

REGULATORY DEVELOPMENT

MEDIPOST Secures FDA Agreement on a Single Pivotal Phase 3 Study for CARTISTEM®

A Biologics License Application strategy based on one pivotal trial, supported by confirmatory evidence from earlier trials and real-world data.

Prepared by St. Luke Hospital · Based on the manufacturer's announcement published via PR Newswire, 3 June 2026.

Focus: What the FDA agreement covers — and what it does not.

Key points

- **Single pivotal study pathway:** The U.S. Food and Drug Administration agreed that a single Phase 3 trial, rather than two separate Phase 3 studies, may support a future Biologics License Application (BLA) for CARTISTEM®.
 - **Confirmatory evidence accepted:** The agreed strategy draws on clinical data from South Korea and Japan, together with long-term real-world evidence from approximately 550 patients treated at least three years earlier.
 - **Development efficiency:** The manufacturer states that this pathway is expected to shorten the North American development timeline while maintaining the safety and efficacy standards required for BLA filing.
- **What this does not mean:** An agreement on study design concerns how a trial is conducted. It is not a decision on approval. No regulatory outcome can be predicted from it.

Original source

Manufacturer announcement (PR Newswire, 3 June 2026)

Regulatory notice. CARTISTEM® is approved in the Republic of Korea (MFDS, January 2012). It is **not approved for commercial use in the United States or in Japan**, where it remains under clinical investigation. Progress in clinical development does not indicate that regulatory approval will be granted. **Medical information notice.** This summary is provided for general information and does not replace an in-person medical consultation, diagnosis, or treatment recommendation. Treatment at St. Luke Hospital is provided within the Korean approved indication, following an individual orthopedic assessment. Outcomes vary between patients.