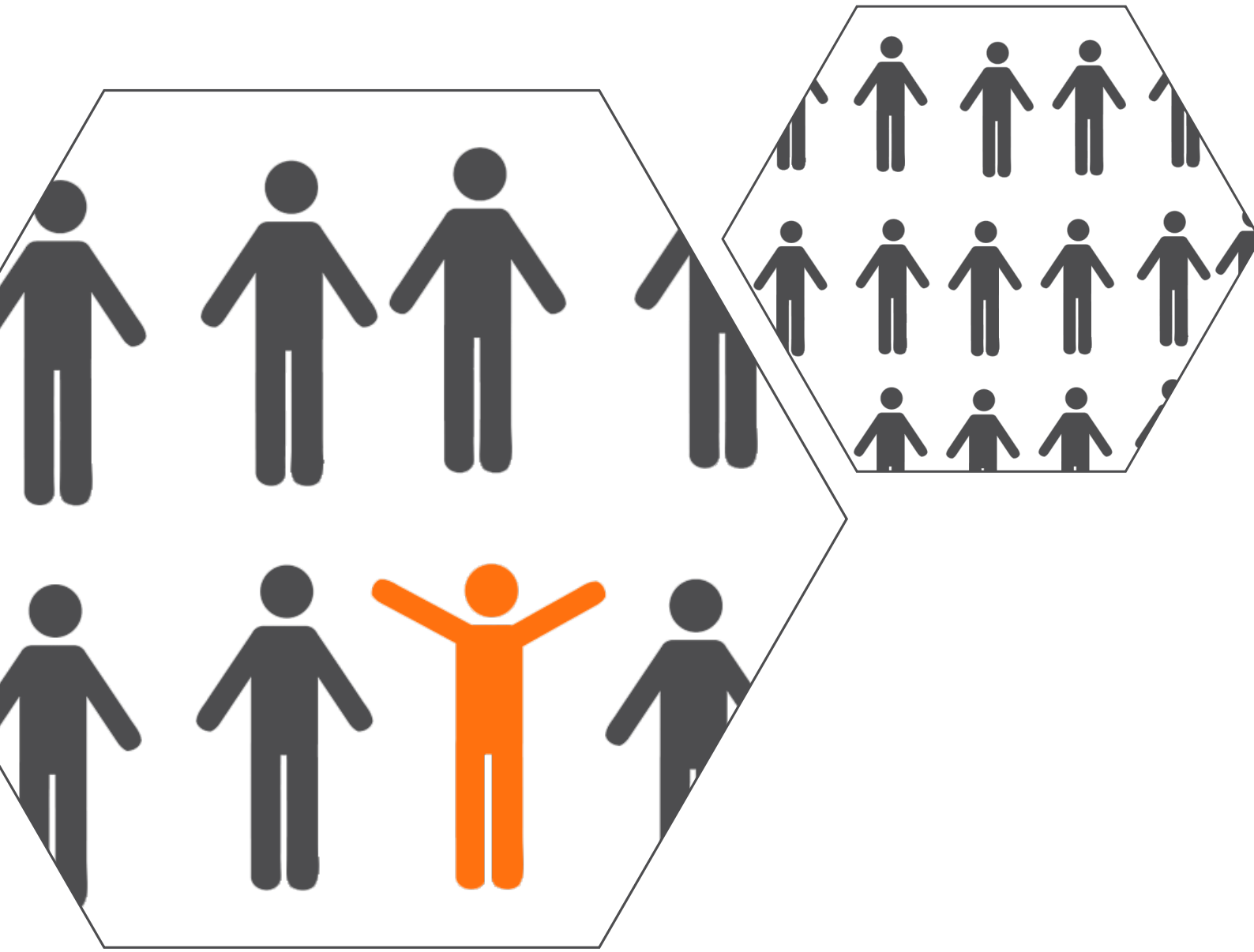
LIFE SCIENCES

ORPHAN DISEASES

ADVANCED CLINICAL RESEARCH SOLUTIONS



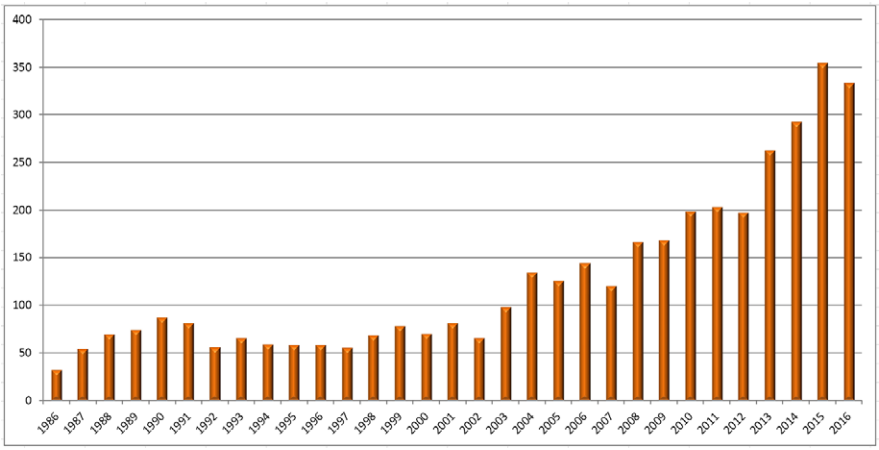
SGS

CHALLENGES & OPPORTUNITIES OF RARE DISEASE DRUG DEVELOPMENT

The growing number of rare diseases lacking treatment is an important public health issue. Orphan medicines legislation provides incentives to pharmaceutical companies to develop and market medicinal products to treat rare diseases. These acts, in both the United States and Europe have raised awareness for the many rare diseases, which has led to an increase in orphan drug designations year by year since the implementation of such legislation.

ORPHAN DRUG DESIGNATIONS BY FDA PER YEAR

FDA Orphan Drug Designations and Approvals Database
https://www.accessdata.fda.gov/scripts/opdlisting/oopd/index.cfm



Clinical trials involving orphan drugs are challenging for a number of reasons including a lack of validated endpoints, difficulties with locating patients and logistical problems in clinical trial organization. The latter two reasons can contribute significantly to the costs of clinical trials. However, clinical trials involving orphan diseases require fewer patients and are often shorter than those involving more common diseases, resulting in shorter time to market.

EXPERIENCE & SOLUTIONS IN ORPHAN DRUG DEVELOPMENT

The Clinical Research division at SGS has 35 years of experience as a contract service organization providing clinical trial services for the pharmaceutical, biotechnology and medical device industry across Europe and North America. During the last five years, SGS has conducted 14 studies in rare or ultra-rare disease indications.

14 STUDIES

11 INDICATIONS

ORPHAN INDICATIONS	
Acute Spinal Cord Injury (ASCI)	Cystic Fibrosis
Lesch-Nyhan Disease	Multidrug resistant tuberculosis
Myasthenia Gravis	Narcolepsy
Necrotizing enterocolitis (NEC) in neonates	Pseudomonas aeruginosa Lung Infection
Severe Von Willebrand Disease	Urea Cycle Disorders (UCD)
ADA SCID	

More than 900 patients were recruited in these 14 trials. SGS provided a wide range of services for these studies, from stand-alone to full service, including:

SERVICE OFFERINGS FOR RARE DISEASES	
Project Management	EDC design
Clinical Monitoring	Data Management
Medical monitoring	Biostatistics
Project feasibility assessment	Bioanalysis
Site identification and selection	Pharmacovigilance
Protocol design and writing	PD/PD analysis and modeling
Regulatory	Medical writing

900 PATIENTS

FULL SERVICE CRO

CASE STUDY

PHASE 2 / 3 ORPHAN DRUG TRIAL

OBJECTIVES

SGS was contracted to conduct a phase 2 / 3, randomized, double blind, placebo-controlled, parallel group, dose response trial in subjects with lamellar ichthyosis. In total, approximately 65 patients were randomized in a 3:3:1 ratio in 1 of 2 dose groups or placebo respectively. The trial was run in 19 sites over 9 countries (Belgium, Canada, Dominican Republic, France, Germany, Italy, The Netherlands, Norway and Sweden).

KEY CHALLENGES AND SOLUTIONS

- The recruitment of the patient population was the major challenge in this trial. During the trial realistic recruitment predictions were created based on site contacts and recruitment was closely managed. To enable this, close interactions between the sites and the clinical research associates (CRAs) were deemed necessary. Several newsletters were put in place to increase the involvement of the investigators in the trial and to motivate them to keep recruiting patients. Furthermore, to enhance recruitment, SGS added additional sites to the trial

- A management and communication plan was put in place clearly defining roles and responsibilities of the different stakeholders (SGS, vendors and Sponsor). Frequent trial status reports, timely and clear communication on additional tasks, costs and a detailed risk management ensured the success of the study

OUTCOME

Firm project management with a good understanding of the needs for the population has proven to be efficient in the management of a trial with an orphan drug. Since the population is more difficult to recruit, a close relationship with the sites was deemed important and the use of CRAs speaking the local language was proved to be the best solution to bridge cultural and linguistic differences.

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