Medidata is Advancing Rare Disease Trials, One Patient at a Time

Rare Diseases by the Numbers

300M+
affected globally, 25M in the US alone[1]

~7000
Rare Disease identified[1]

6+ years
The average time it takes for Rare Disease patients to receive an accurate diagnosis[2]

70%
begin in childhood[1]

95%
do not have an approved drug[2]

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

Medidata Has The Trial Experience, Technology, Data, and Expertise In Rare Disease Trials*

2540+
Rare Disease studies

400+
Unique sponsors with Rare Disease trials

113K+
Sites setup to conduct Rare Disease trials

552K+
Enrolled Rare Disease patients

[1]https://www.rarediseaseday.org/ | [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	Faster study and site set up
	Predominantly pediatric populations	Enrollment and patient tools across borders and languages
	Geographically scattered patient base	Market-leading technology used by sites globally
	Divergent landscape of clinical trial regulations	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	Decentralized Trials flexibility for at-home data collection
	Sites geographically remote from patients	Synthetic Control ArmTM reducing patients number in trial
	Lack of patient experience for participation outside of sites	Lower patient burden with technology like eConsent
	Placebo/standard therapy controls (Trial Design) disincentivized to patients	Patient Cloud Help Desk dedicated to myMedidata and patient support
Limited Data	Difficulty acquiring and managing patient data	Patient-level data from many historical trials
	Limited Real-World Evidence and data	Clinical data integration with omic data to accelerate biomarker discovery
	Lack of biomarker data to inform prognosis and treatment	Advanced insights to understanding the impact of new drugs on rare diseases in the real world
Clinical Targets	Unclear diagnostic criteria and testing strategy	Statistically powered end-points with fewer patients
	Lack of validated surveys for Patient Outcomes Assessments	Patient-centric technology for expedited development and reduced patient burden
	Complex biomarker identification to differentiate patients	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	Medidata Platform powers complex trials through its unified platform
	Non-site data collection	Patient data easily collected from their own devices
	Ineffective traditional study designs	Streamlined work ows 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