SIMBEC-ORION

# Rare & Orphan Study Support

## Delivering Rare and Orphan Studies for Over 25 Years

simbecorion.com

# Our goal is the same as yours– to improve patients' lives

After 25 years delivering rare and orphan studies, Simbec-Orion provides the experience and expertise your clinical development partner needs with a flexible, quality-focused approach.

Simbec-Orion are the perfect partners, understanding your project's needs and providing the infrastructure to take your research goals to the next level, while always focusing on patient care and well-being.

# What we offer

## Phase I-III services

We deliver the full range of clinical development services you would expect from a large CRO, with the structure and size to offer personalised, tailor-made solutions. Delivering a integrated and responsive approach to mitigate risk and smooth the path to your next milestone.

- Project Management
- Regulatory Affairs
- Consulting & Strategic Support
- Clinical Monitoring
- Central Laboratory
- IMP Management
- Medical & Pharmacovigilance
- Biometrics (Data Management & Statistics)
- Technical Writing
- PK/PD Studies
- Bioavailability & Bioequivalence Studies
- Bioanalytical Laboratory
- Scintigraphy
- Scientific Advisory Board (SAB)

## An international, full-service clinical research organisation specialising in rare disease studies

Head office situated in the UK, with local offices in France, the USA and Hungary.

More than 25 years rare disease experience and clinical development expertise.

A quality-focused approach ensuring data integrity and patient safety.

### Supporting clinical studies from phase I

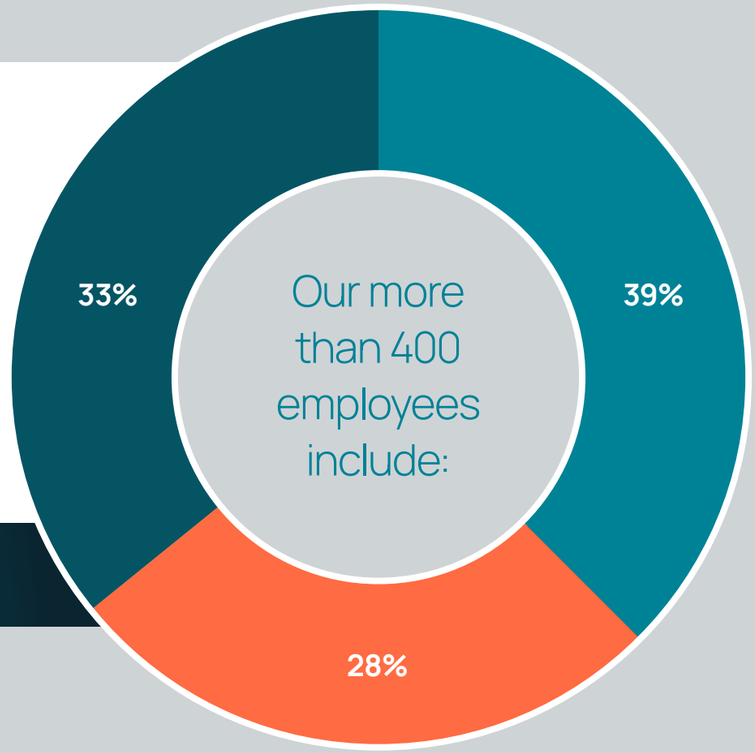
- Dedicated 48 bed phase I facility
- Pharmacovigilance group
- Regulatory group
- IMP management group
- Consulting team

### Central laboratory services and logistics

- Central laboratory services
- Bioanalysis
- Central laboratory network
- Safety testing

## Services

- Experienced CRAs, CTLs, CTAs & PMs
- Bionanalyitcal, clinical pathology & IMP capability
- Support disciplines:
  - Biometrics
  - Regulatory affairs
  - Medical & technical writing
  - Pharmacovigilance




## Scientific advisory board with expertise in rare disease studies

The Simbec-Orion Scientific Advisory Board (SAB) is established to offer our clients additional support and guidance in our core therapeutic areas of rare disease and rare oncology. The SAB consists of experts in their respective fields with extensive experience in:

- Clinical development
- Scientific consultancy
- Complex protocol design
- Orphan drug designation
- Paediatric investigational plans
- Marketing authorisation applications by both the EMA and the FDA





## Your international team

With four main offices in the UK, Europe, and the USA, as well as operational teams more widely located throughout Europe and North America, we have the global reach to deliver studies in every region and across multiple timezones.

## Critical success factors

### A patient-centric approach

Every patient is unique - working with patient associations and patient support groups ensures studies are designed with patients' needs front and centre.

### A deep understanding of rare diseases

Rare and orphan studies require a different approach – experience working with unique patient populations is critical to study success.

### Paediatric experience

75% of rare diseases affect children, so it's vital to understand the complexities of paediatric studies and how to navigate them.

### Agility and resilience

Our size and structure allows us to rapidly respond to challenges. Our lean management structure means we are able to escalate issues quickly when required.

### Global collaboration

We have extensive experience collaborating with a wide range of sites, not only across Europe and North America, but also within Asia, South America, and Australia.



## End-to-end rare disease clinical trial delivery

From conception to completion, we design, manage and assess your rare disease clinical trial programme; devised for the specific challenges of rare disease clinical research, and always with an appropriate mitigation plan.

## Patient advocacy relationships

We build strong relationships with patient advocacy groups in different countries to enable better understanding of the complex needs of these unique patients. We leverage lessons learned and best practices to successfully recruit within rare disease populations in collaboration with, and with the endorsement of, these advocacy groups and associations.

## Strategic site selection

Maintaining a strong patient focus and family-centric strategy throughout, we identify sites which are optimally suited for the patients needs. Factors that influence site selection may include considerations such as geographical location, accessibility and availability of clinical support staff. We utilise technology and data tools to provide real-time insights into recruitment performance.

## Study delivery

More challenging than trials in common indications, rare disease trials require an adaptive and focused team who can respond with flexibility to the needs of the sites and the overall study. We build a responsive study team with the necessary expertise in designing and conducting these complex studies.

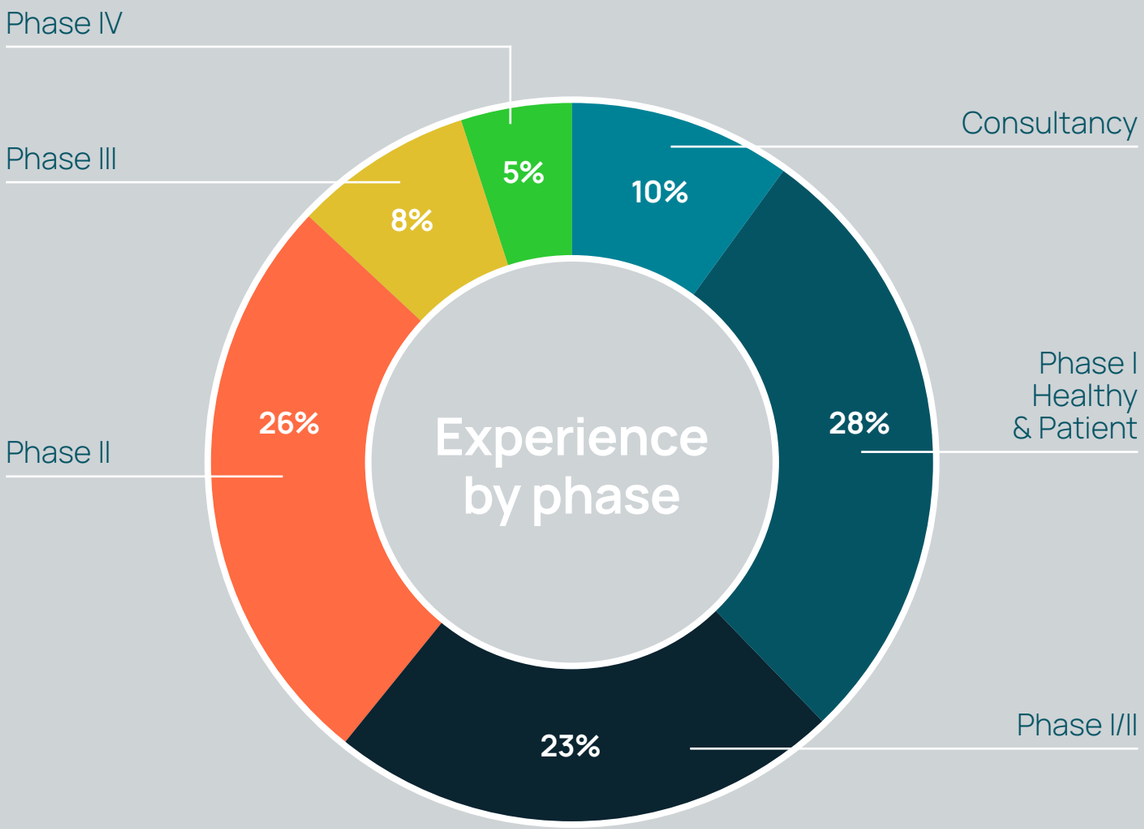
Leveraging internal resources with a proven track record in delivering rare disease studies, we strive to deliver a robust study that comprehensively meets the needs of all the key stakeholders,

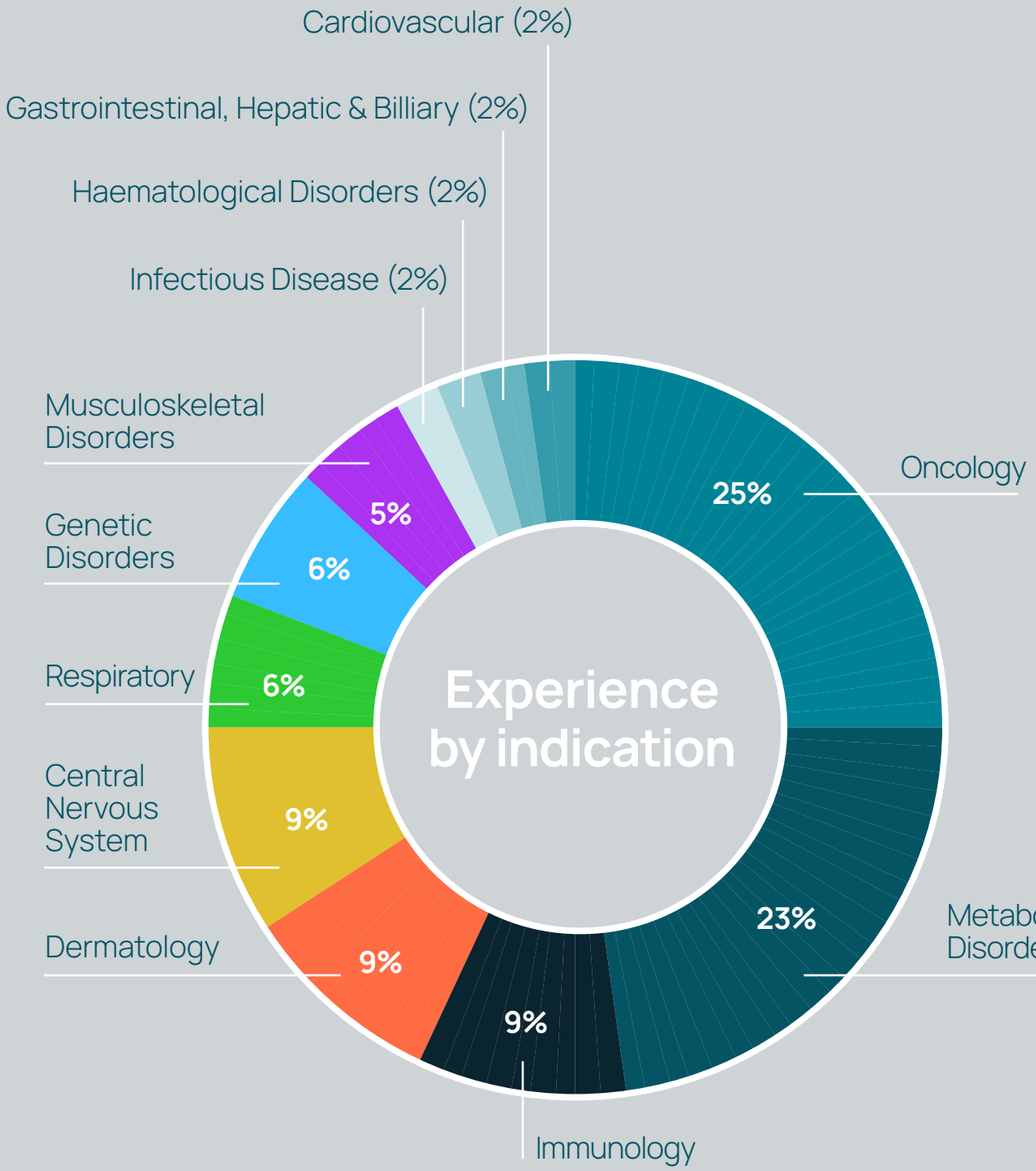
## Experienced in adult and paediatric populations in rare and ultra-rare conditions / indications such as:

Huntington disease, Niemann Pick Type C, ALS, DMD.

Our depth of knowledge built over 25 years of rare disease study experience allows us to meet the complexities and challenges faced in delivering rare disease studies.

## Simbec-Orion has built a wide range of experience across many therapeutic areas in rare and orphan drug development, with 25% of our experience in rare oncology indications



# Caring ambitiously

Caring ambitiously is one of our five core values. We make a difference to people's lives - through the work we do and the way we do it. We make a positive impact on drug development for our clients and - most importantly - their patients.

## 2022 Simbec-Orion charity of the year

Beacon (formerly known as Findacure). Beacon is a UK-based charity that is building a united rare disease community with patient groups at its heart. Beacon up-skills rare disease patient groups through training, guided programmes, community projects and research initiatives, helping them maximise their impact and deliver change for the world's often neglected rare disease patients.

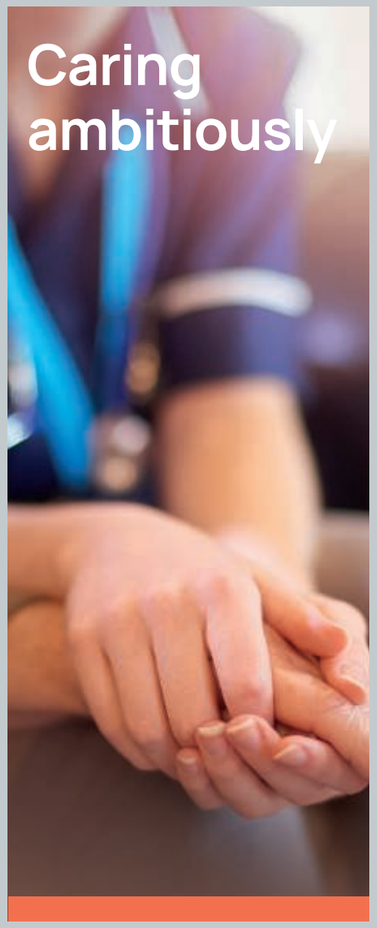
SIMBEC-ORION | beacon for rare diseases no rare journey alone

# Our values

We are defined by our five core values and are committed to making Simbec-Orion a great place to work by building on these values to shape our company culture.

We reference our Values during our hiring and on-boarding processes. In this way, we are confident that we are recruiting like-minded individuals with values that align with ours.

By attracting the right talent to join a team already proud to work for Simbec-Orion, we strive to continue to make a difference. Both to the way we work, and to the lives of the patients waiting for new and improved therapies around the world.

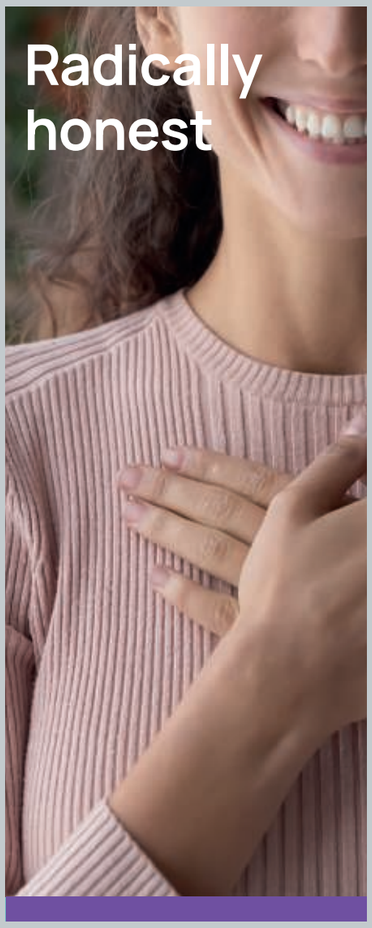



We make a difference to people's lives - through the work we do and the way we do it. We make a positive impact on drug development for our clients and - most importantly - their patients.


We become an extension of your team, dedicated to your clinical development - whatever the challenges.


No matter what the problem. Or how big the challenge. Our dedication to delivery for you and ultimately patients, drives us every time without exception.


We're an agile team, willing and able to adapt to continuously changing circumstances.




Ethics and compliance are at the heart of what we do. So integrity is everything. We work with transparency, because that's the honest thing to do.

# Niemann-Pick type C

## Surpassing timelines & rapid enrolment in an ultra-rare paediatric study

The sponsor needed to produce a new batch of their product with concentrations adapted based on the weight of paediatric patients. An observational study was needed to identify and profile patients before progressing to an interventional study.

Patients enrolled were between 2 to 18 years of age with a diagnosis of NP-C. NP-C is an ultra-rare indication in paediatric patients, requiring an agile CRO who could work with the sponsor as the study evolved.

## Study objective

To characterise the individual patient disease progression profile (disease burden and progression) through the clinical, imaging, biological status, and quality of life prospectively recorded, together with the historic disease information collected from patient medical records.

### Secondary objective

To evaluate the safety data of the disease-related therapy and to record every adverse event (AE) linked to the disease.

## Challenges

Niemann–Pick type C (NP-C) is defined as an ultra-rare disease affecting an estimated 1:150,000 people, with around 500 patients identified globally.

To identify centres which would excel in a patient-centric study.

Critical centres had diagnosed and treated patients with NP-C, and understood the unique medical needs of their patients.

Study must consider patient's individual requirements while adhering to additional paediatric regulations.

## Solution

A consultative, tailor-made approach to study set up and design.

## Direct interaction with each of the clinical authorities & ethics committees

Our regulatory team prepared the necessary documentation and directly interacted with each of the clinical authorities and ethics committees to promptly address any potential concerns.

## Consultative approach to protocol writing and study set-up

We took an active role in the writing of the protocol, and were responsible for designing the eCRF, collaborating with the sponsor to prepare the innovative statistical plan.

## Accelerated timelines by utilising our central laboratory

To accelerate timelines, we were able to organise the sample preparation under special conditions, precisely coordinating the time-critical delivery to our central laboratory.

## Solution

A patient-centric study with capable, experienced sites.

## Evaluated centres of excellence

Deployed CRAs to evaluate centres of excellence with capabilities to perform this complex clinical trial in more than 18 European countries, resulting in 16 sites across 11 countries.

## Study documents adapted to patient's needs

Designed the PICF (Patient Informed Consent Form) in all the required languages, adapting them to be suitable for the different paediatric age groups concerned.

## Study conducted with a patient-centric approach

Mobilised the patients and families to be included in the study and provided the appropriate legal support to adapt the sites contracts upon the individual requirements.

## Outcome

The observational study took just 10 months to complete and was swiftly integrated into the interventional study once the IMP was ready and the drug supply organised.

The sponsor was able expand the Phase II/III interventional study in the US and Europe, with enrolment for the interventional studycompleted in record time.

More than 25 years delivering rare and orphan studies from phase I-IV

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# We are here to help with the next step of your clinical development

Simbec-Orion is a responsive and agile full-service CRO, with wide therapeutic experience and specialist expertise in clinical pharmacology, rare disease and rare oncology. Perfectly structured, we provide full-service clinical development solutions for small and mid-size drug developers – headed up by a centralised leadership team.

With a focus on tailormade and scalable solutions, we'll adapt our delivery style, communications and operations to suit the demands of your project, helping you achieve your clinical and commercial objectives. Because our goal is the same as yours; to improve patients' lives.

Find out more at www.simbecorion.com or contact us at information@simbecorion.com

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