# Landmark Phase 3 Multicenter, Open-Label Repeat-Dose Study[1]

## Patient Population

**High-Grade**
**BCG-unresponsive NMIBC***
**N=157**

**Cohorts:**

- CIS±Ta/T1
- High-Grade Ta/T1

## Key Criteria

**Inclusion**
High-grade BCG-unresponsive NMIBC patients ≥18 years:

- CIS±Ta/T1 (CIS with or without high-grade Ta/T1)
- High-Grade Ta/T1 (without concomitant CIS)

**Exclusion**

- Current or previous evidence of muscle invasive (muscularis propria) or metastatic disease
- Intravesical therapy within 8 weeks prior to beginning study treatment

## Treatment

**Nadofaragene Firadenovec**

75 mL ($3x10^{11}$ viral particles/mL) intravesical administration, 1-hour dwell time, once every 3 months

## Endpoints

**Primary:**
Complete Response (CR) in patients with CIS±Ta/T1 at any time after the first instillation

**Key Secondary:**

- Durability of CR in patients with CIS±Ta/T1 who achieved a CR
- High-grade Recurrence Free Survival (HGRFS) rate in patients with high-grade Ta/T1
- Durability of HGRFS in patients with high-grade Ta/T1
- Time to cystectomy
- Overall survival

## FDA BCG Unresponsive Definition[2]

- Persistent or recurrent CIS with or without recurrent Ta/T1 (noninvasive papillary disease/tumor invades the subepithelial connective tissue) disease within 12 months of completion of adequate BCG therapy
- Recurrent high-grade Ta/T1 disease within 6 months of completion of adequate BCG therapy
- T1 high-grade disease at the first evaluation following an induction BCG course

***FDA BCG unresponsive Definition:** https://www.fda.gov/media/101468/download

**Biologics License Application (BLA) is currently under review by the FDA.**
**Currently not commercially available.**

For more information please contact:
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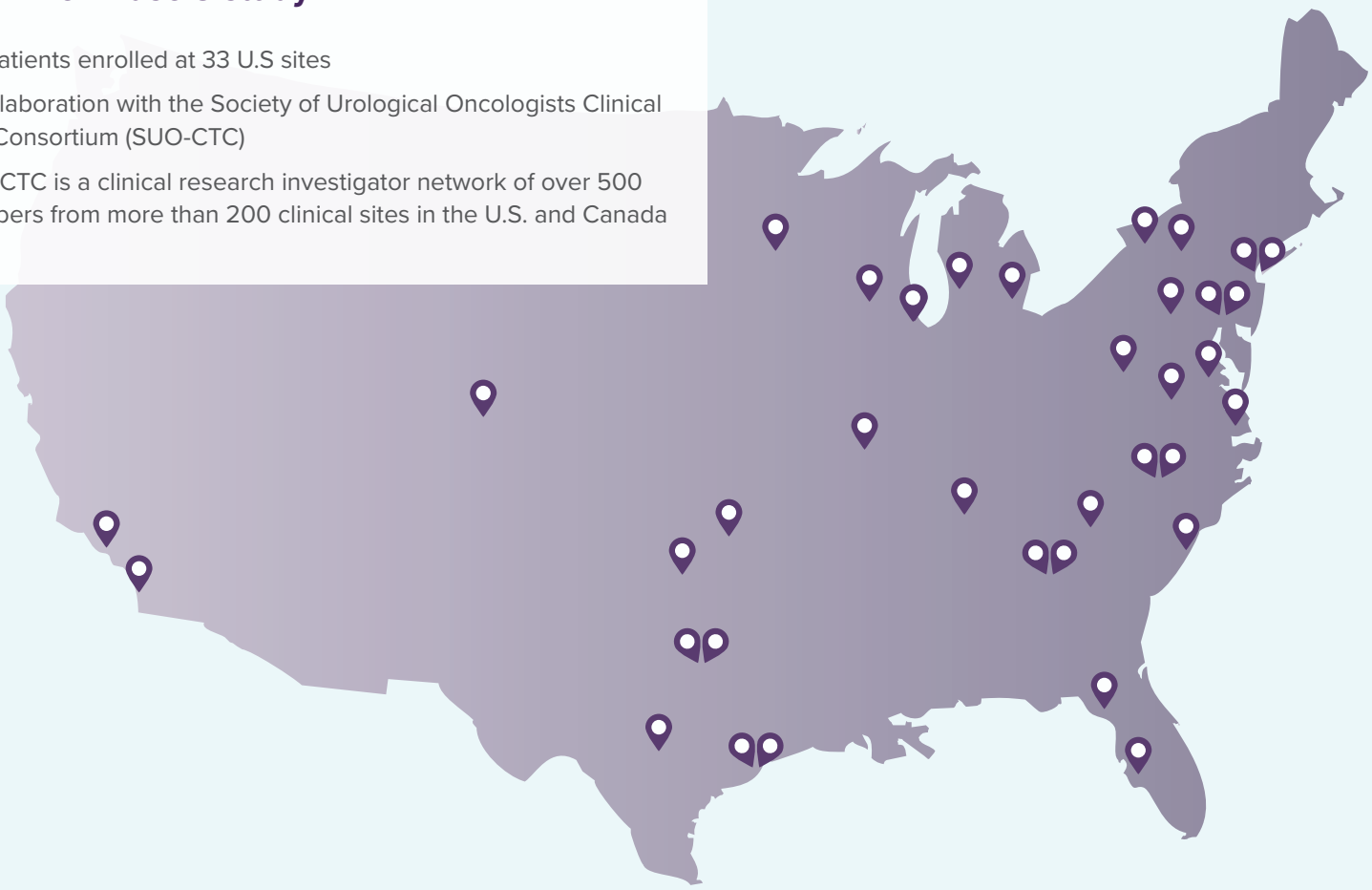
# Landmark Phase 3 Study Design: Multicenter, Open-Label, Repeat-Dose Registrational Study

## The Phase 3 study

- 157 patients enrolled at 33 U.S sites
- In collaboration with the Society of Urological Oncologists Clinical Trial Consortium (SUO-CTC)
- SUO-CTC is a clinical research investigator network of over 500 members from more than 200 clinical sites in the U.S. and Canada



**Not approved, the Biologics License Application (BLA) is currently under review with the FDA**

Link to clinicaltrials.gov to study design and participating sites:

**https://fergene.com/clinicaltrial**

**1.** Boorjian, S., Alemozaffar, M., Konety, B., Shore, N., Gomella, L., Kamat, A. et al. Intravesical nadofaragene firadenovec gene therapy for BCG-unresponsive non-muscle-invasive bladder cancer: a single-arm, open-label, repeat-dose clinical trial. Lancet Oncol. 2020;2045(20)30540. doi:101016/ S1470.**2.** FDA BCG unresponsive Definition: https://www.fda.gov/media/101468/download Accessed Accessed February 16, 2021



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