RARE DISEASE CASE STUDY

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

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

Challenges of rare diseases studies

Specific patient population

✓ LIMITED NUMBER OF PATIENTS

- Patients are **spread around the world** and not necessarily numerous in a given country, so recruiting patients is more difficult.
- As patients are rare, **missing data** is more difficult to compensate in the case where there is a lack of compliance.

✓ PEDIATRIC POPULATION

- Clinical trials in the pediatric population face specific challenges which are compounded when dealing with rare diseases. As rare diseases often affect children, there is added complexity to navigate.

✓ DISCREPANCY IN THE WAY SYMPTOMS OCCUR AND ARE PERCEIVED BY THE PATIENTS

- Symptoms can vary not only from patient to patient but can change day to day in the same patient, which leads to difficulties in making statistical evaluations.
- Cleaning of collected data can generate higher number of **queries** and more **data management activities**.

Higher level of study complexity than in non-rare disease trials

✓ COMPLEX PROTOCOLS

- Rare disease protocols involve a much **higher average number of countries and sites**, require more planned patient visits and **generate considerably more clinical research data**.
- Frequently designed with multiple arms, complex rare disease studies require dedicated resources and solid technology to build the appropriate eCOA solution.

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.

✓ VARIOUS AGE RANGES AND MULTIPLE USERS

- **Several questionnaire versions** are usually required to meet the needs of the protocol and the various ages of the patients.
- Each patient must have **access to the relevant questionnaires** at the right time.
- Involving **caregivers** requires the implementation of specific accounts, appropriate questionnaires, and robust audit tracking.
- Rare diseases trials can also require the implementation of **new questionnaires** which might need more back and forth with license holders.

✓ LONG STUDY TIMELINES

- Study duration may need to be extended with eDiaries & ePROs to be completed over long periods, which can impact both project and data management activities.
- The high regulatory scrutiny and numerous protocol amendments can generate timelines extension.
- Adding countries during the course of the study often happens. This increases the logistics and translations requirements.

KAYENTIS KEY SUCCESS FACTORS

We have developed a deep knowledge of patients' needs

- **We work in collaboration with patients** to understand their needs and specific requirements.
- We directly ask patients about their perceptions and experience of clinical research via surveys (lien) and interviews.
- We collaboratively design solution improvements during patients focus groups in order to make sure the technology evolutions are fit for purpose.

- **The scientific background of our teams and the thorough medical review we do of clinical trial protocols** allow for a deep understanding of the trial complexity and adapt the response to the study needs.

We have the ability to manage complexity

- We are **experts in complex builds** with multiple triggers, multiple accounts, dealing with large range of questionnaires in multiple languages.
- We combine both **strong data management teams & robust functionalities** to support studies from specifications to close-out.
- We have strong **experience in pediatric clinical trials:**
  - Management of multiple-questionnaire visits.
  - Management of numerous questionnaires versions and translations across broad age groups thanks to robust technology and close relationships with LVAs.

We propose options to manage compliance

- **Reminders for study tasks**
  - eDiaries customizable reminders increase flexibility according to the patient's own schedule.
- Additional SMS reminders on patients own smartphones can be implemented thanks to our extended offer via partnerships.
- **Integrated features to improve the patient experience**
  - **Televisit:** allows visits to happen remotely between the patient and the site.
  - **BYOD solution:** this easy-to-use solution has a surprisingly simple set-up via QR code that is convenient for eDiary completion in long-term studies.
  - **Interview mode:** another option for completing questionnaires with the help of the site; answering a PRO via a phone interview can bring relief at certain timepoints in the study.
- **Data Management capabilities**
  - We have implemented specific reporting to track inconsistencies in rare disease studies.
  - We can identify when re training or additional support is required by specific sites.

KAYENTIS at a glance

A KEY PLAYER IN eCOA & DECENTRALIZED CLINICAL TRIALS

Broad experience...

Since 2005
eCOA expert

300+
clinical trials phase I-IV

100,000+
patients

20
different therapeutic areas

...in a wide range of study types

Up to 8,000
patients per study

Up to 1,000
sites per study

Up to 50
countries per study

Studies lasting **up to 10** years

...with strong scientific and operational support

Clinical & Scientific
dedicated team to support your study

Operational capabilities
USA - Europe - Asia

24/7
HELPDESK

Kayentis

To learn more, please contact us at sales@kayentis.com