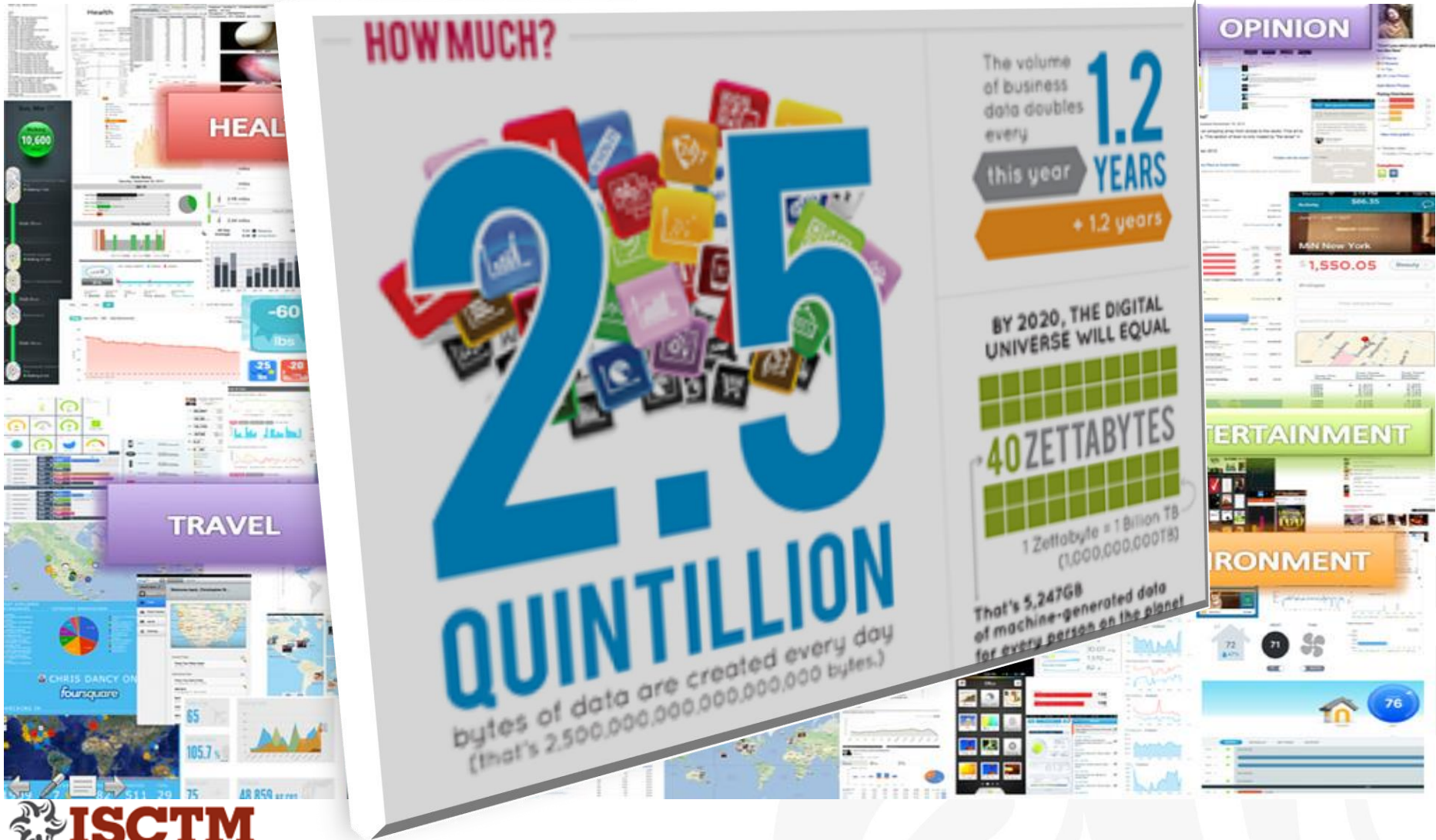


The Big Data Transformation of Drug Development: From Promise to Practice

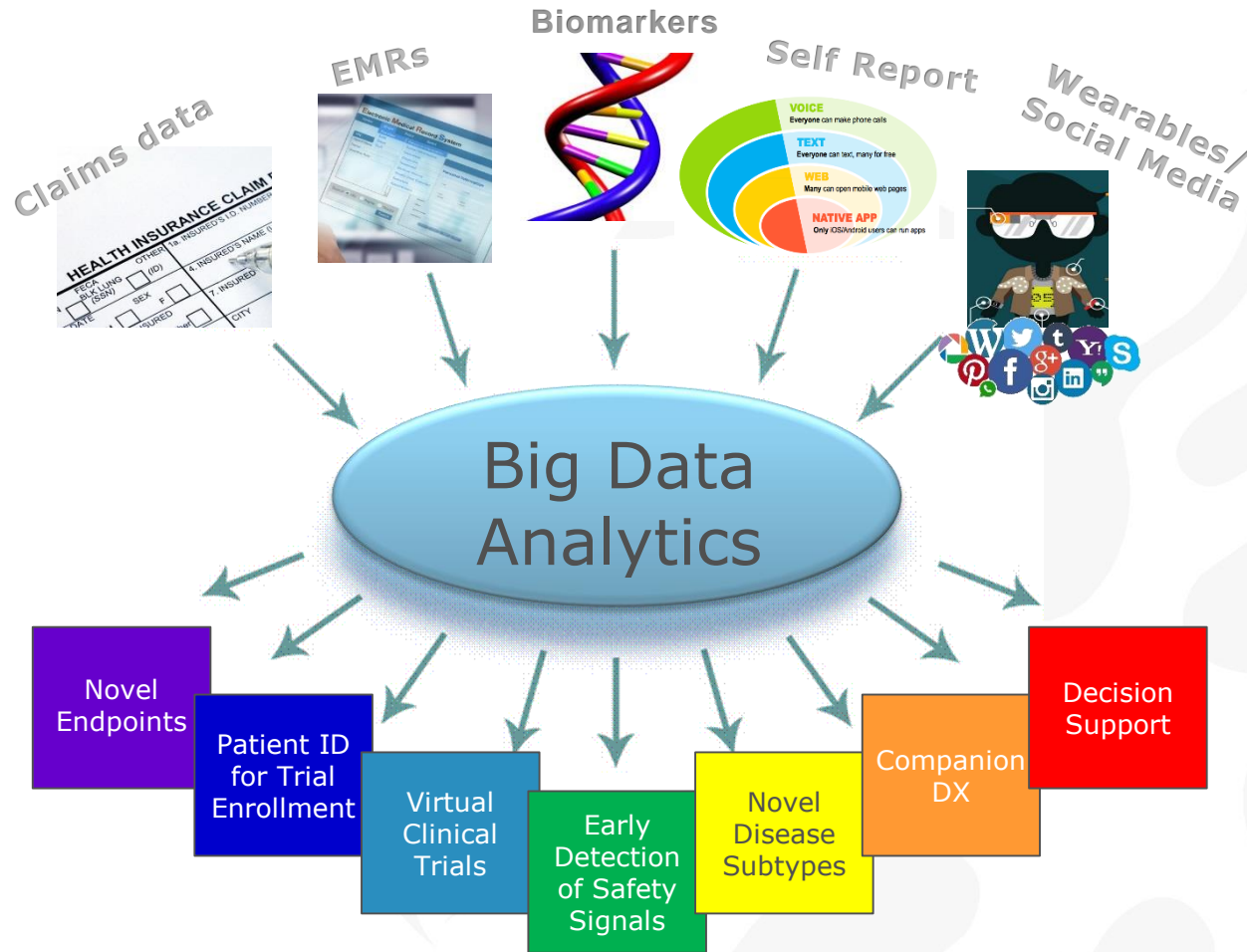
Gayle Wittenberg
ISCTM 13th Annual Scientific Meeting
21-23 February 2017
The Fairmont, Washington DC

Disclosure: Dr. Wittenberg is an employee of Janssen Research & Development, LLC, and a shareholder of Johnson & Johnson.

We are really good at generating data.



“Big Data” is everywhere, but it is not one thing. There are diverse opportunities to impact health care.



But is the impact of big data living up to its potential?

What is the ultimate goal?

Scientific Knowledge



Translation

NICE National Institute for Health and Care Excellence



etc...

More Efficient Trials
Lower Healthcare Costs

Novel, personalized Tx
Improved Outcomes
Life-years/QUALYs



Policy increasingly mandates use of real world data.

2010: Patient Protection and Affordable Care Act

H. R. 3590

One Hundred Eleventh Congress
of the
United States of America

AT THE SECOND SESSION

*Began and held at the City of Washington on Tuesday,
the fifth day of January, two thousand and ten*

An Act

Entitled The Patient Protection and Affordable Care Act.

*Be it enacted by the Senate and House of Representatives of
the United States of America in Congress assembled,*

SECTION 1. SHORT TITLE; TABLE OF CONTENTS.

(a) **SHORT TITLE.**—This Act may be cited as the “Patient Protection and Affordable Care Act”.

Establishment of PCORI
Patient Centered Outcomes Research Institute

2016: 21st Centuries Cure Act^{III}

114TH CONGRESS
1ST SESSION

H. R. 6

IN THE SENATE OF THE UNITED STATES

JULY 13, 2015

Received; read twice and referred to the Committee on Health, Education,
Labor, and Pensions

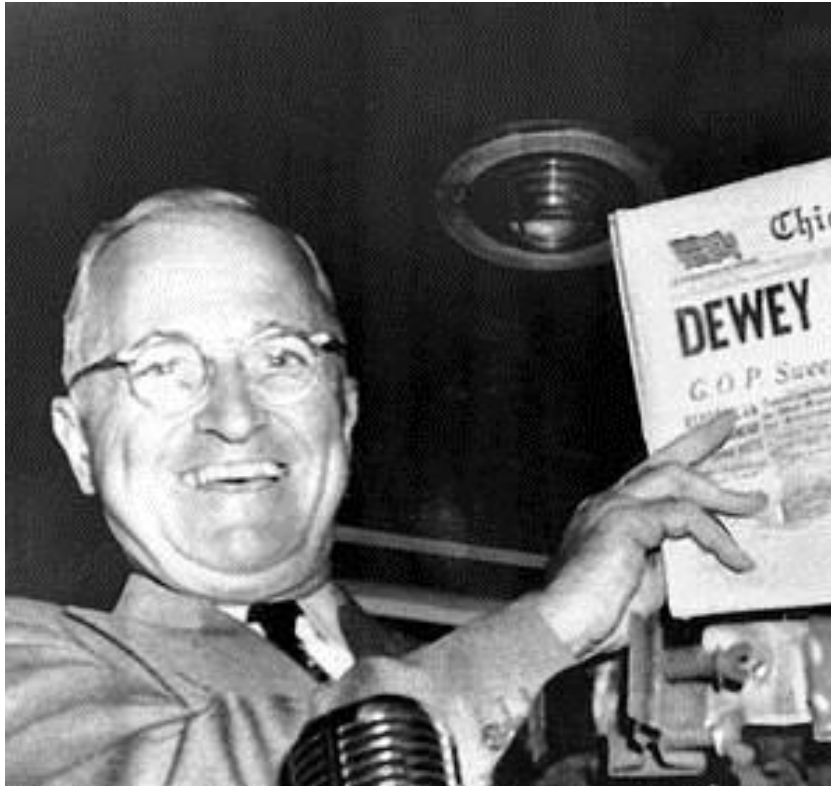
AN ACT

To accelerate the discovery, development, and delivery of
21st century cures, and for other purposes.

Requires FDA to develop a framework
and guidance for evaluating real world
evidence (“RWE”)

- to support approvals of new indications for previously approved drugs
- to support or fulfill post-approval study requirements.

Sampling Bias



- ❖ Clinical trials are designed with precise inclusion/exclusion criteria.
- ❖ Treated populations in the 'real world' do not fall neatly within those criteria.
- ❖ Value of RWE is to quantify efficacy/safety within the actual treated patient population in the real world setting as treatments are prescribed beyond the boundaries of those trial criteria.
- ❖ However, when randomized controlled clinical trials and RWE studies disagree, we need to understand the source of the discrepancy.

Design Challenges

- ❖ A data set is often generated with a specific set of questions in mind, or generated in the Real World to fill a practical purpose (e.g. billing).
- ❖ However, it's utility may expand well beyond its initial design. Post-hoc analysis of data can pose numerous challenges which must be addressed
 - Initial power estimates may not hold.
 - Some relevant variables may not have been collected.
 - Lack of control over protocol/sample collection and analysis standards (e.g. fasting status, time of collection, kits used).
- ❖ A data set may be designed with specific patient behaviors in mind.... In years to come adherence may go beyond taking a pill to appropriate interaction with devices (i.e. don't put your Fitbit on your dog to win your stepbet or hatch eggs in Pokemon Go...)
- ❖ Data aggregation from disparate sources presents challenges from data harmonization and integration to privacy concerns.
- ❖ Misunderstanding the biases within individual data sets as post-hoc studies are designed can lead to erroneous conclusions.



Stratification Challenges

Tracking pie trends

Nov. 9, 2016 - by Charlotte Atchley

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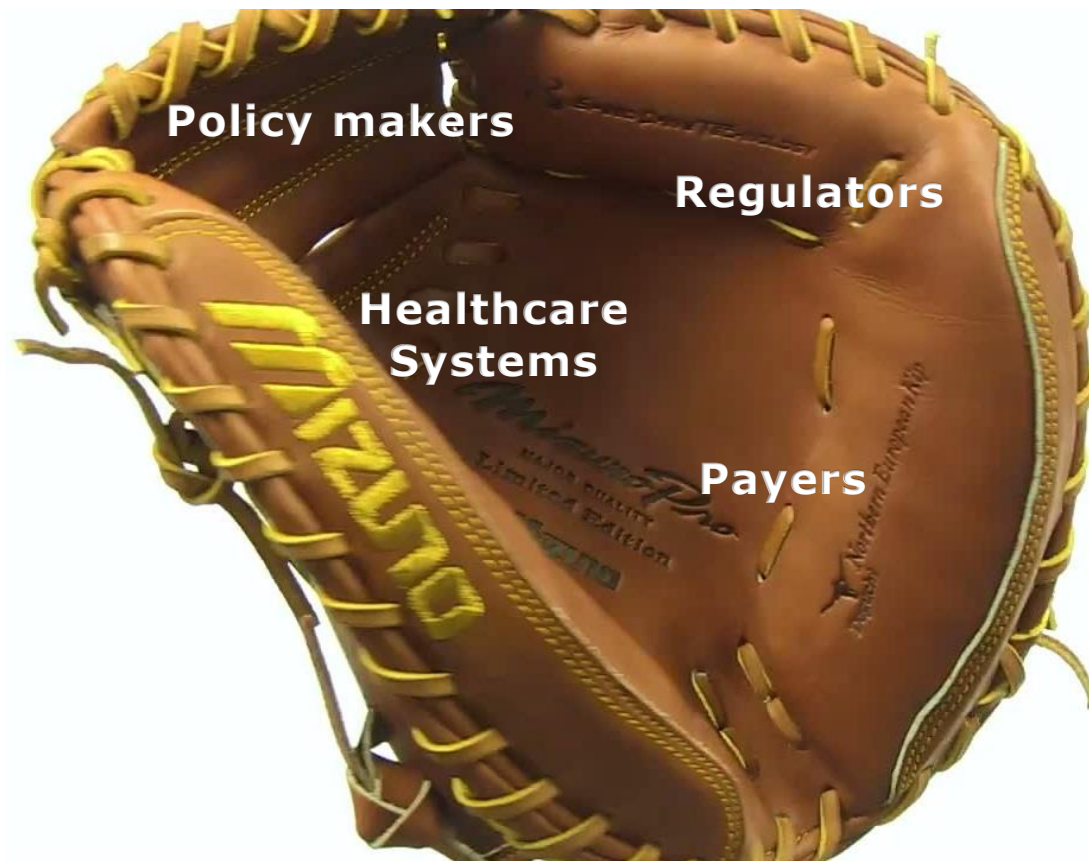
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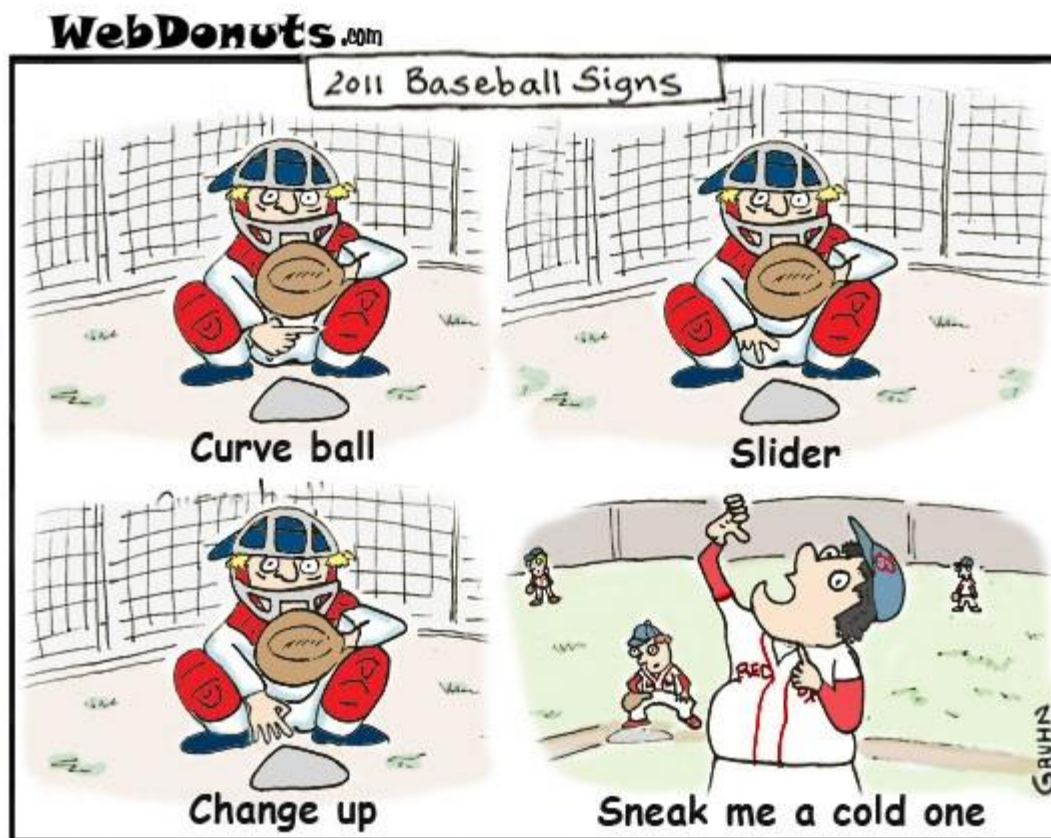
With pie sales traditionally stacked toward
ways to spark sa

- ❖ At the end of the day the goal precision medicine as an important goal.
- ❖ Regulatory paths to developing new indications should be understood early.
- ❖ Risk that policies around comparative effectiveness and RWE could drive decisions based on population-driven rather than precision medicine.
 - Failure to recognize that disease population may be comprised subtypes, we risk promoting treatments that work best “on average”.
 - What we really want is to optimize the overall effectiveness to a population by matching each patient to their best treatment options.

Those involved in Big Data research
can't just "throw ball" and hope for the best.

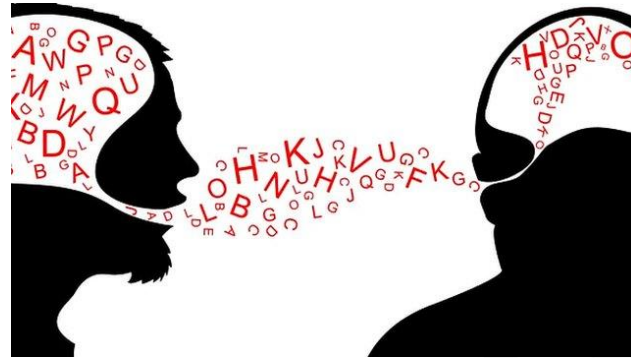


The biggest impact will come when the recipients of big data are consulted up front.



This is why we are here today.

Morning Big Data Projects and Applications



Afternoon Translational Feedback

Time	Speaker	Topic	Time	Speaker	Topic
5m	Larry Alphs	Introduction	5m	Larry Alphs	Review morning
20m	Gayle Wittenberg	The 'Big Data' transformation of drug development: From promise to practice	20m	Robert Temple	Challenges/opportunities in the use of 'Big Data' for the FDA
20m	Patrick Ryan	Generating reliable evidence from real-world data: Lessons from the Observational Health Data Sciences and Informatics (OHDSI)	20m	Luca Pani	Regulatory opportunities and challenges in Europe: From registries to PRIority Medicines (PRIME) and return
20m	Peter Zandi	A model of virtual learning health system for mood disorders	20m	Robert Bossarte	Optimizing use of 'Big Data' for patient identification and disease management—An example from the department of Veterans
20m	Vaibahv Narayan	Harnessing mobile and sensor technology to help patients with diseases of the brain and central nervous system	20m	Alex Contreras	Challenges/opportunities in the use of 'Big Data' in health care services setting (pharmacy/insurer)
20m	Steven Potkin	ADNI as a model of 'Big Data' collaboration	20m	Anirban Basu	Public policy around 'Big Data'
20m	Andrew Kress	Data Linkage Methods and Challenges	20m	Larry/all	Panel Discussion
20m	Larry/All	Panel Discussion	5m	Gayle Wittenberg	Summary/adjourn