EXPLORING THE FRONTIERS OF DATA AND ANALYTICS FOR PRECISION MEDICINE

OCTOBER 3, 2018
12–1PM ET
Tanisha Carino

Executive Director
FasterCures, a Center of the Milken Institute

MODERATOR
10,000 diseases
only 500 treatments
we have work to do.
**Webinars**

**Purpose**
- **Real-time** sharing of ideas, best practices, trends, and lessons learned
- **Amplifies** meaningful solutions, productive tools, and encourages action needed to spur medical progress

**Topics**
- Patient-Centered Measurement
- Patient-Focused Drug Development
- Innovations in Clinical Trials
- Venture Philanthropy

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**Who’s logged on?**

~ 400 registrants from:

- **Nonprofit** 30%
- **Other** 30%
- **Biotechnology** 19%
- **Academia** 4%
- **Pharmaceutical** 11%
- **Government** 3%
- **Consulting** 3%
- **Other** 30%
>70% of patients and caregivers agree that health data is useful even if incomplete

41% of individuals haven’t ever viewed their health data, but when they do, the majority find it helpful

30% aren’t sure there are safeguards to protect health data from being misused

63% report they could not track down who their health data had been shared with

Health literacy is low, only 12% of adults in 2003 being considered “proficient”, how can we expect patients to understand their health data?
Kathy Giusti
Executive Chairman, Board of Directors,
Multiple Myeloma Research Foundation;
Senior Fellow and Co-Chair, the Kraft
Precision Medicine Accelerator at the
Harvard Business School

Gabriel Eichler
Founder and Managing Director, Oak Health
Partners; Data & Analytics Workstream
Lead, Kraft Precision Medicine Accelerator
at the Harvard Business School
Kraft Precision Medicine Accelerator

Strategic Overview

October 3, 2018
The Accelerator leverages Kraft Foundation funding and HBS to solve the challenges in precision medicine.
The Accelerator advances four integrated work streams – all critical elements of Precision Medicine.
Direct to Patient: The Challenge

Cancer patients are overwhelmed and often don’t have the knowledge or tools to optimize their care.

- Only 46% of patients have heard of precision medicine.
- Only 20% of patients are familiar with genomic testing.
- Only 30% of patients know their subtype.

Source: Kraft 5 cancers market research, Kantar Health
Direct to Patient: What We’ve Accomplished

Working across five cancers, the DTP workstream is using DTC best practices to reach and inform patients.
Direct to Patient: Where We’re Going

Integrate Right Track Marketing and Social
Q1 2019

Close Knowledge Gaps
Q1/Q2 2019

Apply to Registries
2019
Data and Analytics: The Challenge

High-volume data sets are scattered and need use cases and machine learning to bring them together.

Question 1
Orien | CoMMpass

Question 2
MSK Impact | Genie
Data and Analytics: What We’ve Accomplished

Completed Real World Oncology Landscape

Completed First-ever AI Company Landscape

Taught the Answer Fund Model
Too many trials and too many drugs are using patients inefficiently.

20% of clinical trials fail to enroll enough patients

80% of clinical trials are delayed

20% of trials in cancer never finish for reasons unrelated to treatment efficacy/side effects

Sources: Journal of the National Cancer Institute; Cutting Edge Information

The New York Times

A Cancer Conundrum: Too Many Drug Trials, Too Few Patients
Clinical Trials: Where We’re Going

Real-time Review of Platform Studies*
Q2 2019

- GBM Agile (Glioblastoma)
- MyDrug (Myeloma)
- Precision Promise (Pancreatic)

Publication
October 2018

JCO® Precision Oncology
An American Society of Clinical Oncology Journal

Challenges Associated with Master Protocol Studies: Project Design to Launch

FDA Meeting
December 2018

*Tracking KPIs, metrics & milestones; will add new studies through outreach & recommendations
The investment landscape is vast, fragmented and inefficient.

<table>
<thead>
<tr>
<th>Cancer philanthropic space is fragmented</th>
<th>Large AMCs are major fundraisers</th>
<th>Need for bold, sustainable funding models</th>
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<tbody>
<tr>
<td>1,362 organizations, 1,223 with annual contributions of $5M or less</td>
<td>Focused on basic research</td>
<td>It takes on average 8-12 years and $1B+ to develop a drug from discovery to approval</td>
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<td>Slow and reluctant to share data</td>
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Sources: NIH, National Center for Advancing Translational Studies; Guidestar
The Accelerator has identified leading venture models to achieve sustained and sufficient funding.

**Venture Philanthropy**

**Bloomberg**

*This Medical Charity Made $3.3 Billion From a Single Pill*

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**Directly Backing Companies**

- **SOLID BIOSCIENCES**
  - Duchenne Muscular Dystrophy (DMD)
- **ENDEAVOR CATALYST**

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**Cancer Megafunds**

Andrew Lo
Professor, MIT
School Sloan of Management

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**CURING CANCER IS EVERYONE’S BUSINESS**

CONFIDENTIAL
Investment: Where We’re Going

Venture Philanthropy Across Disease Groups
October 2018

Vehicle Megafund Meetings
Q1 2019

Investors
Philanthropists
Institutional Investors
Tech

Venture Capital
Academic Medical Centers
Biotech
Patient Foundations
Pharma
Executive Education

Case Studies

- The Multiple Myeloma Research Foundation’s Answer Fund
- Impact Investing For Cancer
- Adaptive Platform Trials: The Clinical Trial of the Future?
- Intermountain Healthcare: Pursuing Precision Medicine

Bylines

Forbes

What Precision Medicine Can Learn from the NFL

Kathy Giusti and Richard G. Hamermesh

Exec Ed Sessions

Oct. 23-25, 2019
Stay in touch with us!

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@KathyGiusti
@HBSHealth

Visit our site
www.hbs.edu/kraft-accelerator
Real World Data Landscapes
A recipe for insights

Question + Data Source + Analytical Method

= Hypotheses (!!)
Update to the KPMA Real World Data Landscape

2016 Data Landscape

Focus: Longitudinally, Openness, Funding Sources
Scope: Datasets of all sizes and types

Since our 2016 landscape:
- Lots of new entrants generating and consuming real world oncology data
- New data assets are becoming available commercially
- New registries and platform trials have burgeoned
- Massive investments in clinical data abstraction technologies and efforts
**Update to the KPMA Real World Data Landscape**

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**2018 Data Landscape**

Focus: Integration of Molecular, Clinical, Longitudinally and Accessibility  
Scope: Most significant datasets by size and disease focus; Dataset linkage and abstracted clinical data. US Focused.

Collected meta-data from Q4 2017 - February 2018

Data Collection:
- Stakeholders Included for-profit and non-profit dataset owners  
- Initially survey outreach with telephone follow-up as needed
2016 Data Landscape

**Opportunity exists to generate publicly available longitudinal data to drive understanding of genetic mutations and find Precision Medicine cures**

- Datasets have potential to include longitudinal data in the future
- Public/private information not available
- Serves as a portal also, has potential to include longitudinal data in the future

1. FoundationCore’s pediatric cancer data has been made public

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Note: Representative selection of landscape, not all inclusive. Dataset selection focused on oncology specific and oncology-inclusive datasets.
Large, integrated data assets are starting to emerge and advance real world data research – but platform trials and registries continue to have highly tuned data for particular diseases.

X-Axis: Dataset Quality refers to the integration of (a) molecular, (b) disease-specific, structured clinical, (C) longitudinal data

Y-Axis: Higher up organizations are sharing openly and for free. Mid-axis organizations have commercial offerings and closed, private data are inaccessible to anyone

Genomic Data Commons contains data from FoundationCORE, Broad’s CCLE program, NCI’s CCGI, TARGET and TCGA programs.
How outsiders think of AMC data assets
(neat, accessible, clean and ready-to-go data assets)
Views of Real World Data Sources

How outsiders think of AMC data assets
(neat, accessible, clean and ready-to-go data assets)

The reality of AMC data assets
(disbursed, complex, dirty & inaccessible)
### Key Finding on 2018 Data Landscape

#### Real World Assets Are Growing in Size and Sophistication:
- 25% growth in median size since 2016.
- Many organizations now exceed 1M patients in their dataset.
- Heavy investments in curation.
- Patient registries still have some of the richest data on specific diseases.

#### Sharing and Linking Models are Winning:
Growing value of linked data assets:
- Flatiron + Foundation Medicine
- CancerLinQ + PH.AI + Tempus
- OPeN
- M2Gen / ORIEN
- HealthVerity (not shown on landscape)

#### Willingness to Share and Link Are Rising:
The largest data assets have embraced a willingness to link:
- Cota, Caris, DFCI Profile, and others
- Many organizations indicated a willingness to share though consent and technical hurdles are prohibitive.

#### The Frontiers of RWD Are Advancing:
Advancing the frontier lies in:
- Linked specimen banks
- Raw imaging datasets for radiomics
- Patient re-contact rights & mechanisms to enable additional research opportunities
- Improved data standards
Real World Data Analytics Landscapes
Healthcare Analytics Explosion

AI to save healthcare $150B by 2025

Philips launches AI platform for healthcare

Big Data, Analytics Ready to Meet Health Care Challenges

AI Holds Promise of Improving Doctors’ Diagnoses

With artificial intelligence, machines can see what many humans may have missed
Healthcare Analytics Uncertainty, Confusion and Concern

Amazon's Alexa Really Isn't Ready For Healthcare
MAY 24, 2018

AI Healthcare Revolution: Doctors and Data Fight Chronic Challenges
May 24, 2018

Artificial Intelligence In Healthcare: Separating Reality From Hype
MAY 14, 2018 Forbes

IBM pitched its Watson supercomputer as a revolution in cancer care. It’s nowhere close
SEPTEMBER 5, 2017 STAT
Market is Complex and Lacks Direction on Emerging and Established Players
Key Stakeholders and Questions for Analytics Landscape

**Stakeholders:**
- Investors
- Researchers
- Non-Profits
- Hospitals/Care Centers
- Payers
- Patients

**Questions:**
- Where are advanced analytics being most aptly applied?
- Who should I invest in?
- Is it best to Build vs. Buy vs. Partner?
- When may these technologies be ready for real-world applications?
Application Areas Explored

Pre-clinical R&D
- Drug Discovery Research
- Biomarker and Target Identification

Clinical Research
- Clinical Trial Analysis
- Clinical Trial Patient Identification

Clinical Care
- Population Health & VBC Analytics
- Clinical Pathways & Decision Support
Companies Analyzed

Companies analyzed:

- 46 Companies identified through online reports, conference agendas, etc.
- Primarily US-based

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<td>Imagia</td>
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<td>Ariana Biopharma</td>
<td>Congenica</td>
<td>Lantern Pharma</td>
<td>Precision Health.ai</td>
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<td>Arteryx</td>
<td>COTA</td>
<td>Medley Genomics</td>
<td>Recursion Pharma</td>
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<td>Atomwise</td>
<td>Deep Genomics</td>
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Primarily US-based
## Companies Excluded

### Companies excluded:

- 17 Companies identified initially but removed during data collection phase
- Excluded because either AI was too small a focus within the larger business, or insufficient data was found

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<tr>
<th>Too Complex</th>
<th>Insufficient Data</th>
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<td>Flatiron Health</td>
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<td>IBM Watson</td>
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<td>Quantitative Medicine</td>
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Our Analysis Methodology

**Acceleration**
August 2017- August 2018:
- Financing
- % job growth
- % open positions

**Establishment**
Total Number of Announced:
- Publications/case studies
- Partnerships/customers
- Financing

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<th>Acceleration Percentile</th>
<th>Industry Standards</th>
<th>Hot Companies</th>
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<td>Uncertain Potential</td>
<td>Emerging Companies</td>
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Some Notes and Limitations

- Mostly focused on US companies
- Quantifies entire organizations not specific capabilities.
- Trailing indicator of company status
- Changes significantly as news emerges
- May fail to capture breakthrough innovators pursuing new use cases or opportunities.
All Companies Included in Analysis
Biomarker and Target ID Companies

Highlighted Co’s

**Tempus**
- “Data-Driven Precision Medicine”
- $110M Series E at $2B valuation
- Licensed ASCO’s CancerLinQ dataset
- 250 hospital partnerships, 2M clinical records.

**BenevolentAI**
- “Accelerating the journey from AI to Medicine”
- $115M raise at >$2B valuation
- Awarded Technology Pioneer Status by World Economic Forum
- 138 employees with 75% yoy growth

**Recursion Pharma**
- “We’re aiming for 100 new treatments by 2025”
- $60M funding in Oct 2017, $105M total
- Partnerships w/ Sanofi, UCSD
Drug Discovery Companies

Highlighted Co’s

**Atomwise**
“We design new molecules for the hardest targets”
- Featured on the 2018 Disrupt 100 list
- 29 employees, 150% yoy growth
- Many leading academic & industry partners

**Deep Genomics**
“Revolutions in AI, biology and automation”
- 31 employees with 43% yoy growth.
- Raised $13M in Sept 2017 with Khosla Ventures

**twoXAR**
“An artificial intelligence-driven drug discovery company”
- $10M investment by SoftBank and Andreessen Horowitz.
- Ambitious chemoinformatics objectives
- Modestly sized at 15 employees
Highlighted Co’s

FDNA
“Genomic insights through next-generation phenotyping”
- 65 Employees
- Expanding into Asia
- Working with large genomics organizations like Ambry, GeneDX and others
- Partnerships in rare disease research at Vanderbilt Medical Center and the University of Hong Kong
Clinical Trial Analysis Companies

Highlighted Co’s

**AiCure**
“Artificial Intelligence for Continuous Patient Monitoring”
- Named to the AI 100 list
- 50 employees, growing at 30% yoy

**NuMedii**
“Pioneering the use of big data, AI and Systems biology in Drug Discovery.”
- Stanford-licensed technology
- Partnerships w/ Allergan, Astellas, Boehringer-Ingelheim, Yale, Brigham and Women’s Hospital.
- Numerous publications and examples of thought leadership
Clinical Pathways & Decision Support Companies

Highlighted Co’s

**Mitra Biotech**
“Delivering powerful predictions for truly personalized cancer treatment.”
- $40M raised in July 2018
- Partnership with Glenmark Pharma
- >25% 12-month employee growth

**Wuxi NextCode**
“A contract Genomics Organization”
- $240M raised in Sept 2017
- Fast Company’s 2018 Top 10 Most Innovative Companies
COTA
“Improving the lives of cancer patients everywhere with data and technology”
- Raised $40 (with $65M in total funding) from IQVIA and others.
- Works closely with Memorial Sloan Kettering and many AMCs.

SHYFT Analytics (now Medidata)
“The leading commercial and clinical data analytics platform for global life sciences”
- Acquired in June 2018
- Working with numerous pharma partners on data products & visualization.
KEY FINDING ON REAL WORLD DATA ANALYTICS LANDSCAPE

**Tremendous Investments:**
- Over $1.4B raised by landscape companies
- Nearly $400M in past 12 months.
- Bullish investors and high hopes of major value creating outcomes.

**Diverse Platform Capabilities:**
- Many companies featured on multiple use-cases;
- Diverse business models underpinned by common analytics capabilities

**Paradigm Shifting Potential:**
- Success in any of these use-cases has the potential to redefine large parts of healthcare and life sciences.
- Targets of breakthrough innovations; Rare Disease Diagnostics; Preclinical informatics; Clinical Trial Design & Execution
A Special Thanks To:

**Kraft Program Support:**
- Robert & Jonathan Kraft
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- Kathy Giusti
- Krys Mroczkowski

**FasterCures**
- Tanisha Carino, Ph.D.
- Kristin Schneeman
- Emily Ball

**Data Landscape:**
- Daniel Evans
- Laura Fontana, Ph.D.

**Analytics Landscape:**
- Ryan Karmouta, MD, MBA
- Michael Milligan, MD, MBA
- Garrett Buxton, MBA
- Patrick Clapp, MS
Q&A

Kathy Giusti  
Kraft Precision Medicine  
Accelerator at the Harvard Business School

Gabriel Eichler  
Kraft Precision Medicine  
Accelerator at the Harvard Business School

Tanisha Carino  
FasterCures  
MODERATOR
Exploring the Frontiers of Data and Analytics for Precision Medicine

*In partnership with the Kraft Precision Medicine Accelerator at the Harvard Business School*

Watch the recording
Visit Health Data Basics for tools to power engagement:
https://www.healthdatabasics.org/
So You Want to Start a Master Protocol Trial...

December 10, 2018
CURING CANCER IS EVERYONE'S BUSINESS

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Get the latest developments in medial research delivered to your inbox every Tuesday and Thursday