Accelerating Decentralized Real-World Research

Smart Omix for Pharmaceutical and Life Science Companies

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ZEENIA FRAMROZE
DIRECTOR OF STRATEGY AND OPERATIONS, SHARECARE

DR. FRANCESCA RINALDO
SVP OF CLINICAL PRODUCT AND INNOVATION, SHARECARE

DR. NIRAV SHAH
CHIEF MEDICAL OFFICER SHARECARE

SmartOmix
by sharecare

Table of Contents

Introduction

Collecting real-world data and generating real-world evidence across products, be they drugs, smartphone apps, medical devices, or procedures, has grown in importance in the sphere of pharmaceutical and life science research. As more and more devices generate patient health data, and more health record systems become interoperable, there is a growing opportunity to leverage this data to more deeply understand study participants and patients. For pharmaceutical and life science companies, the rapid changes in this space have led to new lines of inquiry in how to streamline drug development processes, and how to more effectively run post-marketing surveillance. The industry has certainly answered that call, with the real-world evidence solutions market projected to reach $2.3 billion by 2026 and growing at a rapid rate with a 14.4% compound annual growth rate between 2021 and 2026.[1]

Yet, the acquisition of rich, meaningful, and statistically significant real-world data has so often felt out of reach, however desired it is by researchers and regulators. This has been in no small part due to the difficulty of designing and launching a study that can both reach a diverse set of audiences and capture sufficient real-world data about them. The ubiquity of smartphones and wearables has significantly altered the landscape for observational research. With the number of connected devices ‘on the edge’ – essentially, the last physical connected devices on the network, like a phone or a smartwatch – a smarter approach to the collection of real-world evidence is becoming a reality for pharmaceutical and life science companies.

With this future within grasp, it is crucial to understand the risks and opportunities of virtual and decentralized, real-world research. First, this white paper will outline the mounting costs and consequences of a manual approach to observational research. Next, it will describe how Smart Omix is designed to both mitigate some of these challenges and empower pharmaceutical companies with the tools and software to conduct engaging, participant-centric studies. With examples of how Smart Omix has supported studies across the research industry, this white paper will illustrate the many benefits of the Smart Omix approach to decentralized, real-world research.

The Four Horsemen of Observational Research

There is always a positive tension between the controlled environments of interventional trials and studies, and the real-world data sets gathered through non-registrational trials. One could argue, however, that the pendulum has swung too far towards industry standard RCTs, with less of an emphasis on gathering real-world evidence at every stage of a product or drug development pipeline. When this type of real-world observational research is undervalued, there are multiple consequences, including:

Wasted Money

Squandered Time

Failed Approvals

Clinical Non-Use

The net result?

FRUSTRATION, WASTE, AND MISSED OPPORTUNITIES.

For path-breaking pharmaceutical companies, these are not mere ‘inefficiencies’; they are the difference between getting a patient a necessary medication that can change their life—or save it.

Wasted Money

According to PHRMA, pharmaceutical companies invest nearly $100 billion annually into research and development, and in the past two decades, they have invested almost $1 trillion.[2] For clinical development costs alone, each new medicine requires $1.5 billion in investment on average, including nearly $1 billion in cash.[3] However, clinical studies that fail to draw conclusions—positive or negative—produce a loss of investment for the resources that were poured into them. Without having a holistic picture of the individual, real-world therapeutic benefit (in terms of quality of life and functional status) may be missed. Moreover, patient groups may be lumped together: specific populations—and potential target markets—standing to disproportionately benefit from the therapy overlooked. Side effect profiles of the therapy may be insufficiently characterized, with implications for both regulatory approval and clinical uptake. Even negative findings can help companies undertake strategic pivots. But inconclusive studies that fail to yield a complete picture of the individual—due to inadequate enrollment, inadequate sample observations, or other reasons—can leave researchers rudderless. Accordingly, analyses have estimated that tens of billions of dollars in research funding are wasted annually across the world, including an estimated $28 billion in the United States in 2015.[4]

FDA TIMELINE

PHASE 1	SEVERAL MONTHS
PHASE 2	SEVERAL MONTHS TO 2 YEARS
PHASE 3	1 TO 4 YEARS
PHASE 4	INDEFINITE

Squandered Time

According to the FDA, clinical trials alone (disregarding preclinical research efforts) can take six years or more.[5] Including preclinical development time, studies indicate that the average time from pipette to practice is 17 years.[6] Post-approval, uptake into routine, widespread clinical practice can take even longer. Studies indicate that progress from practice to the page (of guidelines) takes an additional nine years on average; completing the lifecycle of bench to (every) bedside in just over a quarter century.[7] Often these delays are related to the time required for best-practice committees to review sometimes scant literature for new therapies before making a recommendation. When a development drug fails a given trial phase, due to a study with inadequate power or data to prove out its safety and/or efficacy, years can be added to the timeline. The availability of rich and comprehensive data drawn from real-world settings has the potential to vastly accelerate this process.

Failed Approvals

For their enormous investments of time, capital, and labor, pharmaceutical companies rely upon FDA approvals to pave their path to commercialization and revenue generation. However, according to the FDA, only about one out of every 17 drugs that initiates clinical trials obtain approval.[8] This is despite the fact that when a drug "fails to reach approval," it does not mean the drug "does not work." Increasingly, the FDA is embracing decentralized, continuous, virtual, real-world data in its review process to "fil[l] gaps in data that may be difficult to obtain" from contemporary methods.[9] Through the 21st Century Cures Act, passed in 2016, the FDA explicitly encouraged pharmaceutical companies to "modernize clinical trial designs, including [through] the use of real-world evidence, and clinical outcome assessments" in order to "speed the development and review of novel medical products".[10] In its primer specifically discussing real-world evidence, the FDA stated that it "holds potential…to answer questions previously thought [sic] infeasible".[11] In a draft framework document, the FDA also described how real-world evidence can enable "more rigorously designed observational studies" to "contribut[e] to the evidence of drug product effectiveness" beyond being limited to evaluation of safety alone;[12] in other words, real-world evidence can dramatically streamline and improve the regulatory review process.

> "Crossing the 'valley of death' is not only about science."
>
> — **Attila A. Seyhan,** *Translational Medicine Communications*

> "The future of our health care system...depends on our using our limited resources wisely."
>
> — **Peter B. Bach, Leonard B. Saltz and Robert E. Wittes** *The New York Times*

Clinical Non-use

Even after drugs garner FDA approval, their profitability depends on routine and widespread clinical use, ideally among a diverse and representative patient population. As hospitals and payers are increasingly price-sensitive in the setting of ballooning healthcare costs in the U.S., "approval" no longer equates "acceptable" or "useful" when it comes to patient care. There is a necessity for companies to expand their knowledge about the relative and real-world patient experience. For example, in 2012, Memorial Sloan Kettering Cancer Center (MSKCC) boycotted a new treatment for colorectal cancer (Zaltrap) when the drug—at an $11,000 per month price tag—failed to demonstrate incremental effect compared to the previous standard of care. "Ignoring the cost of care, though, is no longer tenable," the MSKCC authors wrote in a New York Times op-ed, "The future of our health care system…depends on our using our limited resources wisely".[13] Similarly, in 2021, numerous health systems (including the Cleveland Clinic and Mount Sinai Health System) and insurers (including Blue Cross Blue Shield in Michigan, North Carolina, and Pennsylvania) refused to pay for a new Alzheimer's drug (Aduhelm) after determining "insufficient evidence of Aduhelm's benefit for patients".[14] Ultimately, study designs that help to conclusively prove out a combination of effectiveness, safety, usability and clinical validity and acceptability in real-world settings can prove to be incredibly useful.

The Smart Omix Solution

Harness the Power of Real-World Evidence Across Development Pipelines

> Data are not necessarily useful simply because they are voluminous. The abundance of data cannot presuppose its needed diversity."
>
> — **Cahan et al**. *Nature Digital Medicine*, 2019

Real-world evidence offers a solution to the challenges facing clinical research. Eric Topol, founder of the Scripps Research Translational Institute and a leading digital health luminary, has previously described the potential for "unprecedented data infrastructure" to "defin[e] each individual's unique biology" and "reboot the way health care can be rendered" through precise and effective therapies.[15] "Patient reported outcomes data from clinical trials and from real-world clinical settings can provide invaluable information on the benefits, risks and impact of new medical therapies," the Tufts Center for Drug Development wrote, "real-time and predictive analytics built around these data have the potential to generate valuable insights and to facilitate higher quality and more efficient drug development".[16]

However, the usefulness of real-world evidence is not etched in stone. Its usefulness depends on 4 "V's": volume, velocity, variety, and veracity. With Smart Omix, researchers can seamlessly harness the power of deep, versatile, and accessible real-world evidence to assess the impact of novel medications and participant-reported outcome measures in real-time.[17] Our platform enables researchers to:

COLLECT RICH AND CONTINUOUS DATA on massive populations, harnessing smartphone-based applications and remote sensors to power your enrollment pipeline.

COLLECT DIVERSE AND COMPREHENSIVE DATA on distinct populations, synthesizing multi-dimensional findings to promote replicable, repeatable, and externally valid results.

COLLECT IN VIVO DATA from "out there" on living and breathing populations, incorporating environmental influences and behavioral patterns to ensure the seamless translation from bench-to-bedside.

COLLECT OBJECTIVE AND SECURE DATA through encrypted, cloud-based exchange platforms pulling information from validated applications, sensors, and instruments.

Build Your Study with Smart Omix

Construct

- Leverage our pre-built data pipelines to gather rich electronic participant reported outcomes (ePROS) and participant-generated data.
- Reduce the time it takes to prototype and iterate on your study using our proprietary all-in-one platform.
- Work with our interdisciplinary clinical AI team to develop unique and intuitive mobile-optimized protocols to build digital biomarkers and gain deeper insight into real-world, lived patient experiences.

Recruit

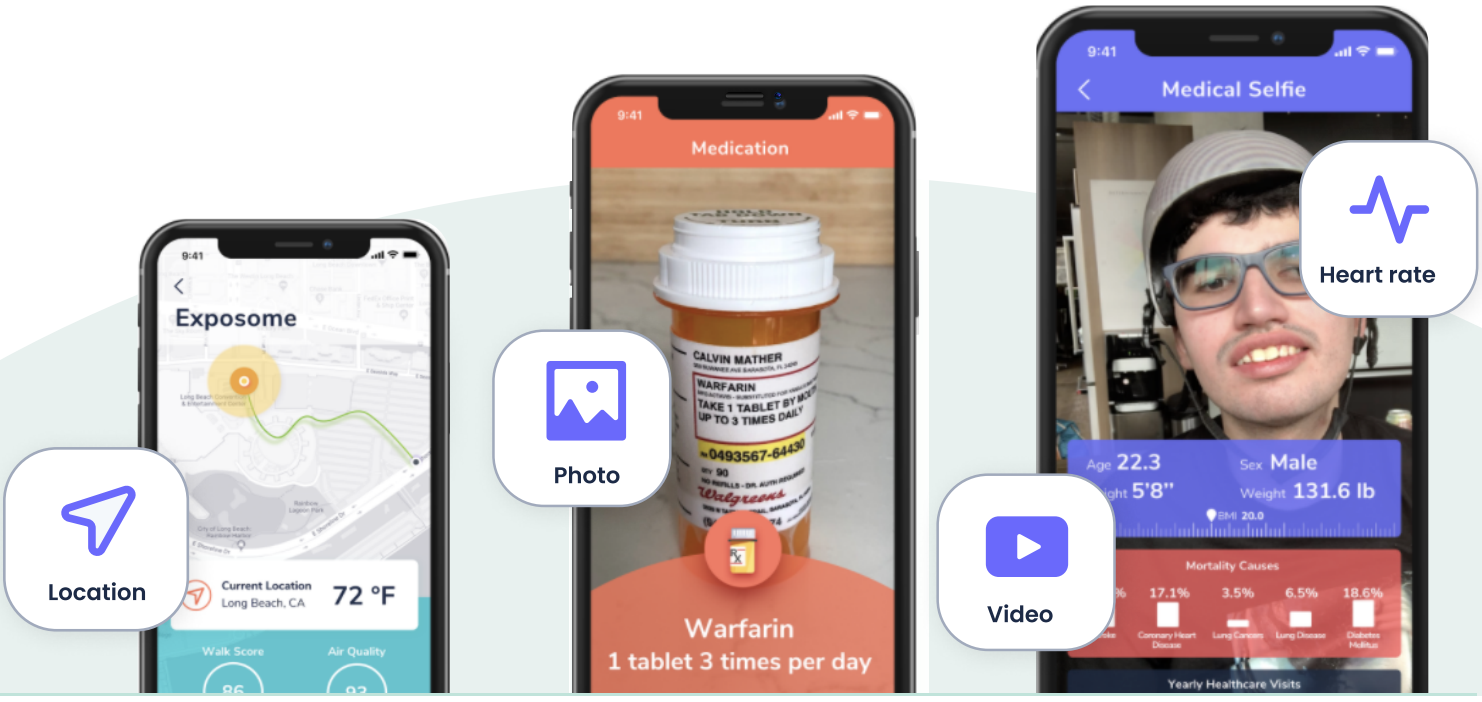
- Launch your study via the participant centric Smart Omix app on iOS and Android.
- Invite diverse and specific participants from a pool of 71M digitally native, verified first-party users via our Real-Time Profiling Engine.
- Rapidly enroll eligible participants using our FDA Part 11 compliant e-Consent modules.

Engage

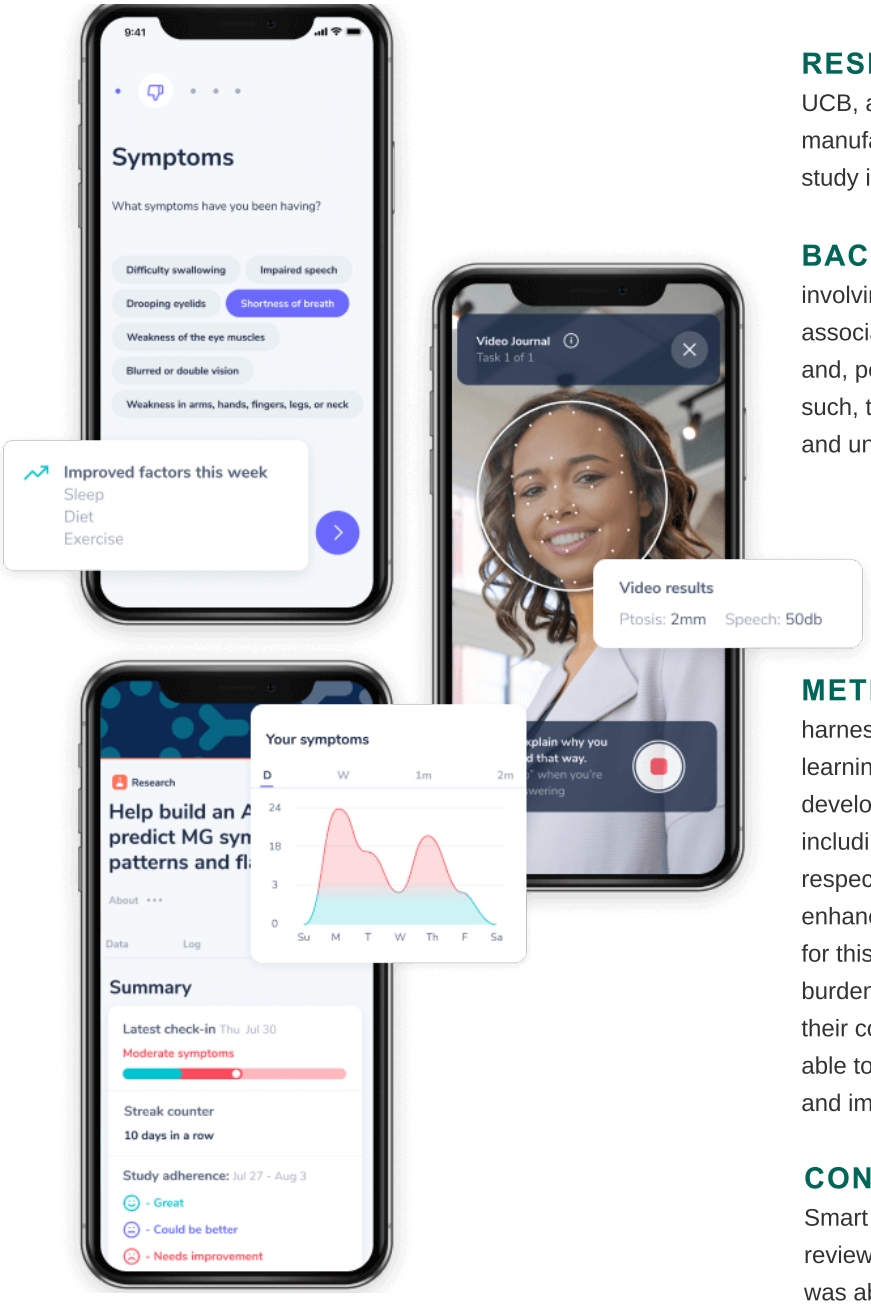
- Empower and engage participants using proprietary ePRO modules, from AI-driven “smart selfies” to custom ePRO tasks, like voice or video recordings.
- Incentivize participants with gamification modules to allow them to accrue and redeem points and rewards.

Analyze

- Leverage our interdisciplinary clinical AI team to identify strong signals in your real-world evidence for a variety of clinical purposes, from the development of digital therapeutics to regulatory submissions for SaaMD.



Smart Omix Spotlight



RESEARCH TEAM: Smart Omix collaborated with UCB, an international pharmaceutical development and manufacturing company, to help conduct an observation study in patients with Myasthenia Gravis (MG).

BACKGROUND: MG is a rare neurologic disorder involving immune-mediated destruction of nerve-associated signaling receptors, leading to profound fatigue and, potentially, life-threatening respiratory depression.[18] As such, the study sought to utilize real-world data to identify and understand exacerbations in this condition.

METHODS: Numerous types of real-world data were harnessed in the study. In addition, cutting edge machine learning models were developed to interpret custom-developed voice and video ePROs for hallmark symptoms, including eyelid drooping (ptosis) and speech deficits, respectively. These multi-modal outcome variables can greatly enhance the number of observations available to researchers for this rare and understudied cohort. Too often, the financial burden of acquiring data on rare disease patients can impede their conduct, using the Smart Omix platform, our team was able to quickly and cost-effectively develop methods to test and implement this new study design.

CONCLUSIONS: The pilot collaboration between Smart Omix and UCB was highly successful, with peer-reviewed publications forthcoming. The Smart Omix platform was able to recruit and retain participants with a rare disease at a high rate. It was also able to develop highly predictive artificial intelligence models to interpret novel data sources including audiovisual clips. Finally, participants reported that the Smart Omix platform was usable, that the study demands were feasible, and that they enjoyed their experience in the study. One participant wrote, “I loved the app;” “It was also good to feel a part of something bigger than yourself,” said another, “something important, something that could push back against an illness.”

Conclusion

As described in the Smart Omix Spotlight case study, Smart Omix can play a significantly beneficial role within pharmaceutical and life science organizations. Whether you are studying new or old therapies for their safety or efficacy, retrospectively or prospectively, over a short or long time period, in conditions with small or large populations—Smart Omix offers a platform capable of harnessing real-world data in a decentralized manner and unlocking the opportunities of next-generation clinical research.

By taking advantage of all Smart Omix has to offer, you can improve the quality and efficiency of your research by maximizing statistical power while minimizing resources required. This can optimize the likelihood of approval and subsequent clinical use, while reducing the amount of monetary, labor, and time-related waste associated with drug development.

Learn More

WWW.SHARECARE.COM/SMARTOMIX

Schedule a demo

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