Case Study

Achieving Success in the Largest-Ever Study of a Hemophilia Gene Therapy

Managing a 9-study, 21-year program with over 1,240 patients enrolled

Sponsor Challenges

The first of its kind to offer gene therapy options to hemophilia patients, this extensive nine-study program required stellar teamwork to mitigate challenges from the outset, including high competition for patients, an intensive patient visit schedule, and complex dosing requirements and lab management. Maintaining consistency in trial oversight over the course of two decades to ensure the highest level of management and governance was crucial for the success of such a trial.

At a glance

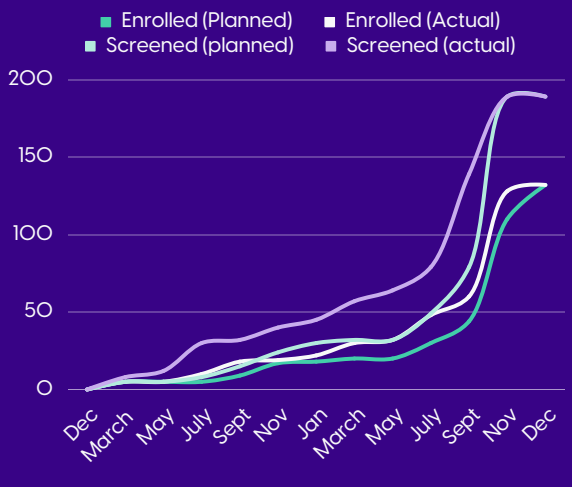
PSI supported the sponsor of a program of studies that allowed hemophilia patients access to a gene therapy product for the first time, including the first gene therapy product to be administered in Brazil. By working closely with sites, building trust, and mitigating staff turnover, PSI was able to achieve FDA and EMA approval for nine studies over the course of a 20+ year program, including the largest-ever study of a hemophilia gene therapy.

Key Metrics

1,240
Enrolled Patients

180
Trial Sites

15+
Countries



PSI Strategy

1 Strong Site Relationships

PSI has been building hemophilia site relationships for over 20 years. PSI knows the investigators and invests time and resources to build close site relationships, so sites want to work with us and prioritize our studies.

2 Building Trust

From the outset, we worked closely with sites, where possible selecting sites that delivered for us on other studies. Additionally, the team implemented meticulous visit planning and site support, using both the Investigator Meeting and co-monitoring visits to build motivation for investigators and help the client gain valuable insights during the program.

3 Low Staff Turnover

PSI enjoys a low turnover rate – just 15% for the key staff on this portfolio. As we retained our staff, there was significant overlap across the portfolio teams, keeping valuable knowledge of the IMP and maintaining site relationships to deliver recruitment that exceeded the global average.

Program Successes and Outcomes

- 7 successful FDA Inspections
- EMA and FDA approvals granted
- Same Project Manager/Project Director for 5 years
- Creation of client-specific data and budget tracking system
- 10+ long-term follow-up studies awarded to PSI based on performance