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CASE STUDY

Reducing Patient Burden While Maintaining High-Frequency Monitoring in a Hemophilia Gene Therapy Trial

> "ProPharma's DCT model allowed us to meet the intensity of early gene therapy monitoring without asking patients to put their lives on hold."

Challenge

A mid-size, US-based pharmaceutical sponsor initiated a hemophilia gene therapy trial requiring intensive early-phase monitoring. The protocol included:

- Extremely frequent safety follow-ups in the early post-dose period **(24 visits within the first 12 weeks)**
- Complex laboratory testing, including hematology, chemistry, liver function, inhibitor testing, and vector shedding
- Long-term follow-up extending up to five years post-treatment

Investigator sites raised early concerns that travel requirements and the frequency of visits would negatively impact recruitment and retention, particularly for patients balancing employment, family responsibilities, and the physical realities of living with hemophilia

Solution

ProPharma implemented a decentralized clinical trial (DCT) model designed to maintain rigorous clinical oversight while reducing patient burden. Key components included:

- Home-based safety follow-up visits during the highest-frequency post-dose period
- At-home blood and laboratory collection with on-site processing and direct shipment to central laboratories
- In-home administration of intravenous corticosteroids, when clinically indicated
- Dedicated, protocol-trained nurses assigned to each participant to ensure continuity and trust
- Electronic source documentation enabling near real-time data flow back to sites
- Decentralized clinicians operating as extensions of the investigator site under delegated authority and defined communication pathways

Results

- **Improved recruitment:** Sites reported increased patient willingness to enroll once home visit options were offered
- **Expanded access:** Reduced geographic and travel barriers broadened the eligible participant pool
- **High protocol adherence:** High-frequency early safety visits were consistently completed within protocol windows
- **Strong retention:** Long-term participation remained high, even after patients experienced clinical improvement

By removing unnecessary travel while preserving clinical oversight, the hybrid model supported both intensive early monitoring and sustained multi-year follow-up

Contact us to learn how our experienced team can help ensure successful outcomes throughout the product lifecycle.

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