

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. alone¹

~7,000
Rare Diseases identified¹

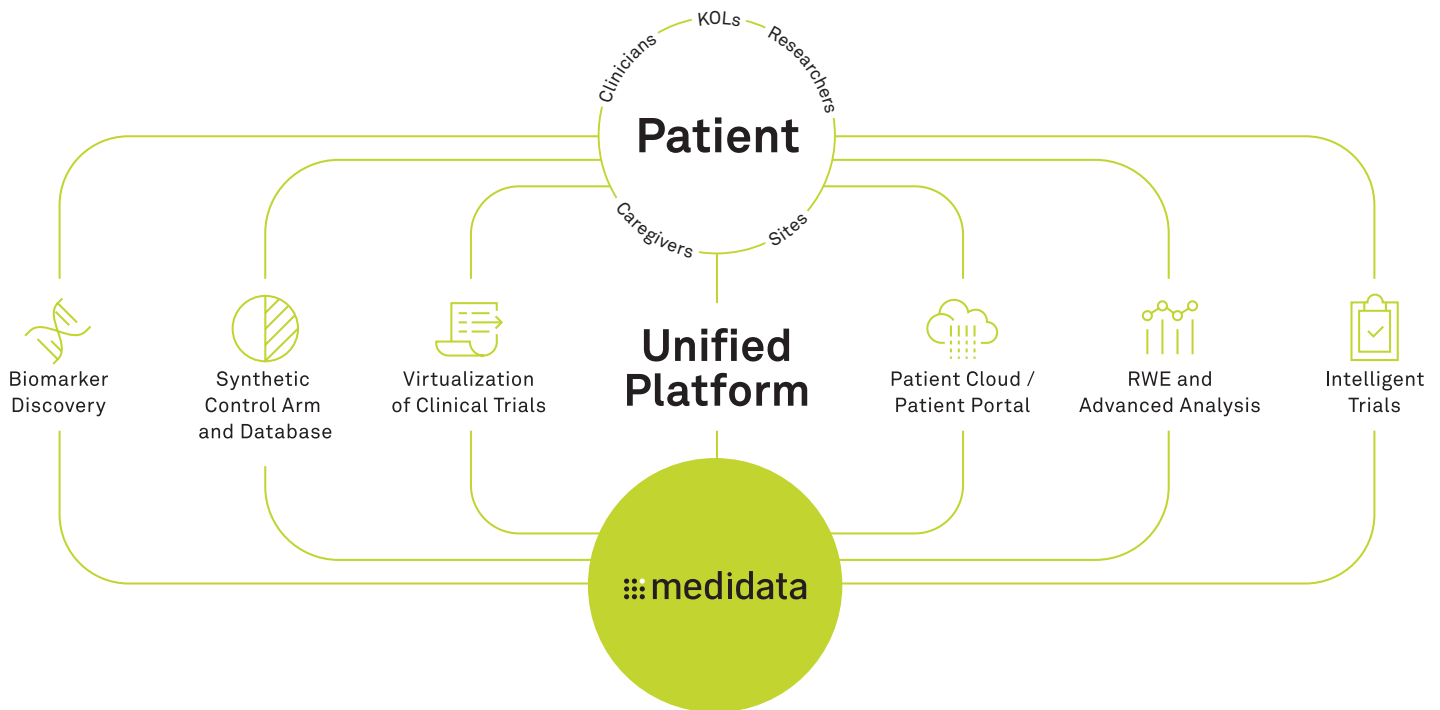
73
new therapies
in the last 3 years

50%
begin in childhood²

95%
do not have an approved drug²

1/3
R&D pipeline by 2024³

WHY MEDIDATA CAN HELP EXPEDITE CRITICAL RARE DISEASE TRIALS



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

1,635+

Rare Disease studies

287+

unique sponsors with
Rare Disease trials

80K+

sites setup to conduct
Rare Disease trials

245K+

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



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