Renal Autologous Cell Therapy in CKD with Diabetes: Pooled Safety and Efficacy Results from Phase 2 studies in Participants With Baseline Characteristics Aligned to the Phase 3 Study Population

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INTRODUCTION | BACKGROUND

- Although there have been considerable advances in treating chronic kidney disease (CKD), there is **no cure.**
- The current standard of care aims to manage and, where possible, delay disease progression to end stage kidney disease (ESKD).
- The expected annual eGFR decline in a similar population based on a meta-analyses of CKD patients with diabetes was -3.4 (95% CI: -3.90 to -2.90)mL/min/1.73 m^2/year.[1]
- **Cellular therapies** have emerged as an investigational option because of the potential to address multiple maladaptive processes simultaneously.
- **Rilparencel** is an injectable product delivered into the renal cortex formulated from the patient's own (autologous) kidney cells, being developed as a potential therapy to preserve kidney function in patients with type 2 diabetes mellitus (T2DM) and CKD.

Figure 1: Rilparencel autologous kidney cell process in the ongoing Phase 3 study

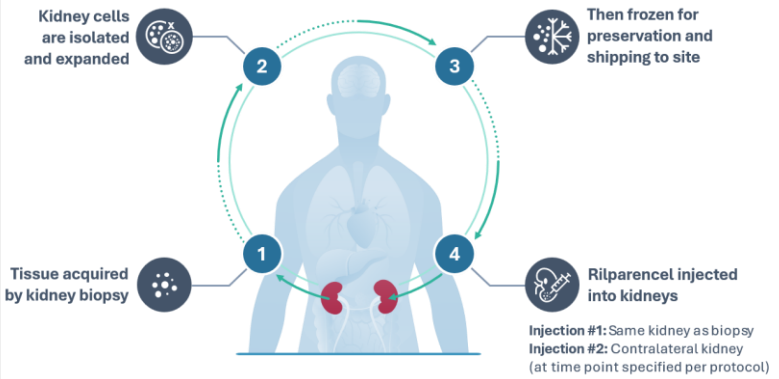


Table 1: Overview of rilparencel Phase 2 development program

Study ID	Design/Subject exposure	Study Population
RMCL-002 (complete; years active: 2016-2023)	**Part 1:** Randomized, double-arm, open-label, repeat-dose: Two doses of **fresh rilparencel** 3x10^6 cells/gKW^{est} by injection, 6 months apart (Cohort 2 dosed 12 months after biopsy) / N= 73 (39 + 24) **Part 2:** Extension, 36-month safety follow-up / N=21	US & Cayman Islands Age 30–80 years; T2DM, CKD; eGFR 20–50 mL/min/1.73m^2
REGEN-003 (complete; years active: 2018-2022)	Open-label, single-arm. Two doses of **fresh rilparencel** 3x10^6 cells/gKW^{est} by injection, 6 months apart / N=10	US - Age 30–65 years; T2DM, CKD; eGFR 14–20 mL/min/1.73m^2 (DKD)
REGEN-007 (complete; years active: 2021-2025)[2]	Randomized, double-arm, open-label, re-dose **Cohort 1:** Two doses of **cryopreserved rilparencel** 3x10^6 cells/gKW^{est} into contralateral kidneys, 3 months apart / N=24; **Cohort 2:** One dose **cryopreserved rilparencel** 3x10^6 cells/ gKW^{est} into the biopsied kidney; if a trigger is met, a second dose 3x10^6 cells/gKW^{est} into the contralateral kidney / N=25	US - Age 30–80 years; T1DM or T2DM, CKD; eGFR 20–50 mL/min/1.73m^2
REGEN-008 S1 (open)	Five-year observational follow-up, up to 30 patients planned	US - Prior RMCL-002 or REGEN-003
REGEN-008 S2 (open)	Five-year observational follow-up/N= up to 30 patients planned	US - Prior REGEN-007

CKD, chronic kidney disease; DKD, diabetic kidney disease; eGFR, estimate glomerular filtration rate; gKW^{est}, estimated kidney weight in grams; T2DM, type 2 diabetes mellitus; T1DM, type 1 diabetes mellitus; US, United States

METHODS

- This analysis used estimated glomerular filtration rate (eGFR) and urine albumin creatinine ratio (UACR) criteria from the ongoing Phase 3 study (**REGEN-006, Figure 3**) to select 61 Phase 2 participants with T2DM for an indicative *post-hoc* analysis:
  - eGFR≤ 29 mL/min/1.73 m^2 and UACR ≤ 5000 mg/g, OR
  - 30 ≤ eGFR ≤ 35 mL/min/1.73 m^2 and 300 ≤ UACR ≤ 5000 mg/g
- 32 patients were included from RMCL-002, 7 from REGEN-003, 15 from REGEN-007 Arm 1 and 7 from Arm 2.
- A linear mixed effects model was used to predict change in eGFR from baseline with random effects (intercept and slope) for time for each subject, a fixed effect of years since baseline, and no additional covariates. Annualized slope was calculated by dividing the number of days from baseline to last measurement by 365.25. eGFR measurements were calculated using the CKD-EPI 2009 equation
- Due to differing study designs, historical eGFR data was available for 40 of the 61 participants (66%) prior to study entry. The linear mixed effects model was repeated with study period added as a covariate to calculate pre/post treatment annualized slope.

RESULTS

- **61 biopsied participants** received a total of **111 injections** (≤2 per participant).
- 51 of the 61 participants in the pooled subgroup had eGFR assessments 12 months following the first injection, and the mean (SD) change from baseline was **−0.29 (5.8) mL/min/1.73 m^2**.
- 42 of the 61 participants had eGFR assessments at 18 months and 25 at 24 months, with mean (SD) changes from baseline of **−1.76 (5.6) and −2.43 (6.5) mL/min/1.73 m^2**, respectively.
- The pooled annualized eGFR slope following first injection was **−1.78 mL/min/1.73 m^2/year**
- For the 40 participants with pre-treatment eGFR values (median duration of pre-treatment data: 7 months, min 3 months , max 20 months), the pre-treatment pooled annualized eGFR slope was **-3.70mL/min/1.73 m^2/year** and post-treatment pooled annualized eGFR slope was **-1.70mL/min/1.73m^2/year (Figure 2).**

Table 2: Baseline characteristics

Baseline characteristic	Full pooled N=61	Subgroup with pre-tx values N=40
Age, years (mean +/- SD)	65 +/- 9	64 +/- 9
Sex (Female %: Male %)	31: 69	32: 68
Race, % Black or African American White Other	 11 82 7	 15 80 5
eGFR, mL/min/1.73m^2, mean ±SD	24 +/- 5	25 +/- 5
UACR, mg/g, median, (Q1, Q3)	859 (77, 1762)	574 (62, 1394)
HbA1c, % (mean ±SD)	7.1 +/- 1.4	7.1 +/- 1.4
ACEi/ARB use at baseline	87%	90%
SGLT2i use at baseline	16%	22%
GLP-1 RA use at baseline	34%	48%

ACEi, angiotensin-converting enzyme inhibitor; ARB, angiotensin receptor blocker; eGFR, estimated glomerular filtration rate; GLP-1 RA, glucagon-like peptide-1 receptor agonist; HbA1c, glycated hemoglobin; SD, standard deviation; SGLT2i, sodium-glucose cotransporter-2 inhibitor; tx, treatment; UACR, urine albumin-to-creatinine ratio.

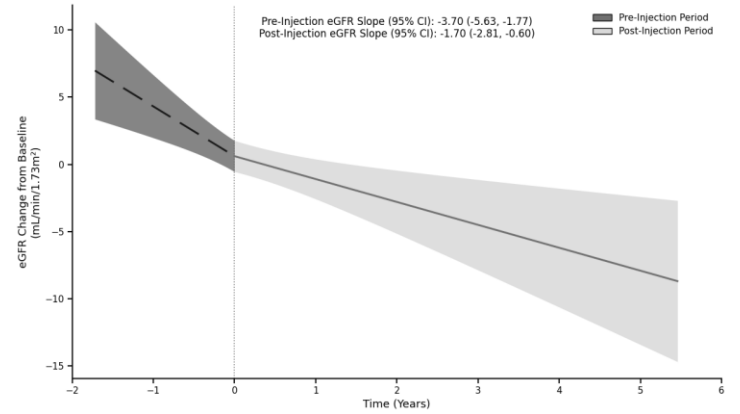
Table 3: Summary of adverse events

Related events (judged by Investigator)	n (%) N=60
SAE (biopsy related)	**3 (4.9)**
Renal haematoma	1 (1.6)
Blood loss anemia	1 (1.6)
Arteriovenous fistula	1 (1.6)
Subcapsular renal hematoma	1 (1.6)
Acute kidney injury	1 (1.6)
Haematuria	1 (1.6)
Hydronephrosis	1 (1.6)
SAE (procedure related)	**4 (6.6)**
Cardiac arrest	1 (1.6)
Acute respiratory failure	1 (1.6)
Ventricular tachycardia	1 (1.6)
Septic shock	1 (1.6)
Syncope	1 (1.6)
Abdominal pain	1 (1.6)
Subcapsular renal haematoma	1 (1.6)
SAE (possibly rilparencel related)	**1 (1.6)**
Cardiac arrest	1 (1.6)
Acute respiratory failure	1 (1.6)
Ventricular tachycardia	1 (1.6)
Septic shock	1 (1.6)
TEAEs (procedure or treatment):‡	29 (48)

SAE, serious adverse event; TEAE, treatment emergent adverse event.
‡The most common were injection-site pain, subcapsular renal hematoma, renal hematoma, fatigue, procedural nausea, and procedural pain — each in <10% of participants.

RESULTS: EFFICACY

Figure 2: Pooled annualized eGFR slope before and after first rilparencel injection (n=40)



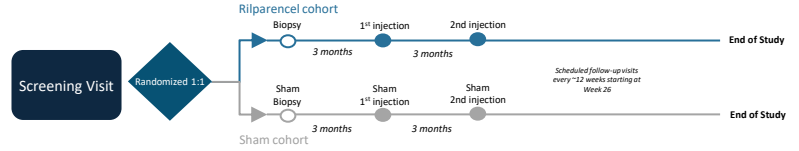
SUMMARY | LIMITATIONS

- The mean baseline eGFR was **24 mL/min/1.73 m^2 (CKD Stage 4)** and the annualized post-treatment eGFR slope was **-1.78 mL/min/1.73 m^2**
- For the 40 patients with pre-treatment data available, the post-treatment annualized slope of -1.70 mL/min/1.73 m^2 suggests attenuation of kidney function decline based on annualized pre-treatment slope.
- Rilparencel, kidney biopsies and injections were well tolerated (**Table 3**)
- **Rilparencel injections may preserve kidney function** with an acceptable safety profile.
- Limitations of this analysis include differences in dosing interval, administration into one vs both kidneys, differing rilparencel product formulations (fresh vs cryopreserved); additionally, the small number of participants and the evolution of standard of care to include newer kidney protective treatments over the years the studies were conducted in limits generalizability.

NEXT STEPS

A multi-center, blinded Phase 3 randomized controlled study, REGEN-006, is ongoing with topline data expected in 2027.

Figure 3: REGEN-006 Phase 3 study design (NCT05099770)



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2. Čižman B, Butler EL, Stavas J, et al. Clin J Am Soc Nephrol. 2026

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