

### **TAILORED SOLUTION FOR RARE DISEASE STUDIES**

#### THE SITUATION



**50**%

impact children.

~7,000 rare diseases and growing



Half of those with a rare disease spend

3+ years

looking for diagnosis and treatment.

Source: National Organization for Rare Disorders

#### THE CHALLENGES

Limited disease knowledge Undefined endpoints

Complex regulatory pathways

Few patients, many countries

Site targeting and engagement Unknown obstacles

Postmarketing commitments

### THE SOLUTION

# **Insights** that streamline your path



- Natural history studies and disease registries
- Biomarker ID and validation
- Patient-centric protocol design
- Clinical outcome assessment
- Alternative study designs

### **Experience** that matters



- Rare disease delivery unit, operations heads and project leaders
- Seasoned CRAs
- Project integration managers
- Rare disease experienced functional leads

### Operational approaches that fit



- Tailored site ID approaches
- Magnet site network and rare disease delivery model
- Home health nurses
- Travel concierge
- Telemedicine technology
- Centralized monitoring
- Drug and lab kit logistics
  IQVIA Infosario™ dashboard

Study dashboard

### **Engagement strategies** that deliver



- Multifaceted patient recruitment
- Social media listening research
- Digital and mobile recruitment
- Expertise in building patient communities
- Patient advocacy relationships
- Clinical Trial Educators

### THE VALUE PROMISE

IQVIA helps anticipate and address the complex challenges of rare disease studies by bringing together a specialized team with rare disease experience and therapeutic expertise, along with tailored patient-centric approaches to global study planning and execution in order to speed treatments to market to transform the lives of patients and their families

#### OPERATIONAL MODELS TAILORED TO THE NEEDS OF PATIENTS AND THE STUDY



On-site monitoring

Centralized monitoring

**245** rare disease studies in 96 countries since 2011

# **Centers of Excellence**

for pediatrics and rare diseases

To learn more about how we can help you simplify rare disease drug development, contact us at **iqvia.com**.

### **CONTACT US**

**IP & lab logistics**