Evidera | PPD

# Helping you meet the unique needs of **clinical development** and **commercialization of rare disease treatments**

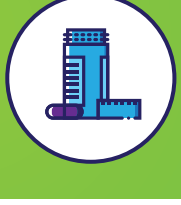
Created **clinical development strategy to maximize market value** for a second-in-indication rare neurological disorder treatment, **resulting in >70% enrollment in SIX months**

**350+** ***rare disease research projects*** *conducted in the past five years*

**75+** ***rare disease indications*** *worked on in the past five years*

**Issued key takeaways on FDA draft guidance:**
***Rare Diseases:*** *Common Issues in Drug Development, Guidance for Industry →*

**50+** ***rare disease drugs*** *supported in the past five years*



Supported the validation and regulatory submission of multiple COAs used as **important efficacy endpoints in rare disease drug development programs** that have **achieved FDA and EMA labeling claims**

**10+** *countries where we have* ***conducted rare disease projects***

Actively involved in the **Rare Disease and Pediatric Center of Excellence**, a team of over 20 thought leaders across PPD focused on surfacing insights and innovation in the rare disease space

**15+** ***peer-reviewed articles, research posters, and presentations published*** *in rare diseases on average per year*

Published two key and **highly-cited articles on hereditary angioedema**, a rare autosomal dominant disorder

1. *The humanistic burden of hereditary angioedema: Impact on health-related quality of life, productivity, and depression →*
2. *Economic costs associated with acute attacks and long-term management of hereditary angioedema →*

Dedicated the entire spring 2019 issue of our publication, **The Evidence Forum**, to rare diseases →

**TOPICS INCLUDED**

**Natural History Studies** in Rare Diseases and Genetic Biomarkers →

**Patient Engagement in Clinical Trial Protocol** Design and Recruitment Strategies: What Does It Mean for Orphan Drug Manufacturers? →

**Clinical Outcome Assessment** Selection for Rare Disease Trial Programs →

How **Social Media** Can Be Used to **Understand What Matters** to People with Rare Diseases →

**evidera.com**