

# Combining the Discipline of Clinical R&D with the Promise of mHealth

Medidata's Vision & Strategy for  
Instrumented Patients

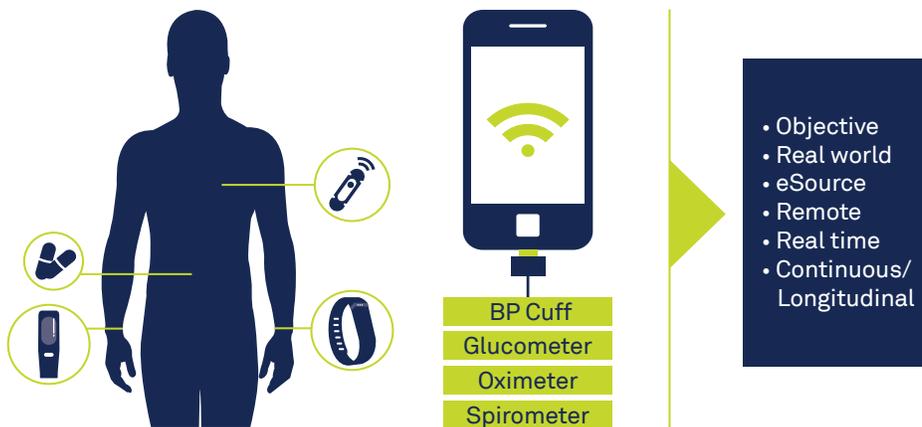
# Opportunity

Mobile health (mHealth) technology will redefine how we conduct clinical R&D. Consumer-friendly technologies, including remote sensors, wearable devices and mobile apps, offer clinical development access to potentially rich, sensitive patient data that not only complements existing sources—providing a more complete view of therapy safety and efficacy—but may also provide a better understanding of disease.

Clinical studies are exercises in data collection and analysis to determine and prove treatment safety and efficacy. Trial sponsors have traditionally employed physician investigators to collect data from patient-volunteers on their behalf and to assess response to therapies. This visit-based, episodic data, using case report forms and surrogate endpoints like the six-minute walk test, once provided the closest approximation of reality available. Now however, a much closer approximation of reality may be available through the increasing availability of mHealth-driven, patient-direct data.

Rapid proliferation of mHealth devices—from unobtrusive biosensors and wearables to smartphone apps—offer continuous, high-frequency data about a patient’s biology, movement and quality of life, opening the door to myriad applications and insights. The potential value our industry can gain from these technologies is profound because this patient-direct data possesses the following compelling attributes:

Figure 1: Instrumenting produces a new class of data



Because it’s real-time, mHealth data provides better insight into potential adverse events (AEs). Because it’s remotely captured, mHealth data increases the pool of eligible patients and has the potential to reduce the patient burden. mHealth data can even serve as a “digital biospecimen”, offering extraordinary look-back potential and enabling new insights on trajectory of disease and impact of therapies. Finally mHealth data offers the promise of objective measurement that could replace much of the subjective data that is gathered today.

## Challenges

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All of the promise of mHealth cited above is the good news. But there are significant hurdles to using mHealth data to improve the quality and velocity of clinical R&D. Challenges spanning a wide range of technical, regulatory and operational issues abound:

- How will regulators and IRBs view and accept sensor-captured data?
- How do we ensure the privacy of this more detailed and sensitive patient data?
- Of the many devices and apps available, which ones should clinical trials be using?
- How granular should the data be for research purposes?
- Will the additional device and app burden result in patient non-compliance?
- How is it possible to scale to manage “big data” loads?

This paper will describe Medidata’s vision, strategy and commitment to mHealth and how we think about addressing the challenges of using mHealth data in clinical R&D.

## Our Vision

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Life Sciences companies exist to produce tools for healthcare providers to fight disease and improve quality of life. Medidata’s vision is to enable better healthcare tools by exposing data from “instrumented” patients to clinical researchers in an easy-to-use and regulatory-compliant way. By producing a richer, more complete dataset through instrumentation, life sciences companies can more easily quantify disease progression, make faster, better informed therapy conclusions, and use the same dataset to prove safety and efficacy to regulators and effectiveness to payors. By working with like-minded partners, we will enable therapeutic innovations that were never before possible.

## Our Strategy

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While mHealth technology has matured rapidly, Medidata has proactively sought to understand the opportunities and challenges, initially by engaging patients in the manner currently used outside of clinic visits: via electronic patient-reported outcomes (ePRO). We launched our innovative ePRO offering (Medidata Patient Cloud®) as a mobile app to understand how studies benefit from engaging with patients via the smartphone already in their pockets.

We continued the learning process by sponsoring our own mHealth-centric studies, first with the MOVE-2014 diabetes study and, more recently, our internal SOLO study to evaluate the use of six promising wearable devices to determine whether they could be a good fit for clinical research.

Medidata's "instrumented patient" vision is not our first effort to transform R&D through the innovative application of technology. Fifteen years ago we built our electronic data capture (EDC) system (Medidata Rave®) which enabled users to define the data they wanted to collect at different times in the study. This definition then manifests in a number of different ways, including a user interface for sites, integration connections, batch upload, and patient reported outcome tools. We are now redefining eclinical technology through the creation of a comprehensive and open clinical data platform, the Medidata Clinical Cloud®.

## Open Framework & Ecosystem

Medidata is working with a diverse and progressive cohort of partners to bring a comprehensive "connected patient" solution to market that unlocks the potential of mHealth in clinical trials. This cohort includes sponsors, contract research organizations (CROs) and technology vendors (device and app vendors, third-party data aggregators, etc.). Many technology companies, large and small, are capturing patient-generated health data using mHealth technology today. To deliver the richest, most complete dataset possible, the Medidata Clinical Cloud employs an open, plug-and-play architecture enabling third-party devices and apps, with the appropriate permissions and controls, to expose data for inclusion in the clinical record.

We actively evaluate devices and apps and work to create standards across these platforms, maintaining a device-agnostic philosophy with an open and transparent perspective on device/app applicability and feasibility.

## Centralized, Regulatory-Compliant Platform

Bringing data relevant to clinical development together in a regulatory-compliant way is not only a core competence of Medidata's—it's our mission. Many companies capture and aggregate data from wearables, but data used for regulatory submissions must answer to a higher scientific standard. The capture and storage of patient data for research must adhere to a strict set of regulatory requirements that are engineered into the Medidata Clinical Cloud. The Medidata Clinical Cloud ingests mHealth data, creates a regulatory-compliant audit trail, and aggregates and transforms the data to extend the clinical record. Medidata uses a well-defined framework of standard operating procedures (SOPs) in accordance with our Quality Systems program overseen by our Quality & Regulatory Affairs organization.

## Regulation of mHealth

The standard for clinical data is high for good reason: peoples' lives depend on it. While some in the industry have taken a pessimistic posture regarding mHealth—any new technology falls under the microscope—Medidata is optimistic about regulators' willingness to accept patient-direct data. The FDA, which often serves as a bellwether for regulatory bodies regarding technology adoption, has on multiple occasions expressed openness to mHealth technology and, specifically, sensor-captured data.

Medidata's QRA organization provides expertise regarding mHealth regulatory requirements and controls, like ALCOA guidelines for eSource data, based on consultation with the appropriate industry voices.

As a trusted provider of technology-based solutions to the life sciences industry, we are in a unique and enviable position of being able to share mHealth experiences with regulatory bodies. We view it not only as a right, but as a responsibility to enable researchers to use data from instrumented patients. After all, EDC and internet access were new not that long ago!

## Extensible, Unified Clinical Record

The Medidata Clinical Cloud is the industry's leading, cloud-based platform used to capture rigorous, highly-structured data from sites. While this site-captured data continues to be foundational, our mHealth offering enables a greatly expanded clinical record. We map the clinical data to the mHealth data enabling a single structured dataset that meets the demanding needs of randomized controlled trials and is also easy for study teams to analyze and visualize. This transformation unlocks the potential of raw mHealth data to produce clinically relevant insights.

By enabling an expansive, dynamic clinical record, we can tap into the growing list of sources of clinically relevant data. For example, this clinical record could be expanded to include a broad range of "omics" data, images, and other sources, producing a comprehensive view of the patients' therapeutic experience.

## Privacy/Security & Big Data Scale

Data privacy and security is critical for traditional EDC, but incorporating direct patient data introduces additional scrutiny. In architecting the Medidata Clinical Cloud, we've added the Patient Cloud Shield which serves as a firewall between personally identifiable information (PII) and study teams. To assure the completeness of our security policies we follow the ISO 27000 and ISO 27001 architectures as a baseline and then we supplement them with portions of other recognized security architectures. We protect data and ensure privacy through the use of a Secure Socket Layer (SSL) encryption with a minimum key length of 256 bits and our threat modeling includes internal and external PEN testing. Our guidance in mHealth originates in NIST SP 800-53 and NIST 800-82 which were the basis of the FDA recommendations as well.

The amount of data that can be collected from instrumented patients is staggering. For example, a recent project generated **18 million data points per patient per day**. The Medidata Clinical Cloud runs on a platform that scales data loads without constraint and in magnitude beyond the data volumes found in the largest traditional studies.

## Data Science: Realizing the Value of mHealth Data

In general terms, data science is the extraction of knowledge from data. mHealth generates massive volumes of data which, in its raw form, adds limited value. Data science is the process of extracting datasets, performing exploratory analyses to refine them and then applying models and algorithms to produce visualizations that yield actionable insights.

Medidata's Data Science organization, led by our Chief Data Officer, employs state-of-the-art tools and techniques to demonstrate the extraordinary value instrumenting patients can have on clinical R&D. These quantitative and analytical tools and techniques, including powerful visualization frameworks, are best-in-class. We utilize big data techniques found in insurance industry disaster modeling and defense industry telemetry systems to ensure our clients derive maximum clinical value from their mHealth investments.

## Comprehensive Clinical Analysis & Visualization

With an abundance of additional data generated on a continuous basis, how do we find the clinically relevant insights? How do we separate signal from noise?

To answer these questions, we built a powerful statistical computing environment into the Medidata Clinical Cloud that facilitates a wide range of analyses like progression analyses, new symptom identification and identification of digital biomarkers.

A key to separating signal from noise is applying sophisticated data science techniques to the data. We have developed a data science offering as a foundational element of our mHealth solution. Using the statistical computing environment, we identify potential non-serious AEs such as vomiting, headache, or pain that may not be self-reported. As we build out datasets, we apply additional predictive analysis techniques that help forecast such events. These analytics open the door to a better understanding of disease using a broad range of health events and conditions.

Figure 2: Better phenotypic data and endpoints - examples

	Old	New (from pharma sponsor feedback)
<b>IBD / Crohn's disease</b>	Steroid-free remission	Geographic distance traveled from home
<b>Stroke</b>	NIHSS (National Institutes of Health Stroke Score) or Rankin Scale	Gait, cadence, balance of steps between left foot and right foot
<b>Parkinson's</b>	Unified Parkinson's Disease Rating Scale (UPDRS)	Level of vocal tremor during phone conversations
<b>COPD</b>	6-minute walk test	Daily steps for trial duration
<b>Asthma</b>	PRO surveys on number of night awakenings	Number of night awakenings detected by wearable device
<b>Obesity</b>	Weight, blood pressure and hypoglycemic events	Active minutes / steps walked

## About Medidata

Medidata is the leading global provider of cloud-based solutions for clinical research in life sciences, transforming clinical development through its advanced applications and intelligent data analytics. The Medidata Clinical Cloud® brings new levels of productivity and quality to the clinical testing of promising medical treatments, from study design and planning through execution, management and reporting. We are committed to advancing the competitive and scientific goals of global customers, which include over 90% of the top 25 global pharmaceutical companies; innovative biotech, diagnostic and device firms; leading academic medical centers; and contract research organizations.

**info@mdsol.com | mdsol.com**  
**+1 866 515 6044**

## Our Commitment

We will continue to push the frontier of mHealth by working with forward-thinking sponsor/CRO innovators, regulators and mHealth vendors until connected patients are as common as connected sites. By applying scientific rigor to integrated databases of biometric and activity data, we will find essential clues about disease and the human condition, much like the Rosetta Stone helped decode the secrets of ancient Egypt. Together we will advance medicine and therapies at speeds never before imagined.

To learn more about our commitment to mHealth, please visit our [website](#).

### Medidata Clinical Cloud™

Cloud-based clinical research solutions | Innovative technology | Data-driven analytics  
 Reduced costs | Improved time to market | Faster decisions | Minimized risk