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
- **50%** begin in childhood
- **~7,000** Rare Diseases identified
- **95%** do not have an approved drug
- **73** new therapies in the last 3 years
- **1/3** R&D pipeline by 2024

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

* Based on U.S. Rare Disease Definition

Visit medidata.com/en/rare-disease/ to learn more.
# SOME CHALLENGES OF RARE DISEASE TRIALS AND HOW MEDIDATA HELPS

## INDUSTRY CHALLENGES

### PATIENT ENROLLMENT
- Small cohorts
- Predominantly pediatric populations
- Geographically scattered patient base
- Divergent landscape of clinical trial regulations

### 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

### LIMITED DATA
- Difficulty acquiring and managing patient data
- Limited Real-World Evidence and data
- Lack of biomarker data to inform prognosis and treatment

### CLINICAL TARGETS
- Unclear diagnostic criteria and testing strategy
- Lack of validated surveys for Patient Outcomes Assessments
- Complex biomarker identification to differentiate patients

### CLINICAL TRIAL EXECUTION
- Identification and retention of qualified investigators
- Non-site data collection
- Ineffective traditional study designs

## MEDIDATA SOLUTIONS

### PATIENT ENROLLMENT
- 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
- 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
- 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
- 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
- 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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