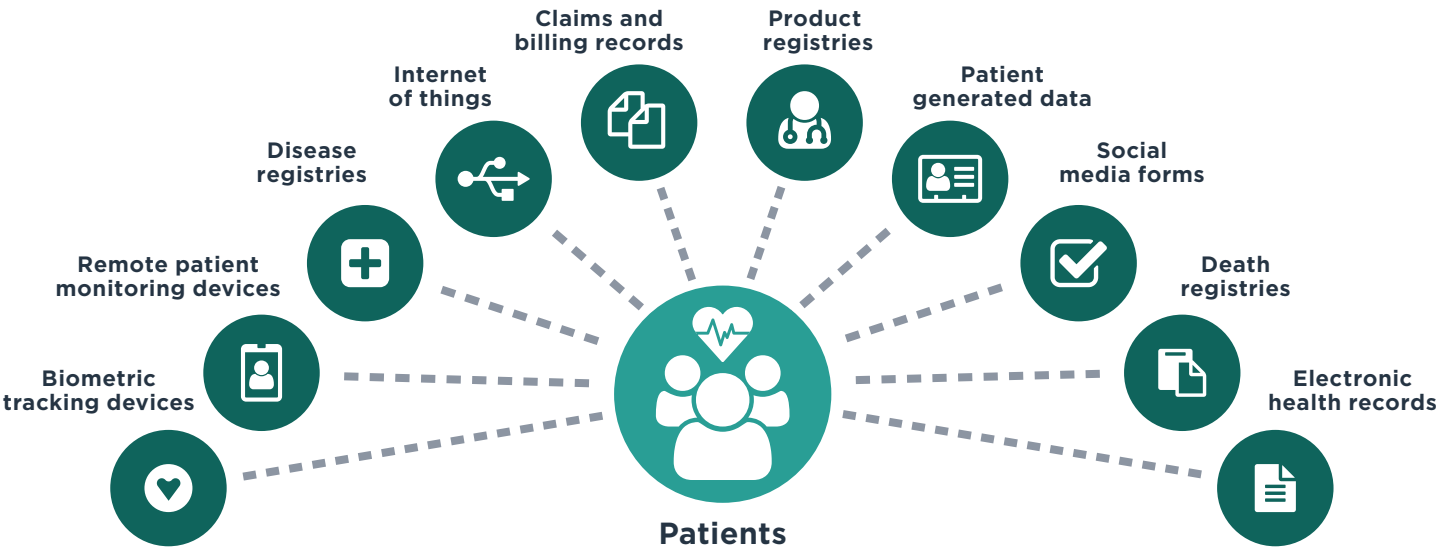
Contents

Introduction

The quantity of routinely collected data has skyrocketed in recent years due to the rise of mobile and digital health apps, computers, electronic health records, biometric trackers, and remote patient monitoring devices. Because patient data related to health status and/or the delivery of healthcare is collected in real time, it is referred to as "real-word data" (RWD). When structured and analyzed, this data can provide real-world evidence (RWE), which the Food and Drug Administration defines as "clinical evidence regarding the usage and potential benefits or risks of a medical product derived from analysis of RWD."[1] This RWD and subsequent RWE has the potential to be useful across the entire healthcare ecosystem, including for clinical trials, care provision, determining treatment outcomes, and post-care safety monitoring. However, impact potential is relative to the quality and quantity of data available for analysis. Startups are emerging to create, aggregate, and standardize data to reveal powerful insights.

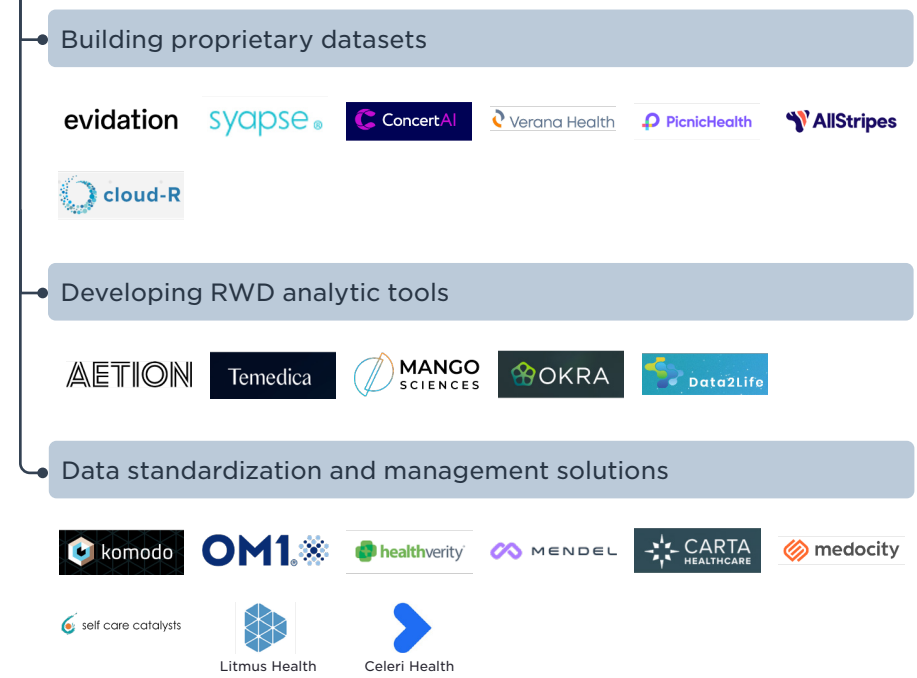
We expect RWE to drive significant opportunities within the healthcare industry. We have tracked 57 startups that we view as primarily focused on RWE business opportunities. Since 2019, these startups have raised $1.8 billion in VC and have an aggregate valuation of $16.0 billion (although valuations are not available for many of these companies). While the startup ecosystem for RWE may be nascent, the regulatory landscape is relatively developed, with frameworks in place designed to improve access to health-related data while ensuring consumer privacy. We forecast the RWE solutions market to grow at a 15% CAGR from $1.1 billion in 2020 to $2.9 billion in 2027.

Types of real-world data



1: "Real-World Evidence," US Food and Drug Administration, n.d. Accessed July 30, 2021.

RWE market map



Leading RWE incumbents
Anthem
Clinigen Group
Cognizant
IBM
ICON
IQVIA
Optum
Oracle
Parexel
PerkinElmer
Pharmaceutical Product Development
SAS Institute
Syneos Health

Source: PitchBook | **Geography:** Global

Regulatory landscape

RWE relies on sharing health data between providers, data companies, and devices. This raises concerns around future regulations on the use of electronic source data, signatures, and health records. Regardless of future regulations, companies must focus on balancing patient privacy protections with advancing data-driven clinical research and care delivery.

In many instances, regulations have likely helped RWE companies. In 2016, the 21st Century Cures Act defined RWE, mandated the launch of an RWE program, and called for the development of regulations that accelerate medical product development and innovation while protecting individual data.[2] In 2018, the RWE Program developed a framework for using RWE to support new indications for already-approved drugs and post-approval studies.[3] More recently, the Office of the National Coordinator for Health Information Technology (ONC) published a new regulation in May 2020 to eradicate electronic health information blocking between health systems, apps, and devices in response to the COVID-19 pandemic.[4] The ONC also calls for increased standardization across application programming interfaces (APIs), which will increase research utilization. In the UK, the GP IT Futures contract, implemented in December 2020, requires electronic health record (EHR) vendors to provide access to standardized APIs and extracts for third-party vendors.[5] Standardized APIs mean data registries do not have to have a direct commercial relationship with the EHR systems and can gain access to data without additional overhead.[6] We anticipate regulations will progress to limit the power of any specific vendor to obstruct access to data by third parties; this is essential to achieving interoperability and developing a thriving ecosystem of tools that can improve patient care.

2: "21st Century Cures Act," US Food and Drug Administration, n.d. Accessed August 5, 2021.
3: "Framework for FDA's Real-Word Evidence Program," US Food and Drug Administration, December 2018.
4: "Information Blocking and the ONC Health IT Certification Program: Extension of Compliance Dates and Timeframes in Response to COVID-19 Public Emergency," Federal Register, November 11, 2020.
5: "GP IT Futures Systems and Services," NHS Digital, n.d. Accessed July 29, 2021.
6: Ibid.

Use cases

Providers, academic researchers, and medical societies

RWE solutions can help drive individualized care decisions by helping providers predict outcomes based on characteristics such as comorbid conditions, genetics, lifestyle, individual and family medical history, and so on. Providers can use RWE to not only compare different treatment methods but determine optimum drug dosages and refine treatment guidelines to best suit individual patients. Academic researchers and medical societies can use RWE to advance understanding of current treatments and diseases by conducting observational, retrospective, and prospective studies.

Company examples

OM1

Founded:
2015

Raised to date:
$176.25M

Employees:
130

Valuation:
$855.0M

OM1 partners with medical societies, providers, and hospitals to collect medical data at a clinical level. The desire to track care quality and apply data to specialty research incentivizes organizations and providers to partner with OM1. In some cases, OM1 pays clinicians for supplemental data, but generally, no fee is exchanged. OM1 primarily focuses on chronic disease in the US but seeks to expand into new geographic regions and specialties.

Verana Health

Founded:
2008

Raised to date:
$138.8M

Employees:
117

Valuation:
$250.0M

Verana Health, a clinical database developer, currently partners with the American Academy of Ophthalmology (AAO) IRIS® Registry (Intelligent Research in Sight), and the American Academy of Neurology (AAN) Axon Registry. These organizations provide Verana with free data; in return, Verana covers all registry operating costs. Physicians and medical societies use these registries to track performance against federal quality measures and submit quality reporting information.

Healthcare organizations

RWE enables healthcare organizations to track their outcomes and benchmark quality measures against peers. Organizations use this information while discussing coverage with payers and developing outcome-based reimbursement strategies.

Company examples

ArborMetrix

Founded: 2011	Raised to date: $29.3M
Employees: 96	Valuation: $36.1M

ArborMetrix develops analytical tools to deliver targeted, clinically deep insights for providers, health organizations, medical societies, and pharmaceutical organizations. Health organizations use the company's software to engage clinicians in outcome improvement and to evaluate and elevate performance.

Pharmaceutical and device companies

Drug discovery: To develop a new drug, researchers must understand how a disease functions over time. RWE allows researchers to observe patient variances concerning coexisting conditions and previous and past treatment responses and collect longitudinal clinical data.

Drug approval: Historically, randomized controlled trials were used to evaluate new interventions. However, random trials have many issues, including homogeneous patient subsets, a lack of diversity, patient recruitment difficulties, and a multi-year timeline before making it to approval by government agencies. While RWE is unlikely to fully replace random trials, it can serve as a complement and is beginning to take on a larger role in drug approval as regulatory agencies develop new legislation and frameworks to encourage its use. The FDA has also indicated an increased willingness to employ simulated control groups, which could expedite clinical trials but potentially shrink the opportunity for patient recruitment startups such as Citrus Lab and Clara.

Post-market evaluation: After drug approval, RWE can be used to monitor post-market safety and adverse events, providing insight into off-label use cases and facilitating strategic marketing decisions. RWE facilitates awareness of patient populations, with the potential to affect how doctors prescribe medication and how pharmaceutical companies launch new drugs. Real-time IoT and regulatory data may also help reveal new market opportunities or provide market intelligence.

Company examples

DotLab

Founded:
2016

Raised to date:
$10.0M

Employees:
16

Valuation:
N/A

DotLab, a molecular diagnostic company, is currently developing DotEndo, an endometriosis diagnostic test. Once DotEndo is available commercially, the company plans to initiate registries to continue evidence generation. The data collected in these registries will evaluate outcomes of DotEndo use (for example, quality of life improvements, impact on healthcare utilization, and ability to achieve a pregnancy), enable DotEndo to be used for additional applications such as routine monitoring, and help DotLab develop new diagnostic and therapeutic products.

uMed

Founded:
2017

Raised to date:
$5.75M

Employees:
14

Valuation:
$9.7M

UMed aggregates data from EHRs into a data repository and then applies data science to help providers select patients for clinical studies. Once selection occurs, the company engages patients on behalf of their recognized provider to fill in medical history gaps and continually gather data throughout the study via patient surveys and medical devices. This allows clinical trial providers to assess patient experiences and symptoms that are rarely captured in the EHR and helps uMed validate if patients have interacted with other physicians/health systems. Finally, uMed also uses this patient access to collect samples that can be linked back to EHR, including using home genomic testing kits. Over the long term, the company hopes to develop a leading prospective repository targeting specific disease areas.

Key startup opportunities

Building proprietary datasets

Startups are leveraging unique business models and data sources by building proprietary datasets. Data collection occurs through the following methods:

- **Consumer-direct healthcare apps:** These consumer-focused platforms allow individuals to store and gain access to all their healthcare data in one place. A core advantage to this method includes minimizing data gaps. While traditional medical records such as EHRs and medical claim data record major events (such as hospitalizations), they often lack clinical-level granularity and long-term patient history. VC-backed companies in this space include Picnic Health and Allstripes.

- **Genetic tests:** Genomic data has become highly sought after. Genetic test providers such as 23andMe, Foundation Medicine, Helix, and EverlyWell are actively building genomic databases.

- **Academic partnerships:** Many academic societies have aggregated large amounts of data for research. Startups pursuing this opportunity include ConcertAI and Verana Health.

- **Biometric devices:** Some startups are developing remote patient monitoring devices and biometric tracking wearables to help individuals track their health. While the device itself may seem like these companies' main products, the real-world data collected can create valuable databases as well.

- **Analytic and EHR software:** Companies are building EHRs that focus on medical specialties or specific healthcare categories to record patient health and billing data. EHRs have larger, more complete datasets relative to many other data sources (such as pharmacy records or RPMs), and data collected from other sources is commonly integrated into EHRs. Analytical software integrates into EHRs to provide additional levels of insight, such as patient management recommendations. EHRs and analytic software benefit from two revenues: the technology itself and the data. These technologies are well-positioned to develop relationships with providers. Key VC-backed companies in this area include Holmusk, Syapse, and Optum.

Related startups

PicnicHealth

Founded:
2014

Raised to date:
$35.1M

Employees:
163

Valuation:
$135.0M

PicnicHealth collects and manages medical records on behalf of patients who seek to manage their medical records in a singular place. It then shares de-identified records with researchers. PicnicHealth charges patients a one-time retrieval fee of $299 and then $39/month for upkeep, though the company waives the fee for patients involved in clinical trials.

Developing RWD analytical tools

Startups, such as Mango Sciences and Aetion, are developing business intelligence tools to help life sciences companies analyze and derive insights from RWE. These startups may build the database themselves, similar to proprietary database companies, or leverage third-party databases.

Related startups

MANGO SCIENCES

Founded:
2013

Raised to date:
$7.5M

Employees:
37

Valuation:
$17.5M

Mango Sciences is a healthcare fintech company focused on increasing access to healthcare in emerging markets where the patient is largely responsible for healthcare costs. Its personalized medicine analytics platform enables providers to determine which patients will benefit from immunotherapy, the company's current focus area. Once selected, Mango Sciences connects patients with banks to receive loans for treatment. If the patient is not treated, Mango Sciences pays back part or all of the loan on behalf of the patient. The company builds its data registry, which drives its analytical software from data gathered from hospitals before, during, and after treatment. Mango Sciences generates revenues exclusively from pharmaceutical companies that pay for increasing access to new markets.

AETION

Founded:
2012

Raised to date:
$203.6M

Employees:
185

Valuation:
$580.0M

Aetion enables biopharma companies and payers to engage in value-based care by analyzing RWD to provide answers regarding treatment outcomes and costs. It partnered with the FDA on a three-year study to recreate 30 randomized clinical trials through RWE. It sits as a neutral third party between drug developers and payers that wish to better understand a drug's efficiency for reimbursement purposes.

Data creation, standardization, and management solutions

Startups are developing artificial intelligence & machine learning (AI & ML) technology to extract and structure RWD. RWD is often captured in ways that limit accessibility; for example, patient symptoms notes lack standardized language, and differing EHR formats between providers create difficulties linking patient data. These startups often gain access to the data by helping insurers, clinical researchers, and health providers manage, analyze, and de-identify the data. Given the challenges of data standardization and interoperability of data sets, startups are leveraging and developing AI, natural language processing (NLP), and robotic process automation (RPA) technology. NLP can help extract relevant data from patient charts; however, medical NLP is more complex than standard NLP due to complex verbiage, medical notation, and varying medical notation. Thus, medical NLP tools are in preliminary stages and often specializes in a particular disease. Startups in the space, such as Datavant, Data2life, and HealthVerity, help these organizations manage data and create data exchanges where customers can link their data with other sets.

Related startups

komodo™ HEALTH

Founded:
2014

Raised to date:
$327.5M

Employees:
415

Valuation:
$3.3B

Komodo cross-links patient data from over 150 payer datasets with clinical encounters to develop a real-time healthcare map that facilitates payer, provider, and pharmaceutical access to unified, longitudinal patient encounters. It leverages AI to anonymize and link data from over 320 million patients across siloed data sources.

DATAVANT

Founded:
2017

Raised to date:
$83.0M

Employees:
98

Valuation:
$7.0B

Datavant receives information directly from healthcare providers and institutions that share information for de-identification and/or storage purposes. The company then synthesizes and links the data to derive insights. Datavant announced a partnership with Paraxel in October 2019 and acquired CIOX health in June 2021.

Outlook

Over the short term, startups will focus on building disease-specific data registries; a few data bureaus will own the market in the long term: Startups will likely focus on the most monetizable datasets. These include those focused on illnesses that attract large investments related to drug development or those that have highly variable patient populations and treatments. VC-backed companies with specialty focuses include Holmusk (behavioral health), Verana Health (ophthalmology and neurology), Mango Sciences (oncology), UMed (neurology), and AllStripes (rare diseases). Over the long term, we believe these registries will merge and a few healthcare data bureaus with cross-disease data will dominate the market. These data registries may one day become publicly available as governments, health associations, and hospital systems are actively working to increase access to data. However, we foresee companies will continue developing analytical tools that enable researchers to derive insights from healthcare data.

AI & ML will be key to gleaning information from large datasets: Making sense of the growing quantities of RWD will likely require sophisticated analytics, which could result in extensive use of AI & ML techniques suited for processing and deriving insights from exponential data volumes. This could be a growth-limiting factor to the extent startups can develop and sustain competent in-house AI processes. We expect startups could pursue various business models, including the development of tools and platforms that help external researchers analyze proprietary datasets, as well as startups that use their data to generate internal insights to sell to customers. We view the former as a potentially larger market opportunity.

Pharmaceutical companies to bring RWE in-house: According to a Deloitte study, 70% of pharmaceutical companies are planning to bring RWE analysis functions in-house.[7] This could drive exit opportunities for startups to the extent that large incumbents pursue acquisition strategies.

RWE will increase the personalized medicine market: Personalized medicine is estimated to be a roughly $200 billion market consisting mostly of genomics and precision medicine.[8] While several startups focus on opportunities within personalized medicine, incumbent payers and providers are largely hesitant to adopt and invest in these technologies. However, we expect RWE may catalyze the development of more personalized medicine and foresee this industry could experience significant growth over the next decade.

Decreased demand for patient recruitment solutions: In double-arm randomized trials, patients are assigned a placebo. This increases the number of patients required for the trial, raises ethical concerns, and creates a barrier to patient recruitment and retention. RWE can create synthetic control arms that are comparable to traditional control arms. We believe RWE will increase trial retention and ease the recruitment process. As a result, the need for patient recruitment and retention solutions may decrease.

7: "Mission Critical: Biopharma Companies are Accelerating Real-World Evidence Adoption, Investment, and Application," Deloitte, Brett Davis, et al., 2018.
8: Rich Gliklich, phone interview with Kaia Colban, July 20, 2021.