MEDIDATA IS ADVANCING RARE DISEASE TRIALS, ONE PATIENT AT A TIME.

RARE DISEASES BY THE NUMBERS

400+ MILLION
affected globally,
25M in the U.S. alone[1]

~7,000
Rare Diseases identified[1]

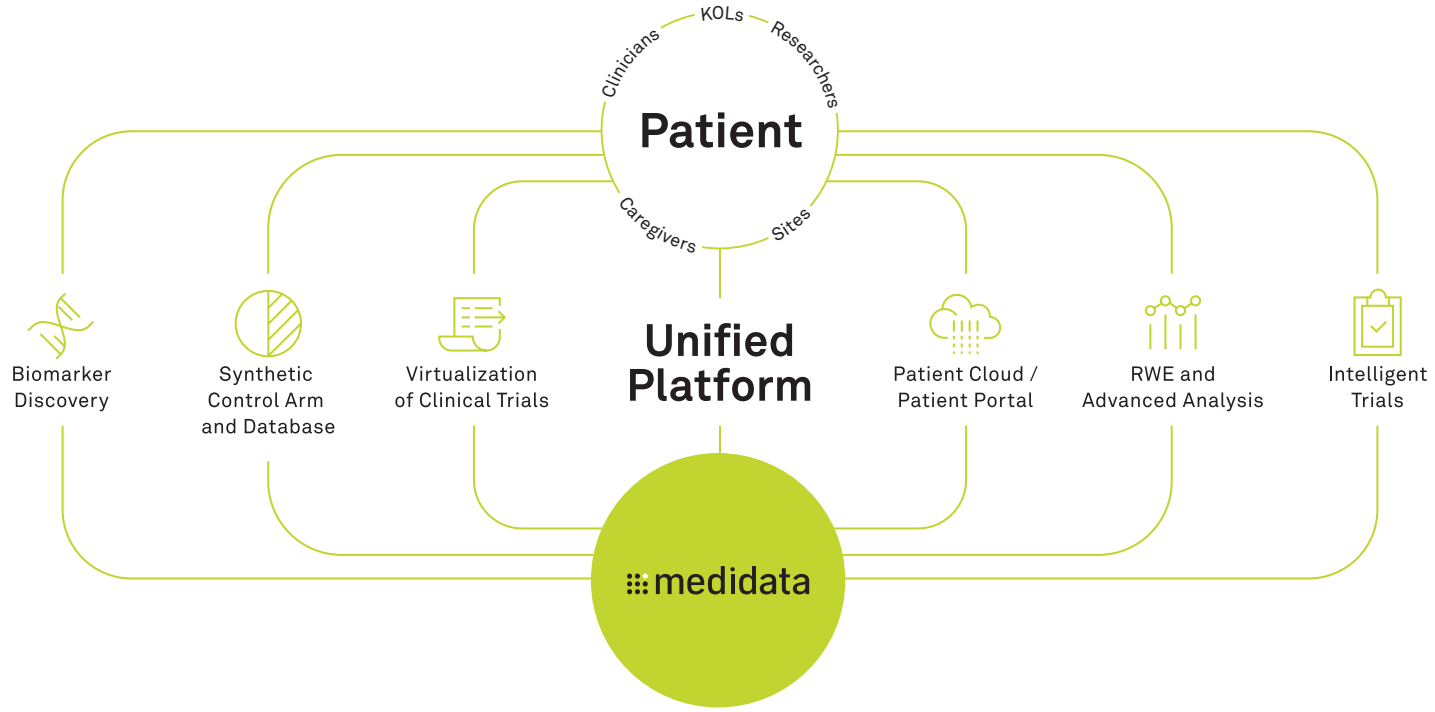
73
new therapies
in the last 3 years

50%
begin in childhood[2]

95%
do not have an approved drug[2]

1/3
R&D pipeline by 2024[3]

WHY MEDIDATA CAN HELP EXPEDITE CRITICAL RARE DISEASE TRIALS



MEDIDATA HAS THE TRIAL EXPERIENCE, TECHNOLOGY, DATA, AND EXPERTISE IN RARE DISEASE TRIALS*

1,240+
Rare Disease studies

170+
unique sponsors with Rare Disease trials

65K+
sites setup to conduct Rare Disease trials

190K+
enrolled Rare Disease patients

[1] https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3932940/ [2] https://globalgenes.org/rare-facts/ [3] https://bit.ly/2PtfCg3

* Based on U.S. Rare Disease Definition

Visit medidata.com/en/rare-disease/ to learn more.

medidata joins DASSAULT SYSTEMES

SOME CHALLENGES OF RARE DISEASE TRIALS AND HOW MEDIDATA HELPS

INDUSTRY CHALLENGES	MEDIDATA SOLUTIONS
PATIENT ENROLLMENT	
• Small cohorts • Predominantly pediatric populations • Geographically scattered patient base • Divergent landscape of clinical trial regulations	• Faster study and site set up • Enrollment and patient tools across borders and languages • Market-leading technology used by sites globally • Ability to integrate multiple diverse data sets and use predictive analytics to identify high priority sites and investigators via Intelligent Trials
PATIENT RETENTION	
• Predominantly life-threatening and debilitating diseases • Sites geographically remote from patients • Lack of patient experience for participation outside of sites • Placebo/standard therapy controls (Trial Design) disincentivized to patients	• BYOD/Hybrid/Virtual Trials flexibility for at-home data collection • Synthetic Control Arm™ reducing patients number in trial • Patient Centricity by Design™ • Lower patient burden with technology like eConsent • Patient Cloud Help Desk dedicated to patient support
LIMITED DATA	
• Difficulty acquiring and managing patient data • Limited Real-World Evidence and data • Lack of biomarker data to inform prognosis and treatment	• Patient-level data from many historical trials • Clinical data integration with omic data to accelerate biomarker discovery • Advanced insights to understanding the impact of new drugs on rare diseases in the real world
CLINICAL TARGETS	
• Unclear diagnostic criteria and testing strategy • Lack of validated surveys for Patient Outcomes Assessments • Complex biomarker identification to differentiate patients	• Statistically powered end-points with fewer patients • Patient-centric technology for expedited development and reduced patient burden • Replicate the outcomes of a randomized control arm by using propensity score matching and historical trial data through a Synthetic Control Arm
CLINICAL TRIAL EXECUTION	
• Identification and retention of qualified investigators • Non-site data collection • Ineffective traditional study designs	• Medidata Rave Clinical Cloud™ powers complex trials through its unified platform • Patient data easily collected from their own devices • Streamlined workflows across a global study ecosystem • Optimized trial design • Advanced analytics for trial design, feasibility, and monitoring through Intelligent Trials • Support and expertise in virtual hybrid studies

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