SCOPE

FACT SHEET

YOUR CRO OF CHOICE FOR

ATMP DEVELOPMENT

Advanced therapy medicinal products (ATMP) are medicinal products involving cells, gene therapy or tissue engineering. They are used in many different indications, such as immune diseases, Parkinson's and Alzheimer's disease, cartilage defects, cardiac repair, skin replacement and cancer immunotherapy. ATMP differ from other medicines. As they are based on chemical entities or are of biological / biotechnology origin, they require special legislation when it comes to market authorization. Therefore, this product class covering different new therapeutic strategies requires special knowledge and experience.

PREVIOUS **TRIAL EXAMPLE**

Outline of a clinical trail covering the testing of ATMP in a joint: The goal of this phase III clinical study was to demonstrate the efficacy of an ATMP in the treatment of cartilage defects in a joint. The efficacy is evaluated by patients' subjective assessments and physicians' evaluations of the functional efficacy of the product. The cartilage regenerative effects as well as the safety profile are additionally assessed.

With our structured and supportive approach, our excellent communication skills with investigational sites aided by our acquired experience in trials with ATMP, SCOPE was able to maintain the initial proposed timelines despite many unforeseen challenges.

III

PHASE III

STUDY **DETAIL**

- **Phase III clinical study**
- **Orthopedic indication**
- **100 subjects to be enrolled within 15 months, 20 sites in 5 different countries in Europe**

100

SUBJECTS

Services provided by SCOPE: In addition to general project management and clinical monitoring activities, we provide the following operations in the trial: budget planning and budget management, regulatory affairs, data management, EDC programming, safety management, pharmacovigilance, medical monitoring activities, statistical programming and analysis.

5

COUNTRIES

Challenges: Due to regulatory timelines, only 10 sites were able to recruit during the first six months. Additionally, one country was unable to participate due to regulatory changes during the approval process. SCOPE had to replace seven sites during the recruitment phase of the trial. SCOPE's success in replacing these sites was due in large part to the strong cooperation with sites of our investigator-network in already active countries. Additionally, a new country

with three sites was added. The basis for this decision was founded in strategic knowledge of regulatory requirements for the country and the submission process. Another hurdle successfully handled was the unforeseen protocol update after the initial submission /approval. The initial recruitment goal was successfully met first half of the trial recruitment period. This was obtained despite regulatory delays that resulted in only 50% of the sites being active for the first half of the trial.

RESULT

Despite the many hurdles and difficulties during the study planning and submission, all timelines were kept and all subjects were successfully recruited within the projected timelines. The challenge of the long-term follow-up and close cooperation with the sites is part of our current service.

AT A **GLANCE**

SCOPE has experience in conducting clinical studies dealing with ATMPs and can therefore provide the specific knowledge necessary for navigating such a study.

GENERAL CHALLENGES FOR STUDIES DEALING WITH ATMPS	OUR EXPERIENCES / RECOMMENDATIONS
Complex regulatory interactions such as: regulatory challenges at site level and submission requirements multiple and complex regulatory framework for ATMP development.	SCOPE knows the ATMP-related requirements and has deep knowledge of special regulatory requirements in several countries.
Country and site selection	SCOPE has profound experience with special criteria for site evaluation and selection. The countries are selected with respect to the special country requirements.
Competent authority and ethics committee timelines	Prolonged evaluation / approval timelines from competent authorities and ethics committees for ATMP trials must be taken into consideration. Experience in country selection based on individual evaluation / approval timelines.
Tissue release	SCOPE has an excellent cooperation with external partners for tissue release in several countries and very good relationships with investigators across Europe.
Traceability: One of the organizational challenges is traceability. This describes the ability to locate and identify each cell / tissue unit during the entire production of the individual ATMP from application to recipient.	We are familiar with the traceability procedures and the documentation process. We have appropriate networks, tracking systems and knowledge to ensure regulatory traceability requirements.
Safety challenges: EMA guideline long-term data paramount – particular focus on late AERs, tumorigenicity	SCOPE provides up-to-date safety management associated with product and trial procedures.

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FOR FURTHER INFORMATION

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