CASE STUDY – PEDIATRIC

Tackling the complexity of pediatric clinical trials

Despite the evolution in pediatric legislation and a renewed interest in developing therapeutics for pediatric and rare diseases, pediatric clinical research remains deeply challenging.

- What makes pediatric clinical trials so difficult?
- How can we contribute to making this a smoother experience for the patients, their caregivers, and the clinical trials' industry?

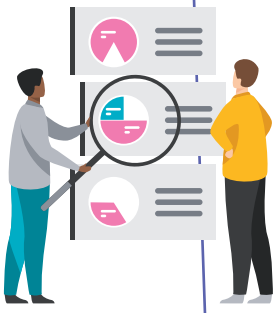
KAYENTIS EXPERIENCE IN PEDIATRIC TRIALS:

- Multiple therapeutic areas including Oncology, Hematology, Respiratory, Metabolism, Dermatology and Rare diseases
- Multiple patient age groups within the same study
- Numerous specific ePROs, eObsROs and eDiary questionnaires
  - EQ5D-3L, EQ-5D-5L Self, EQ-5D-5L PROXY version
  - PedsQL Child (Young adult, Adolescent and Pediatric versions)
  - CGI-I, CGI-S, CGIS-H, CGIC-H, CgGIS-H, CgGIC-H
  - PROMIS, BPI-SF, HQ-CT, cDLQI, UPDD/UAS, C-ACT

Unravelling the specific challenges of pediatric clinical trials

Large programs, multiple studies, time constraints and quality requirements for all projects

EACH PIECE OF DATA IS EXTREMELY VALUABLE



- The pediatric population is limited (20% of US population is younger than 14)
- A small number of patients: fortunately, children are much less likely than adults to suffer from serious illnesses, however, they are very difficult to recruit
- Clinical research on rare diseases and in pediatric population is limited, and parents may be reluctant to give consent for their children for fear of exposing them to an unproven therapy

> Kayentis is focused on developing a flexible and user-friendly solution to facilitate daily data collection, minimize the need for site visits, and reduce the impact of the study on day-to-day life

THE PATIENT POPULATION IS VERY DIVERSE

- Preferences and habits vary across age ranges, 7-8 year olds will behave and react very differently from adolescents
- When patients cross an age boundary between two assessments, it may cause confusion in attribution of questionnaires
- Social life will be impacted by both the disease and the clinical trial conflicting with school and sport activities

> Kayentis has built a team of experienced eCOA specialists, with in-depth knowledge of pediatric population, and flexible solutions to accommodate the wide range of ages and questionnaires

COMPLEX eCOA BUILD

- Several questionnaire versions are usually required to meet the needs of the protocol
- Complex branching logic will be programmed to ensure that each patient has access to the appropriate questionnaires at the right time
- Involving caregivers requires the implementation of specific accounts, appropriate questionnaires and robust audit tracking

> Kayentis has developed extensive experience in managing complex clinical trials, requiring flexibility and adherence to high data quality standards.

KAYENTIS KEY SUCCESS FACTORS FOR CONDUCTING PEDIATRIC TRIALS

Solid knowledge in the specifics of pediatric research

✓ HIGH LEVEL OF INVOLVEMENT OF THE MEDICAL AND SCIENTIFIC DEPARTMENT

- **Systematic review of protocols** to identify any areas of concern prior to the start of the project
- **Specific analysis of complex studies** to propose the best implementation from a clinical, scientific and product point of view

✓ SKILLED AND EXPERIENCED OPERATIONAL STAFF, ABLE TO:

- **Understand the challenges of the protocol**, the patient population, the various age ranges, and the need for multiple accounts
- **Anticipate the risks** if patients move from one age category to another
- **Propose a suitable eCOA build implementation**, e.g., by implementing the relevant triggers, and proposing appropriate edit checks that will ensure data quality

✓ SOLID OPERATIONAL STRUCTURE AND STABLE STAFFING

- Project managers & data managers work closely together in a spirit of mutual trust
- Low attrition rate, minimizing staff turnover during studies

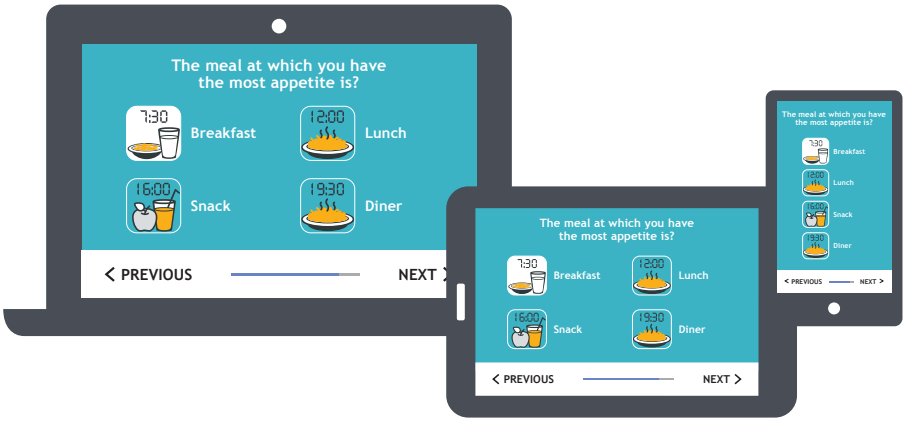
Ability to manage complexity

✓ ROBUST TECHNOLOGY TO MANAGE COMPLEX BUILDS

- **Ability to program relevant complex branching logic** to ensure data collection and integrity
- **Management of multiple-questionnaire visits and complex score calculations**
- **Management of numerous questionnaires versions and translations across broad age groups** thanks to robust technology and close relationships with LVAs
- **Management and audit trail** of multiple user accounts for ePROs, eObsROs and eClinROs

CREATIVE eDIARY DESIGN TO FIT THE SPECIFIC NEEDS OF THE PEDIATRIC POPULATION

- **Design and implementation of specific eDiaries adapted to a young patient population**, with either cartoon emojis or animated characters



"I appreciated having less writing and more illustrations, I figured it out on my own."

An 8-year-old child, after testing a specific eDiary designed by Kayentis

Flexibility of the solutions proposed

eCOA DATA COLLECTION METHODS ADAPTED TO PATIENTS' AGE AND PREFERENCES

- **Flexible data collection modes** across the provided device, Bring Your Own Device (BYOD), WebCOA, interview mode)
- **Ability to mix data collection modes** within the same study to adapt to both patients' and caregivers' preferences

POSSIBILITY TO COMPLETE ePROS AT HOME OR ON SITE

OPTION TO USE TELEVISIT WHEN PROTOCOL ALLOWS

- Patients might be too ill to go to site on certain days
- Caregivers may not be able to combine site visits with the demands of daily life

A SINGLE PLATFORM FOR MULTIPLE USES

- **eCOA and eConsent** can be accomplished using the same device
- **Other data source systems** can be integrated into the same platform

KAYENTIS at a glance

A KEY PLAYER IN eCOA & DECENTRALIZED CLINICAL TRIALS

Broad experience...

Since **2005**
eCOA expert

300+
clinical trials phase I-IV

20
different therapeutic areas

...in a wide range of study types

From **15** patients in an early phase trial to **+10,000** in a late-phase study

From local to global studies
100%
of countries covered

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