



## TX 200 – KT02

### Status ongoing: active inclusion

S-nummer: S63288 Phase I - IIa study

Principal Investigator: prof. dr. Dirk Kuypers

Title: A multicenter, open-label, single ascending dose, dose-ranging-, phase I/2a study to evaluate the safety and tolerability of an autologous antigen-specific chimeric antigen receptor T regulatory cell (CAR-Tregs) therapy (TX200-TR101) in living donor renal transplant recipients.

Purpose and rationale: To evaluate the short-term safety and tolerability of TX200-TR101 when given as a single ascending dose to HLA-A\*02 mismatched living donor renal transplant recipients (HLA-A\*02 negative recipient and HLA-A\*02 positive donor).

Primary endpoint: Incidence and grade of TEAEs, including SAEs, within 28 days post TX200-TR101 infusion.

Medication/treatment: TX200-TR101 is a genetically modified cell therapy product. It consists of ex vivo expanded autologous regulatory T cells (Treg) collected from an HLA-A\*02 negative organ recipient prior to transplant, which are genetically modified to express a CAR specific to the donor HLA-A\*02 at their surface membrane. TX200-TR101 will be re-administered approximately 12 weeks posttransplant surgery.

Duration of study: The duration of study participation will be up to 112 weeks for transplant recipients to be administered TX200-TR101 (up to 28 weeks pre-transplant and 84 weeks post-transplant) consisting of approx. 31 visits. Up to 88 weeks for control transplant recipients (up to 4 weeks pre-transplant and 84 weeks post-transplant) consisting of approx. 29 visits. The duration of study participation for transplant donors will be up to 4 weeks (for screening assessments and collection of blood samples for biomarker analysis) consisting of approx. 2 visits.

#### Key inclusion criteria:

- Male or female aged between 18 and 70 years
- Have diagnosis of ESRD and currently waiting for a new kidney from an identified live donor
- Single organ recipients
- HLA-A\*02 negative typing (the kidney graft needs to be HLA A\*02 positive)
- HLA-A\*69 negative typing

#### Key exclusion criteria:

- HLA identical to the prospective organ donor
- Prior organ transplant
- Known hypersensitivity or contraindications for ATG, tacrolimus or MPA/MMF
- Positive serology for HIV or active infection (HBV, HCV or EBV)
- Positive flow cytometric crossmatch using donor lymphocytes (T and B cells) and recipient serum
- History within the last 5 years of malignancy
- Hb < 9 g/dL, platelets < 80x10<sup>9</sup>/L, WBC < 3x10<sup>9</sup>/L, AST/ALT ≥ 3xULN, total bilirubin ≥ 2xULN