# Chilli Consultancy

Bespoke, creative solutions to your COA challenges, from concept to statistical analysis and interpretation

**www.chilli.global**

Chilli Consultancy

In an ideal world, your strategy for patient-reported outcomes (PROs) and other clinical outcome assessments (COAs) is established before trial start.

In reality, however, objectives, timelines and plans may change, or the need for COAs – or the true value of COA data – may emerge after trial start.

At Chilli, we are all too familiar with the challenges that our industry colleagues face in the modern COA environment and the evolving expectations and requirements of regulatory and HTA agencies.

We don't believe in the "one size fits all" approach; we work closely with our clients to develop – and execute – bespoke, creative solutions to their COA challenges, informed by our many years' experience in industry and consultancy.

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# COA services

We cover all aspects of COA strategy, from concept development through to statistical analysis, interpretation and reporting of results:

- Identification of relevant COAs
- Selection and validation of appropriate instruments
- Development and adaption of instruments
- Data collection within and outside of trials
- Statistical analysis plans for COA data
- Qualitative data collection and analysis
- Statistical analysis of COA psychometric and efficacy data
- Interpretation of findings and strategic planning
- Leveraging the COA story with internal colleagues
- Collection of observational data using bring your own device (BYOD) technology, in collaboration with Vitaccess
- External communication development and support.

Our project teams are experts with extensive experience in industry and consultancy.

We have a close network of consultants with whom we work regularly to provide programming, writing and editing support.

> Mark excels at handling complex healthcare data, and is able to provide creative solutions

## Mark Nixon PhD

**Co-founding partner, Biostatistics**

Leads the statistical consultancy team

Over 17 years' experience in the pharmaceutical and healthcare industry in Europe and the USA

Expert in biostatistics, with substantial experience in the validation of COAs, development of COA-specific statistical analysis plans, and analysis of COA data from clinical and observational studies

## Annabel Nixon PhD

**Co-founding partner, COAs**

Specialist in FDA, EMA and HTA requirements for COAs

20 years' international experience consulting in the pharmaceutical industry in Europe and the USA

Co-chair of the Drug Information Agency (DIA) Study Endpoints community (2012–2016)

Lead author of *Patient Reported Outcomes: an Overview* (etextbook and paperback)

## Diane Wild MSc

**Consultant, COAs**

Expert in qualitative research, instrument development, translation and linguistic validation, and validation of electronic COAs

25 years' international experience consulting for the pharmaceutical industry in Europe and the USA

Chair of the ISOQOL special interest group reviewing translation methods for COAs

## Qualitative investigation into HRQL and caregiver burden of a rare childhood genetic disease

### The challenge

Health-related quality of life (HRQL) is difficult to assess in rare diseases using standard instruments because the small number of patients weakens the conclusions that can be drawn from statistical analysis. However, HRQL data were required to provide a broad context for the safety and efficacy of a treatment in development, for HTA submissions.

### Our solution

To supplement the clinical trial data, we designed an interview-based study to explore caregiver reports of the impact of the disease on the children and themselves as caregivers, with a goal of peer-review publication.

### Our approach

We interviewed 50 caregivers of children with the rare disease. The study was conducted to the highest scientific standards, including guidance from clinical experts, development of a study protocol, ethics review, flexible study design to accommodate the sensitive nature of the interviews, highly experienced interviewers, and analysis of data using recognised qualitative analytic approaches.

### The outcome

The study report was written up as a peer-reviewed publication reporting the enormous burden of the disease on patients, their caregivers and families, to support HTA submissions.

These cases studies illustrate the scope of our PRO expertise; further case studies can be found on our website.

COA CASE STUDIES

## Development of a COA dossier to support a new clinician-reported instrument, for submission to the FDA

### The challenge

Our client had developed a new clinician-reported instrument to support the primary endpoint in a phase 3 trial. The instrument had been developed according to FDA methodology but the development process was not written up; all the evidence was contained in electronic source documents.

### Our solution

We worked closely with the team who had developed the new instrument to rapidly review and identify the evidence required for the COA dossier and to synthesize it in the format required to facilitate FDA review.

### The outcome

Our substantial experience ensured a streamlined and efficient process to develop a high-standard COA dossier supporting the use of the new clinician-reported instrument as a tool for collecting data to support the primary outcome in the trial.

# Validation of a patient-reported e-diary to support a primary trial endpoint

## The challenge

Our client planned to use a patient-completed electronic diary in a phase 3 trial of a product for a sleep disorder. However, the electronic version of the diary had not been validated according to FDA standards.

## Our solution

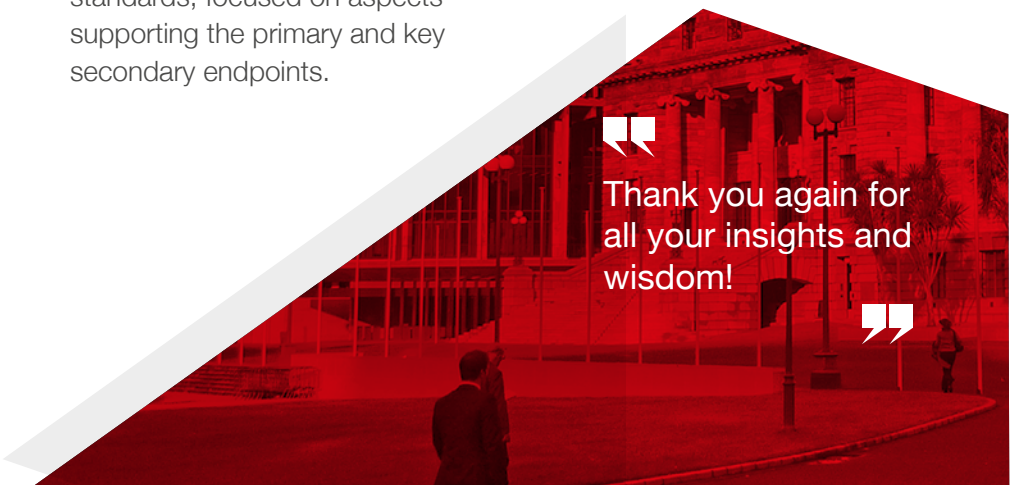
We designed a bespoke study to evaluate the content validity, usability and psychometric properties of the electronic diary according to FDA standards, focused on aspects supporting the primary and key secondary endpoints.

## Our approach

We conducted interviews with patients to evaluate the content validity and usability of the e-diary. We also designed a collaborative study to develop the study documentation, which included training of interviewers at the sleep clinic in the US conducting the trial, development of the statistical analysis plan for psychometric testing, and analysis of the data according to regulatory standards.

## The outcome

The evidence required to support validation of the e-diary was developed rapidly and to a high standard, for incorporation into the clinical trial.






vitaccess

# Partnership with Vitaccess to collect COA data using BYOD technology

Vitaccess, led by Dr Mark Larkin, has developed a BYOD (bring your own device) platform to capture real-world data, informed by substantial expertise in observational studies to support HTA and market access. Chilli is working in close partnership with Vitaccess to collect COA data in a flexible and patient-focused way using this BYOD platform.

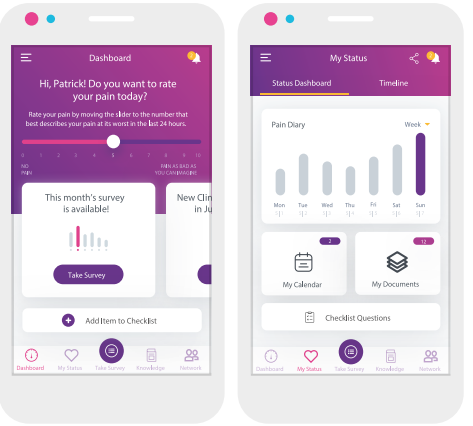
**We have harnessed digital technology to create a powerful real-world evidence platform to quantify patients' experience of illnesses and treatments.**

Dr Mark Larkin, Vitaccess

As an example of this partnership, we have developed data collection and statistical analysis strategies for a study using the BYOD to collect real-world data using the BYOD platform from patients living melanoma. The study has been developed jointly with the leading patient advocacy group Melanoma UK and sponsored by the Royal Marsden NHS Foundation Trust (London).

Annabel Nixon from Chilli led a patient focus event at the Royal Marsden that contributed to collaborative development for the melanoma project.

The observational data generated using the platform will provide real-time insight into the evolving treatment of melanoma.



In addition to COA expertise, Chilli offers bespoke statistical consultancy services – see our website for more details.

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