

Rapid Data Insights into Rare Diseases:

From External Data to End-to-end Automation and Data Intelligence

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Advancing Clinical Research is challenging...

Perennial challenge of **patient recruitment** remains one of the top challenges

Complexity of trials is increasing with novel therapies and trial designs

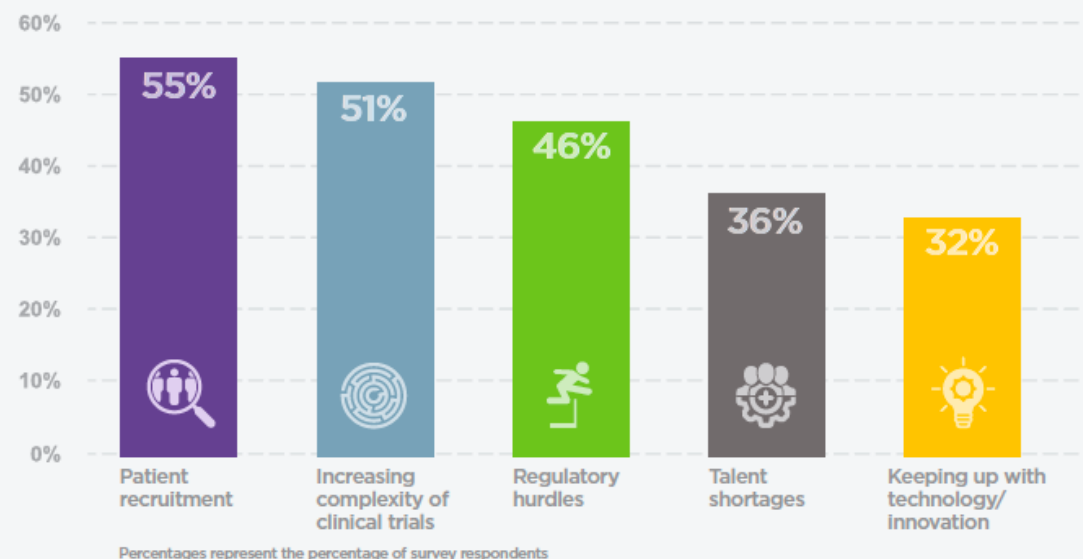
Continually evolving Regulatory requirements add pressure on sponsors, CROs, sites and other stakeholders to drive change in process & tools

Technology innovation, accelerated by the pandemic, has increased **adoption of Hybrid & Decentralized Clinical Trials** bringing in millions of new data points per study

PPD surveyed more than 150 drug developers worldwide to gather insights on the state of the drug development industry.



Drug developers reported that the COVID-19 pandemic and evolving industry landscape have created various disruptions, increasing challenges in executing clinical trials.



Source: Top 5 Challenges and Opportunities Drug Developers Face in Executing Clinical Trials by The Editors of the Insights Hub, PPD clinical research business of Thermo Fisher Scientific

Protocol Customization and Clinical Trial Performance

Protocol Design Practice

Phase III Pivotal Trials (Means)	2010	2020	10-Year Change
Total Endpoints	13	22	69.2%
Total Eligibility Criteria	34	30	-11.8%
Total Procedures	187	263	40.6%
Total Countries	9	15	66.7%
Total Investigative Sites	65	104	60.1%
Procedures per Visit	11	13	18.2%
Total Patients Randomized	597	632	5.9%
Total Data Points Collected	929,203	3,560,201	283.2%

Protocol Performance Experience

Phase III Pivotal Trials	10-year Change
Initiation Duration (approval to FPFV)	27.2%
Enrollment Duration (FPFV – LPLV)	36.9%
Closeout Duration (LPLV to DBL)	16.3%
Total Substantial Amendments	113.3%
Drop-Out Rates	105.1%

Source: Tufts CSDD



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Tufts Center for the
Study of Drug Development
TUFTS UNIVERSITY

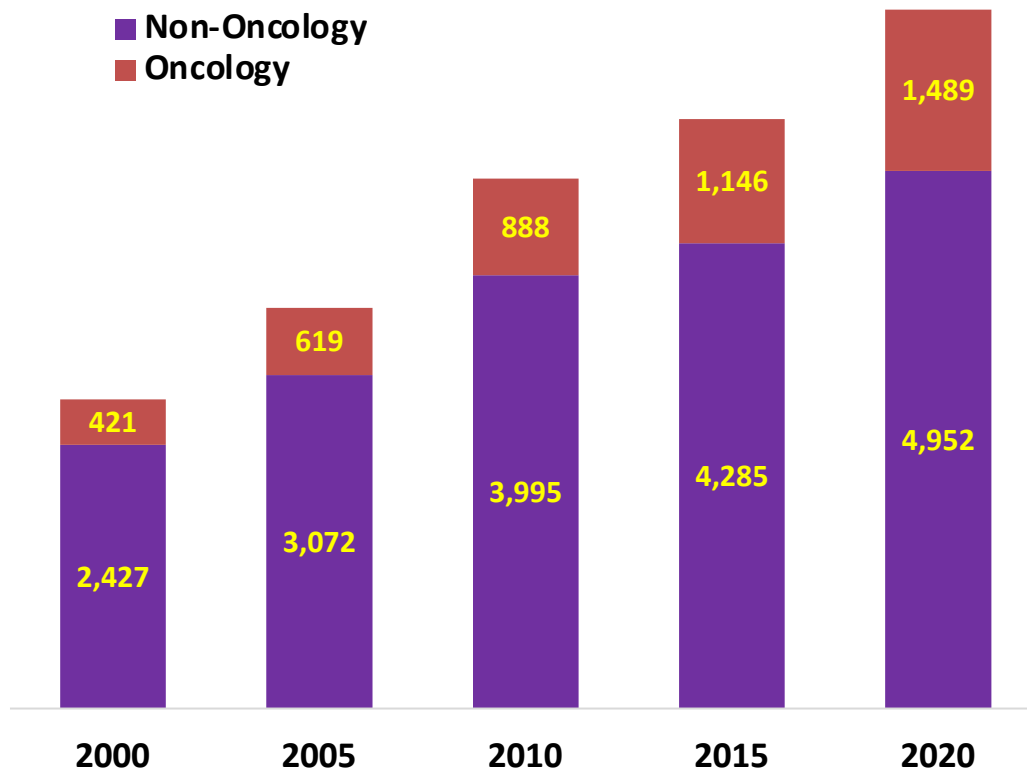
Data-Centricity: The Big Data Flip



- Past: **80%** of study data was entered by the site and **20%** was external data
- Present: **20%** of study data is entered by the site and **80%** is external data
- Past: Processes were **manual**, dependency and latency was high
- Present: Focus is on **automation**, self-serve and real-time insights
- Past: Focus was on **restricting access**
 - Present: Focus is on **data democratization**

The Evolving Global Pipeline of Drugs in Development

Total Drugs and Biologics in Clinical Testing



Changing Pipeline Composition

	Companies with Active Drugs in R&D	Percent of Drugs in R&D that are Personalized Therapies	Percent of Drugs in R&D Targeting Rare Diseases
2000	1,043	3%	9%
2010	2,207	19%	14%
2020	4,105	61%	34%

We need to cross the chasm of Data Infrastructure & Analytics



Rapid digitalization
and increasing data
complexity

Complex digital
ecosystems

Artificial
Intelligence and
data sharing



Cycle time
Reduction

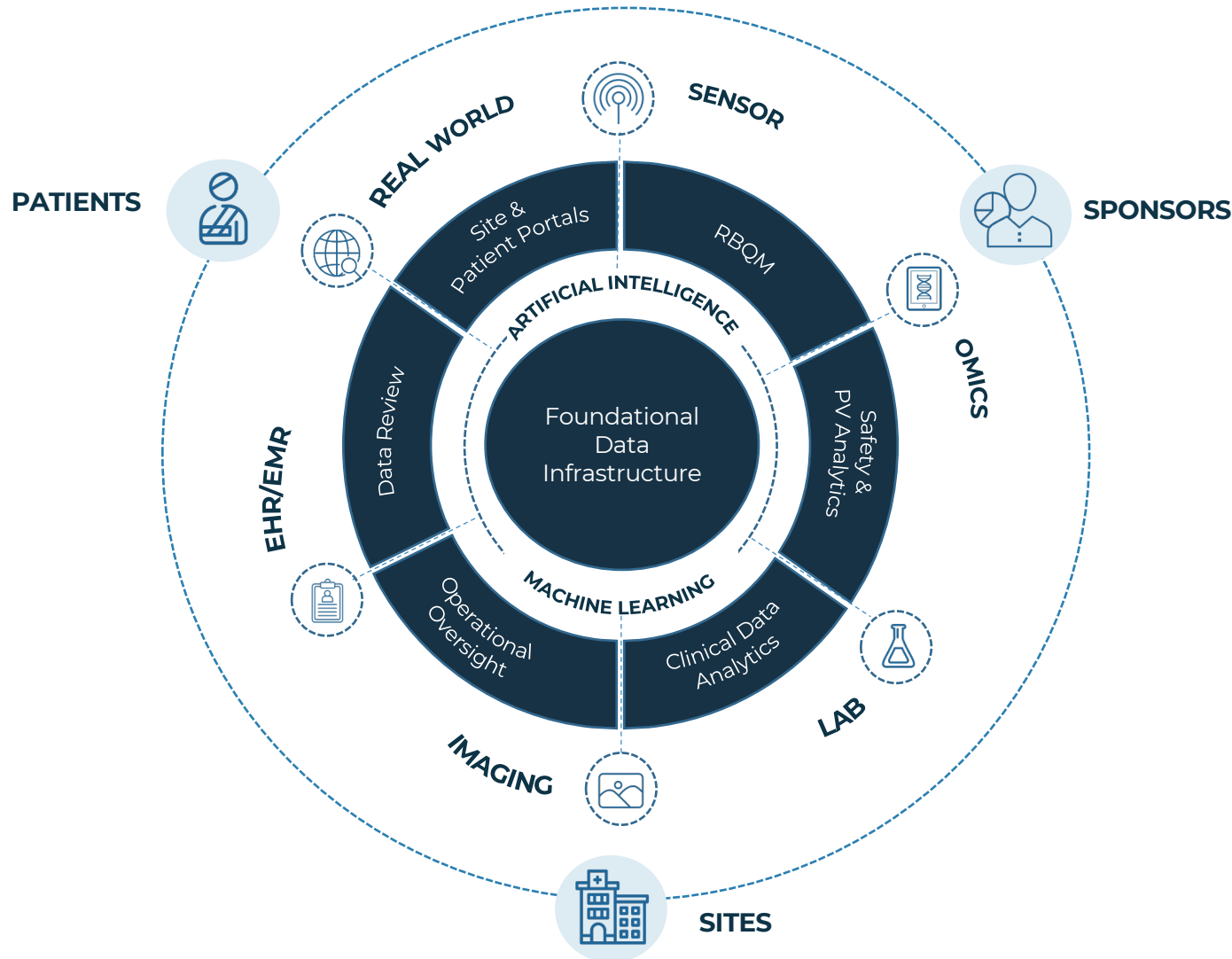


Overall business
system efficiencies



Long-term value
creation with
innovation-led
growth

The data affair is getting more complex



What's shaping our data ecosystem?

Breakthrough innovations, rapid technology advancements

High volume, variability of EMR and RWD data from several sources

AI/ML now in use pervasively for automation and insights

Provide analytics to patients and sites
analytics beyond sponsors

There are critical data needs that should be fulfilled

Security &
Privacy

Collection &
Management

Standardization
&
Interoperability

Integration &
Analysis

Timeliness &
Real-Time
Monitoring

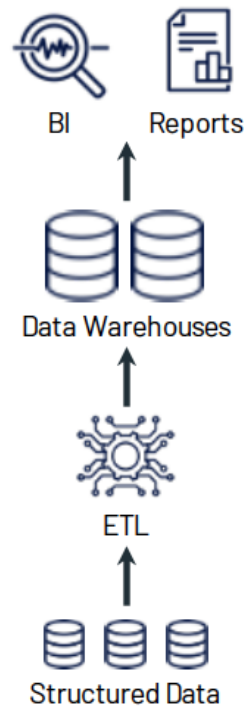
Quality Control

Regulatory
Compliance

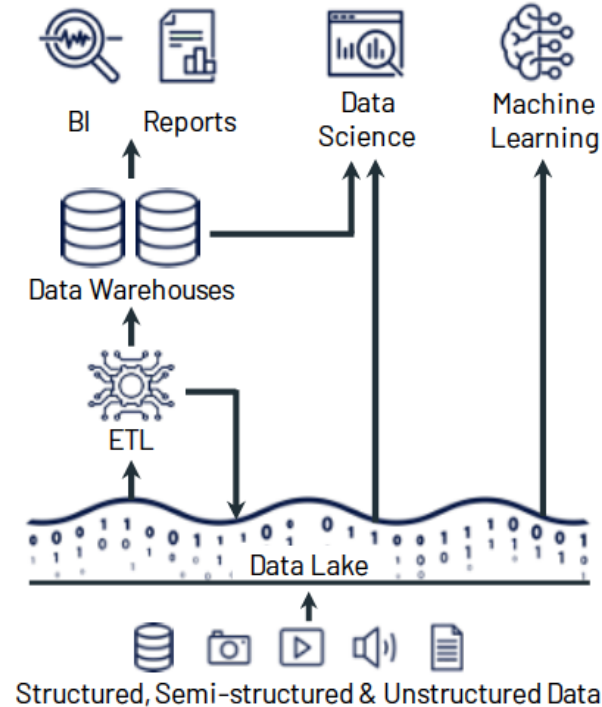
Sharing &
Collaboration



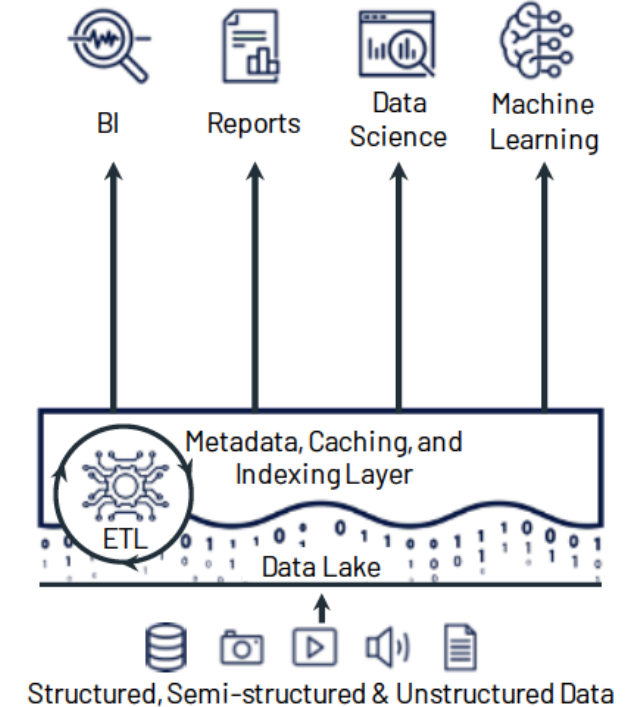
Data technology architectures have evolved



(a) First-generation platforms.



(b) Current two-tier architectures.

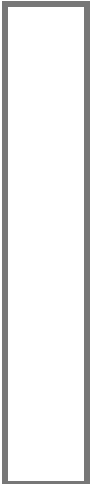


(c) Lakehouse platforms.

Source : [Lakehouse: A New Generation of Open Platforms that Unify Data Warehousing and Advanced Analytics](#) by Michael Armbrust, Ali Ghodsi, Reynold Xin, Matei Zaharia



Rare Disease Case Study – bluebird bio



bluebird bio's experience

Sean McNiff, Director for Clinical Programming

- He has worked at bluebird for over 7 years.
- Prior to bluebird, Sean was with Genzyme/Sanofi for almost 19 years, Parexel for 4 years, and spent several years programming list-fulfillment, personnel, and logistics systems.
- His 30-year industry experience has been a combination of roles like IT systems manager and business system owner.
- These roles cover solution development, delivery, enablement, and support in a variety of environments.
- These solutions range from clinical data acquisition, safety data processing, study planning and metrics, to data cleaning and exploration.
- Sean is a decorated U.S Army Veteran.
- He is a graduate from Merrimack College and lives in Wakefield, MA with his Travel Agent wife, Mary.
- His two grown children have flown the nest and are off following their own careers – Brian is a Data Analyst and Amanda is a Physical Therapist.



PHUSE SDE Recording

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Key Takeaways



Digital trials are leading to **increasing data complexity** and rendering traditional data management approaches inefficient



Technology architectures are evolving to leverage some of the **newer innovations like cloud, big data, AI/ML** etc.



It is critical to adopt **novel approaches** to make data available near real-time for critical decision-making



Verticalized data Lakehouse architectures are well positioned to deliver ROI



For companies in **rare disease**, integrated data and new, modern digital data workflows are helping enable the automation and rapid insights that are critical to precision medicine





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Single Day Events



Thank You

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