FDA U.S. FOOD & DRUG ADMINISTRATION

Leveraging Big Data in Drug Development:
Toxicity Assessment & Signal Detection

Sean Khozin, MD, MPH

Associate Director (Acting): *Oncology Center of Excellence*

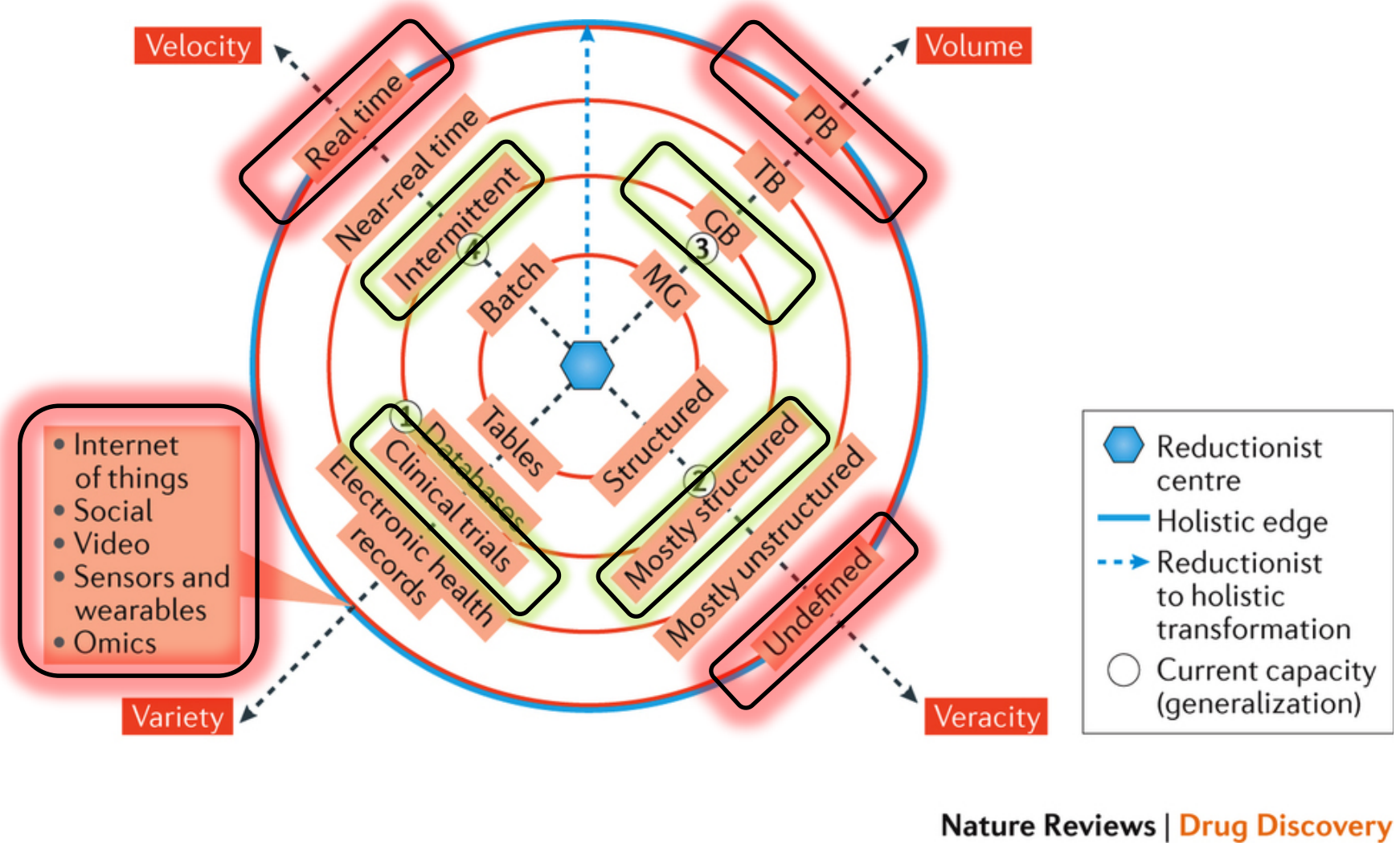
Founding Director: *Information Exchange and Data Transformation (INFORMED)*

Food and Drug Administration

The views in this presentation do not necessarily represent the policies of FDA

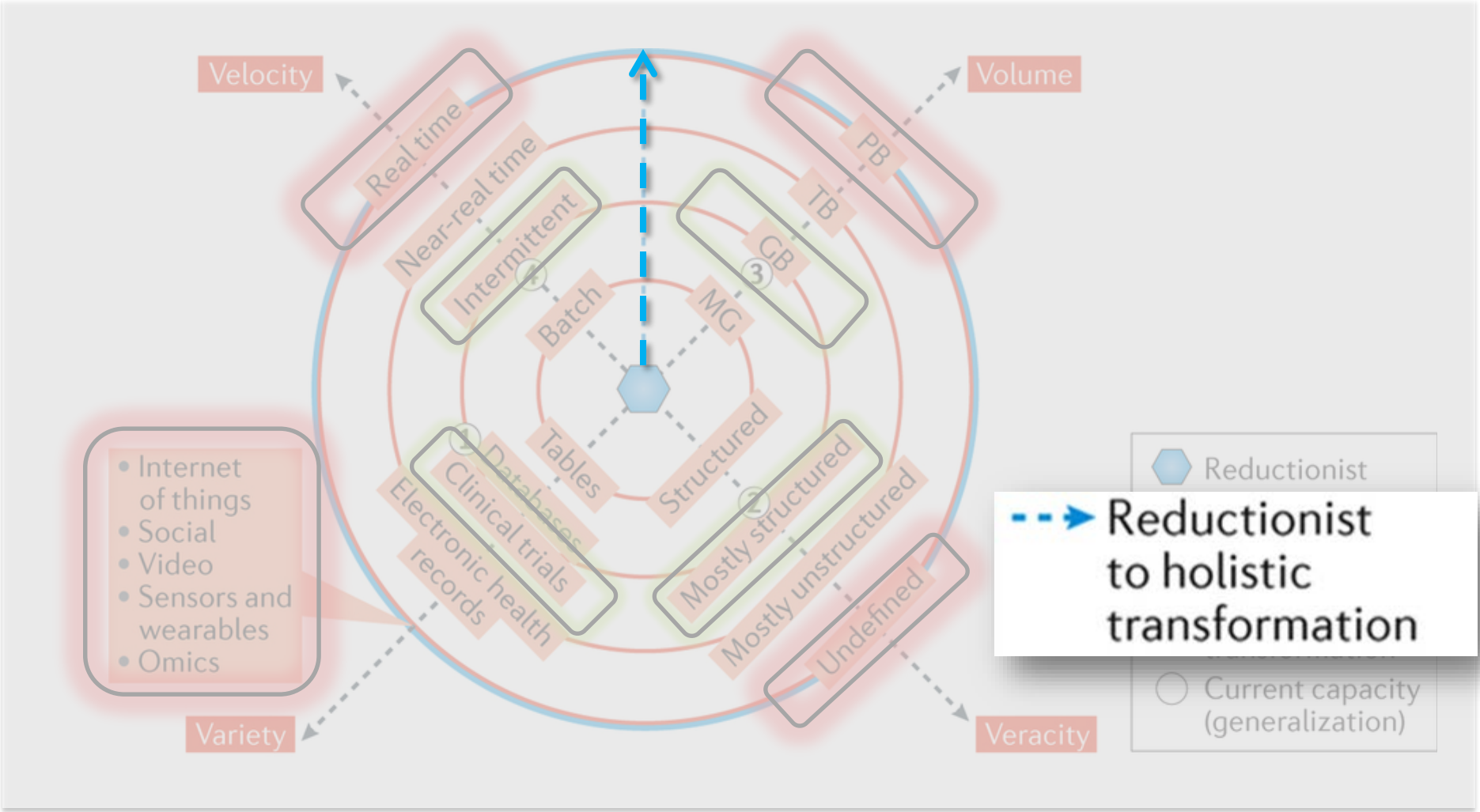
Disclosures: None

What is big data?



Khozin S, et al. Nat Rev Drug Discov. 2017.

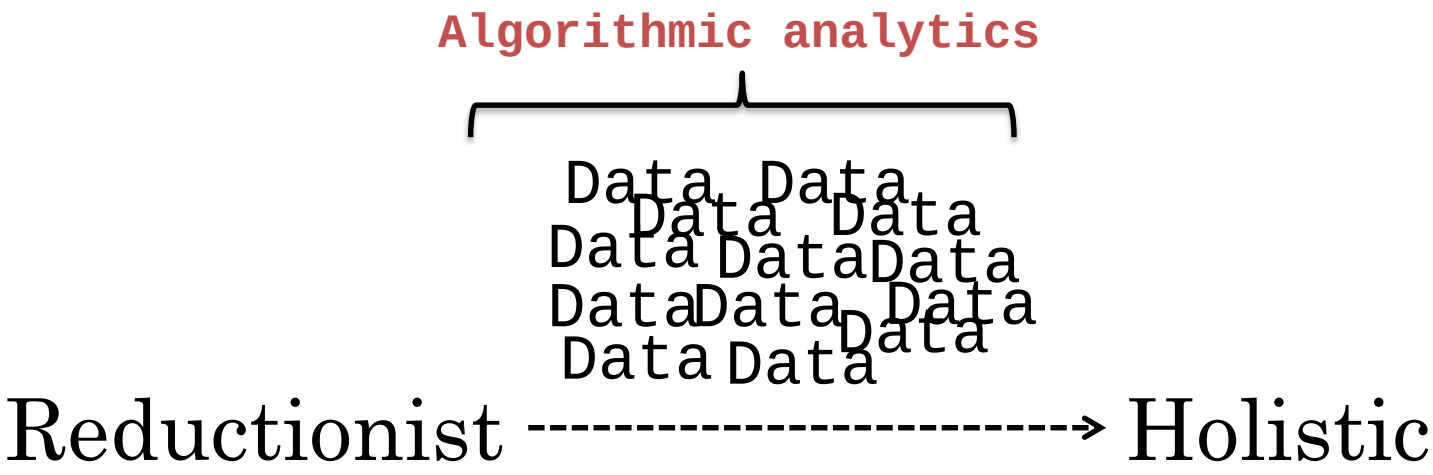
What is big data?



Nature Reviews | Drug Discovery

Khozin S, et al. Nat Rev Drug Discov. 2017.

Inflection point



Information Exchange and Data Transformation (INFORMED)

A holistic approach to oncology regulatory science

Clinical trials

- Aggregation
- Analysis

Novel pipelines

- Real world
- Sensors (IoT)
- Omics

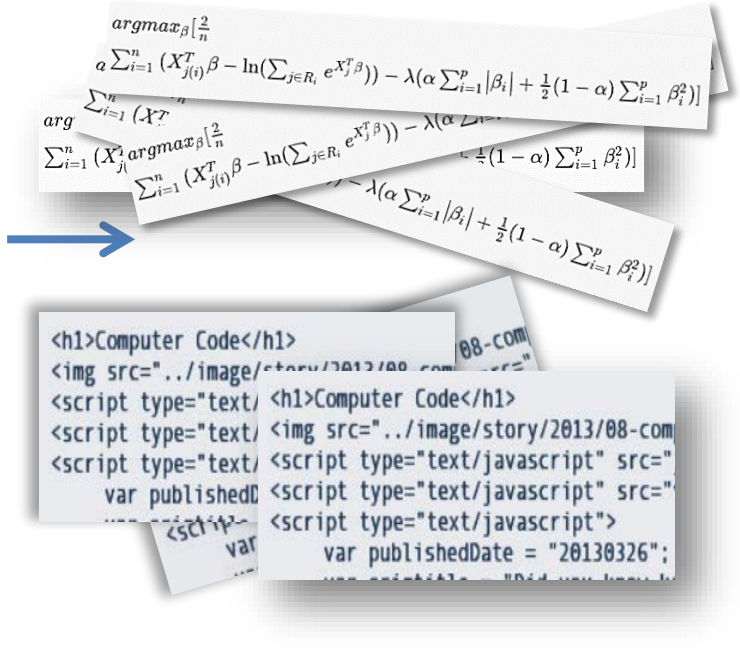
Information Exchange and Data Transformation (INFORMED)

Algorithmic Analytics

Input

Data Data Data Data Data Data Data Data Data Data Data Data Data

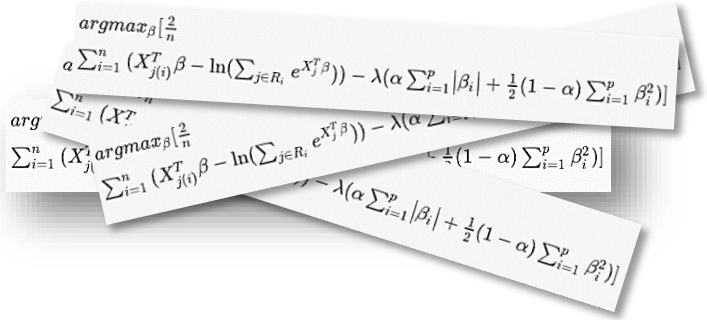
Output

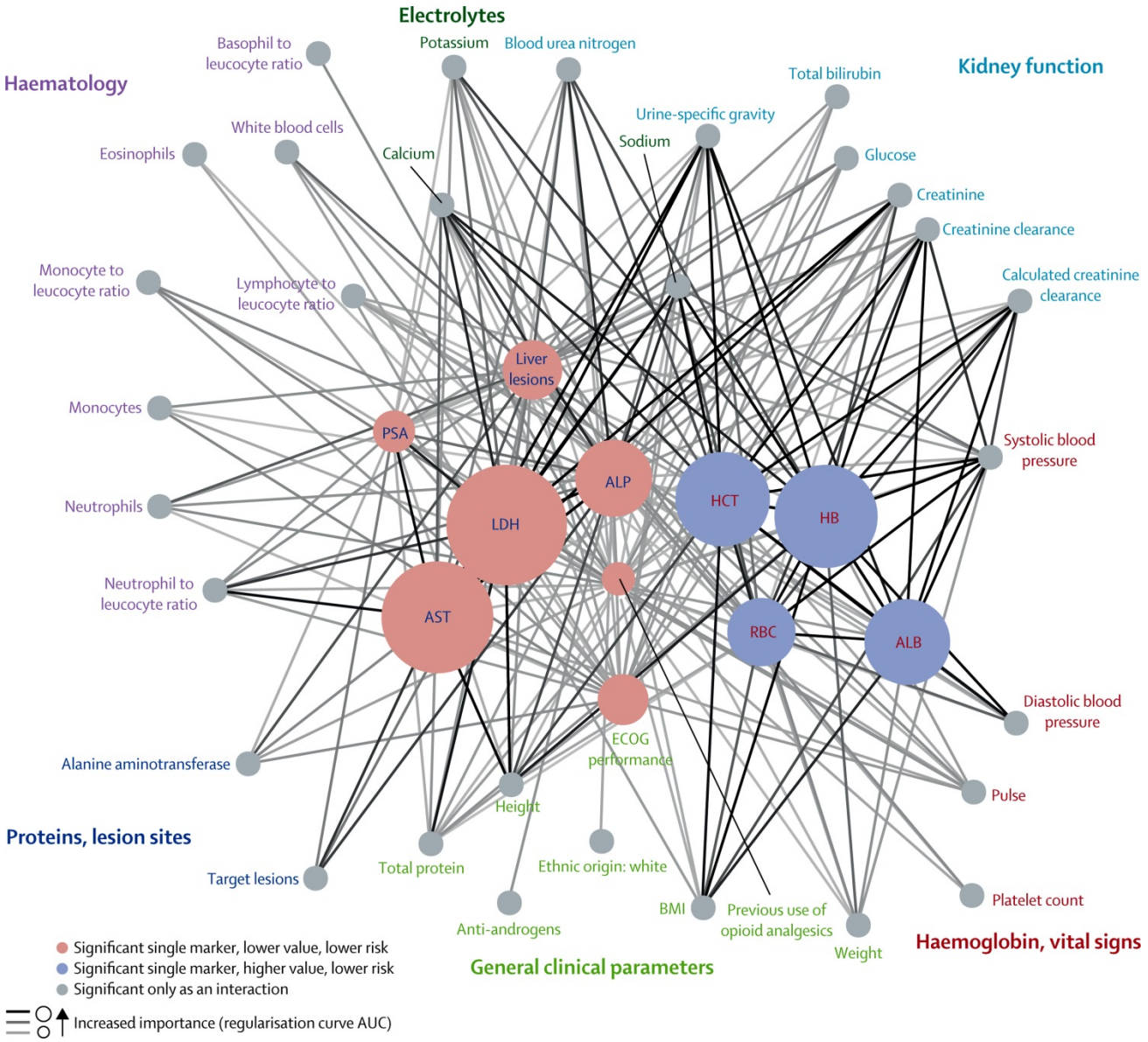


Drug development tools

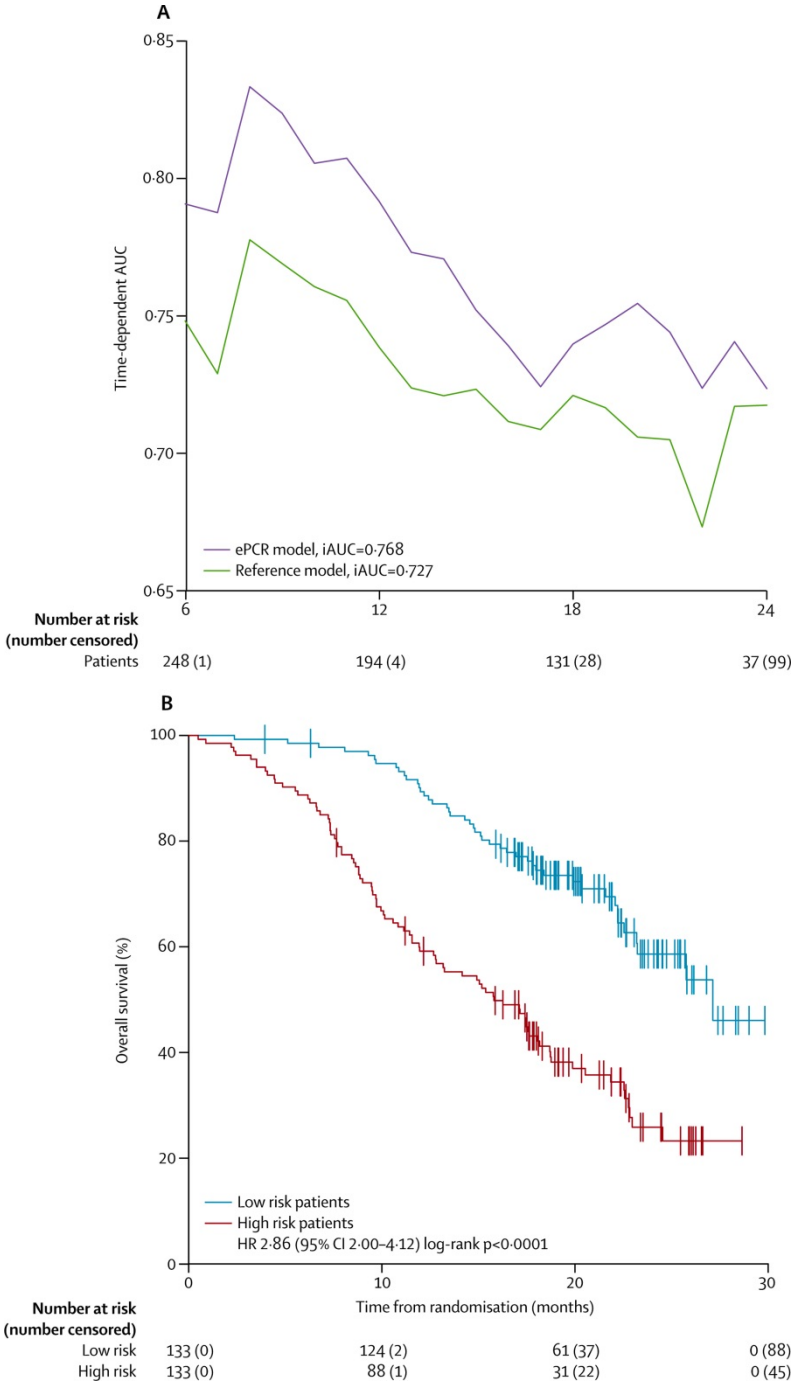
Information Exchange and Data Transformation (INFORMED)

Algorithmic Analytics





Guinney J, et al. Lancet Oncol. 2017.



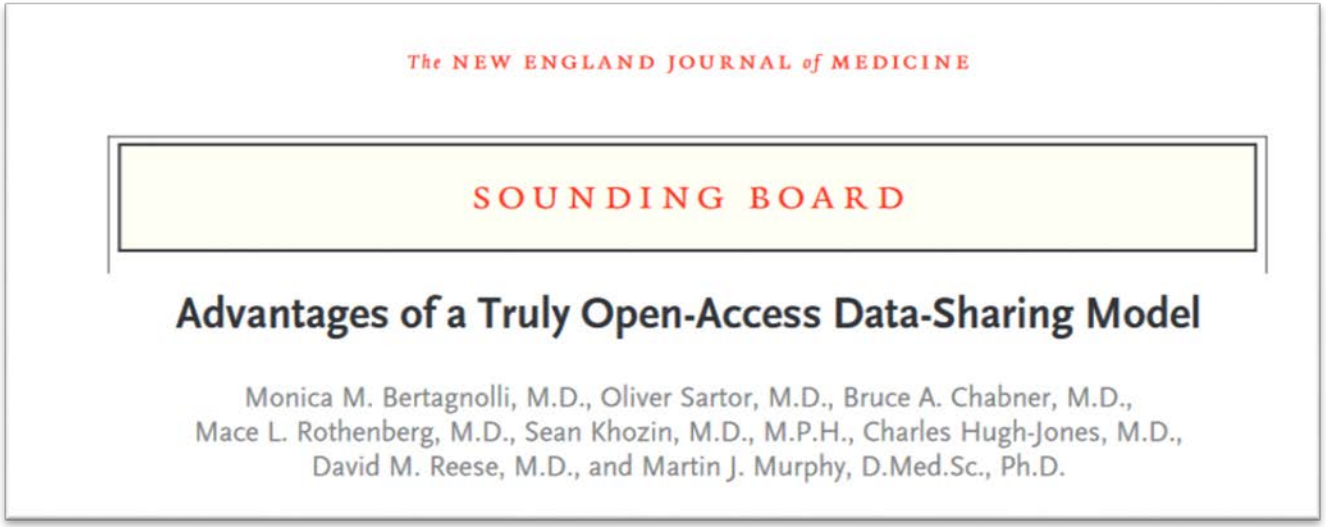
Guinney J, et al. Lancet Oncol. 2017.

Ingredients for successful deployment of algorithmic analytics solutions for safety signal detection

- Leverage existing digital assets through *data sharing*
  - Imaging
  - ECGs
  - Clinical trial/EHRs
- Increase digital footprint
- Match innovation with need

Data sharing

Leveraging existing digital assets



The NEW ENGLAND JOURNAL of MEDICINE

SOUNDING BOARD

Advantages of a Truly Open-Access Data-Sharing Model

Monica M. Bertagnolli, M.D., Oliver Sartor, M.D., Bruce A. Chabner, M.D., Mace L. Rothenberg, M.D., Sean Khozin, M.D., M.P.H., Charles Hugh-Jones, M.D., David M. Reese, M.D., and Martin J. Murphy, D.Med.Sc., Ph.D.

COMPUTERWORLD FROM IDG

Home > Vertical Industries > Health Care

NEWS

IBM Watson, FDA to explore blockchain for secure patient data exchange

The initial focus for blockchain will be oncology-related data exchange

Cardiologist-Level Arrhythmia Detection with Convolutional Neural Networks

Pranav Rajpurkar* PRANAVSR@CS.STANFORD.EDU
Awni Y. Hannun* AWNI@CS.STANFORD.EDU
Masoumeh Haghpanahi MHAGHPANAHI@IRHYTHMTECH.COM
Codie Bourn CBOURN@IRHYTHMTECH.COM
Andrew Y. Ng ANG@CS.STANFORD.EDU

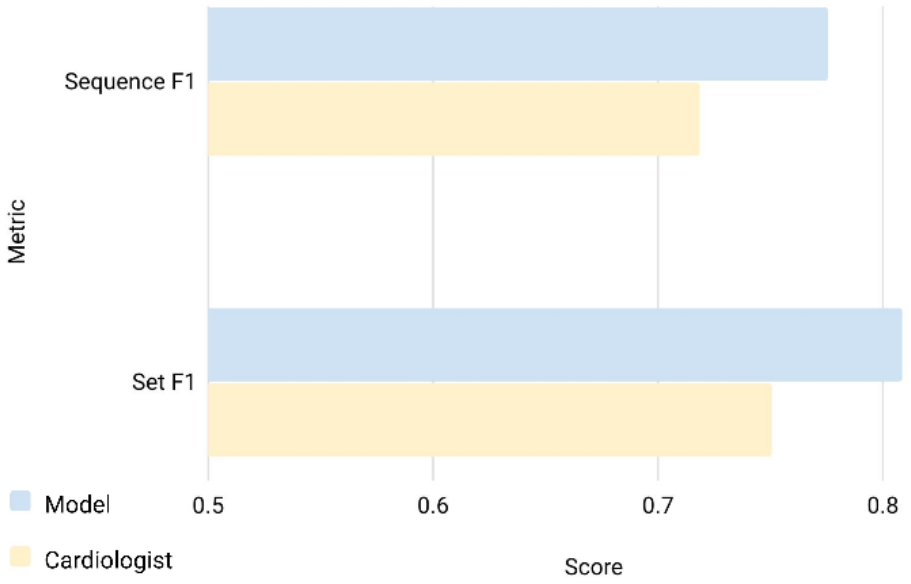


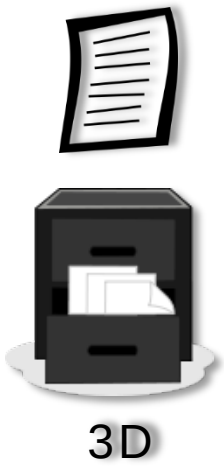
Figure 3. Evaluated on the test set, the model outperforms the average cardiologist score on both the Sequence and the Set F1 metrics.

	Seq		Set	
	Model	Cardiol.	Model	Cardiol.
Class-level F1 Score				
AFIB	**0.604**	0.515	**0.667**	0.544
AFL	**0.687**	0.635	**0.679**	0.646
AVB_TYPE2	**0.689**	0.535	**0.656**	0.529
BIGEMINY	**0.897**	0.837	**0.870**	0.849
CHB	**0.843**	0.701	**0.852**	0.685
EAR	**0.519**	0.476	**0.571**	0.529
IVR	**0.761**	0.632	**0.774**	0.720
JUNCTIONAL	0.670	**0.684**	**0.783**	0.674
NOISE	**0.823**	0.768	**0.704**	0.689
SINUS	**0.879**	0.847	**0.939**	0.907
SVT	**0.477**	0.449	**0.658**	0.556
TRIGEMINY	**0.908**	0.843	**0.870**	0.816
VT	0.506	**0.566**	0.694	**0.769**
WENCKEBACH	**0.709**	0.593	**0.806**	0.736
Aggregate Results				
Precision (PPV)	**0.800**	0.723	**0.809**	0.763
Recall (Sensitivity)	**0.784**	0.724	**0.827**	0.744
F1	**0.776**	0.719	**0.809**	0.751

Table 1. The top part of the table gives a class-level comparison of the expert to the model F1 score for both the Sequence and the Set metrics. The bottom part of the table shows aggregate results over the full test set for precision, recall and F1 for both the Sequence and Set metrics.

Increasing digital footprint

Paper in file cabinets



3D

PDF in "electronic" file cabinets

2D

Missing

The fax of life

It's 2017. Why does American medicine still run on fax machines?

Updated by Sarah Kliff | sarah@vox.com | Oct 30, 2017, 8:00am EDT

TWEET SHARE

Increasing digital footprint

Regulatory watch: Evaluating the potential for digital submission of expedited premarket safety reports to the FDA

Sean Khozin, Meredith Chuk, Tamy Kim, Suranjan De, Sanjay Sahoo, Geoffrey Kim & Richard Pazdur

Nature Reviews Drug Discovery **15**, 670–671 (2016) doi:10.1038/nrd.2016.189
Published online 29 September 2016

Biometric monitoring devices for assessing end points in clinical trials: developing an ecosystem

Stephen P. Arnerić, Jesse M. Cedarbaum, Sean Khozin, Spyros Papapetropoulos, Derek L. Hill, Michael Ropacki, Jane Rhodes, Penny A. Dacks, Lynn D. Hudson, Mark Forrest Gordon, Volker D. Kern, Klaus Romero, George Vradenburg, Rhoda Au, Daniel R. Karlin, Maurizio F. Facheris, Cheryl J. Fitzer-Attas, Ottavio V. Vitolo, Jian Wang, Bradley M. Miller & Jeffrey A. Kaye

Nature Reviews Drug Discovery **16**, 736 (2017) doi:10.1038/nrd.2017.153
Published online 22 September 2017

Matching innovation with need

Thank you