# RIDGE™-1 Phase 1b clinical trial for *PKP2*-associated ARVC

## Patient dosing began in November 2024

### Study Objectives

- Safety, tolerability, dose-finding and pharmacodynamics

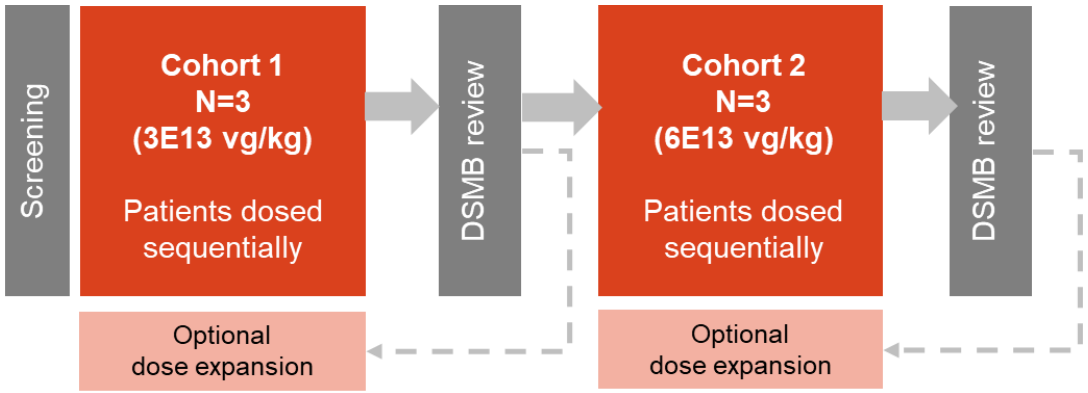
### Eligibility

- ARVC diagnosis
- *PKP2* mutation carriers
- Adults (age 18-65)
- ICD present
- Mean PVCs >500 per 24 hours
- LVEF > 50%
- NYHA class I-III
- Low AAV9 titer

### Design

Open-label, multi-center dose-escalation and dose-expansion study

- Preventive immunosuppressive regimen + close safety monitoring
- Regular assessments for safety, PK and PD
- 5-year safety and efficacy follow-up




### Endpoints

- **Safety and tolerability**
- **Pharmacokinetics (PK)**
  - Transgene and mRNA via cardiac biopsies (baseline, 8wk & 52wk)
- **Pharmacodynamics (PD)**
  - Changes in PVC & NSVT
- **Exploratory efficacy endpoints**
  - Frequency of ICD shocks
  - Frequency of VTs
  - Imaging biomarkers (structural/hemodynamic changes by echo)
  - Plasma biomarkers
  - Patient reported outcomes

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