

Sciensus and Disc Medicine demonstrate scalable multinational rare disease survey framework at ISPOR 2026

LONDON, UNITED KINGDOM, May 19, 2026 /EINPresswire.com/ -- Sciensus and Disc Medicine announced data, presented at ISPOR 2026 (May 17–20, Philadelphia), showing that a centralised yet locally adaptable methodological framework can efficiently deliver high-quality, patient-centred insights across multiple European countries in a rare disease setting.



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Dr Sherif Raouf, Clinical Director, Cancer, Sciensus

The study, selected for the inaugural Rare Disease Poster Tour at ISPOR (Poster PT17), tested the framework in erythropoietic protoporphyria and X-linked protoporphyria (EPP/XLP), rare genetic diseases of the heme synthesis pathway often associated with severe phototoxic pain, impaired health-related quality of life (HRQoL), and increased risk of liver disease. The approach was designed to overcome common challenges in rare disease research, including small and geographically dispersed populations, inconsistent local research requirements and the high cost of running separate country-level studies.

Working together, Disc Medicine and Sciensus developed and validated a cross-sectional survey framework deployed near-simultaneously across five European countries – the UK, France, Germany, Italy and Spain – using a single central infrastructure with defined local adaptations. The framework successfully recruited 101 participants (90 adults and 11 adolescents; mean age 43 years). All participants completed the questionnaire, yielding a 100% completion rate in under eight months from project start to study close.

Dr Sherif Raouf, Clinical Director, Cancer at Sciensus, commented: "This framework demonstrates how a single, centralised infrastructure can unlock the kind of patient insights that traditional siloed studies struggle to generate in rare diseases. Our teams were able to deliver a five-country study in under eight months, with 100% completion and high engagement."

The framework blended validated patient-reported outcome measures with novel items on symptoms, HRQoL, health care utilisation and treatment preferences, focusing on the patient perspective from study design through to analysis. Operationally, a master protocol was adapted

into national versions, mapped ethics and regulatory requirements per country, and implemented multilingual electronic data capture with embedded validation rules and proactive data quality checks. The methodology is designed to be generalisable across rare conditions, providing life sciences partners with a scalable model that supports health technology assessment, reimbursement, market access and patient-centred decision-making, particularly in settings where traditional evidence is fragmented or limited.

The poster (PT17), "An Effective Methodological Framework for Executing Multinational, Patient-Centred Cross-Sectional Surveys in Rare Diseases," was presented this week during the Rare Disease Poster Tour and Poster Session.

About Disc Medicine

Disc Medicine (NASDAQ: IRON) is a biopharmaceutical company committed to discovering, developing and commercializing novel treatments for patients who suffer from serious hematologic diseases. The company is building a portfolio of innovative, potentially first-in-class therapeutic candidates that aim to address a wide spectrum of hematologic diseases by targeting fundamental biological pathways of red blood cell biology, specifically heme biosynthesis and iron homeostasis.

About Sciensus

Sciensus is a life sciences organisation specialising in patient access, clinical services and insight solutions. The company supports patients with complex and chronic conditions through homecare services, digital tools and real-world data capabilities, partnering with healthcare providers and pharmaceutical companies across the UK and Europe. For more information, visit www.sciensus.com.

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