
Over 90% of rare diseases have no treatment...

- There are more than 6,000 known rare diseases...
- Approximately 80% of them have a genetic component... 75% affect children...
- And most rare diseases are chronic conditions, many of which progress overtime and are potentially fatal...

Understanding the rare disease framework is key to be able to offer study participants adapted solutions and as such, positively contribute to important and necessary clinical research conducted on rare diseases.

Kayentis offers an extensive experience in rare disease clinical trials, in indications such as:

- Prader-Willi Syndrome
- PIK3CA-related overgrowth spectrum
 - Auto-Immune Hepatitis (AIH)
 - Hypoparathyroidism
 - Sjögren's syndrome
 - Acute Vertigo

RARE DISEASE CASE STUDY

Kayentis offers an extensive experience in rare disease clinical trials

IN INDICATIONS SUCH AS:


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UP TO 38 COUNTRIES

AROUND 2,500 PATIENTS ABOUT 1,000 SITES ACROSS THE GLOBE: Europe – US – Asia – Oceania

ASSESSMENTS

- **PROs:** Appetite NRS, EQ-5D-5L SELF, Vertigo Intensity, DSS-SF, Nausea Severity, DHI, Vestibular Disorders Activities of Daily Living Scale - PROMIS Fatigue, SF 13A, SF-12, Injection-Site Reaction Questionnaire
- **Pediatric PROs:** PedsQL™ Young Adult (ages 18-25), PedsQL™ Pediatric (ages 12-18)
- **ObsROs:** EQ-5D-5L PROXY, PedsQL™ Adult, HQ-CT, CgGIS-H, CgGIC-H, Zarit Burden Interview Caregiver, DBC-P
- **eDiary:** Vestibular Disorders Activities of Daily Living Scale - eDiary for drug intake (IMP daily injection compliance/ Site injection/ Calcium and Vit D supplements intake count/ other treatment intake - Sjögren syndrome patient diary - SSSD
- **ClinROs:** PhGA / ESSDAI / CDAI



Download the case study
Contact us for more information

Key Words:

- eCOA

- ePRO
- eDiary
- rare disease
- clinical trial

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