



# Frequently Asked Questions

## RP-L301- Gene Therapy Clinical Trial for Pyruvate Kinase Deficiency

### What is Pyruvate Kinase Deficiency?

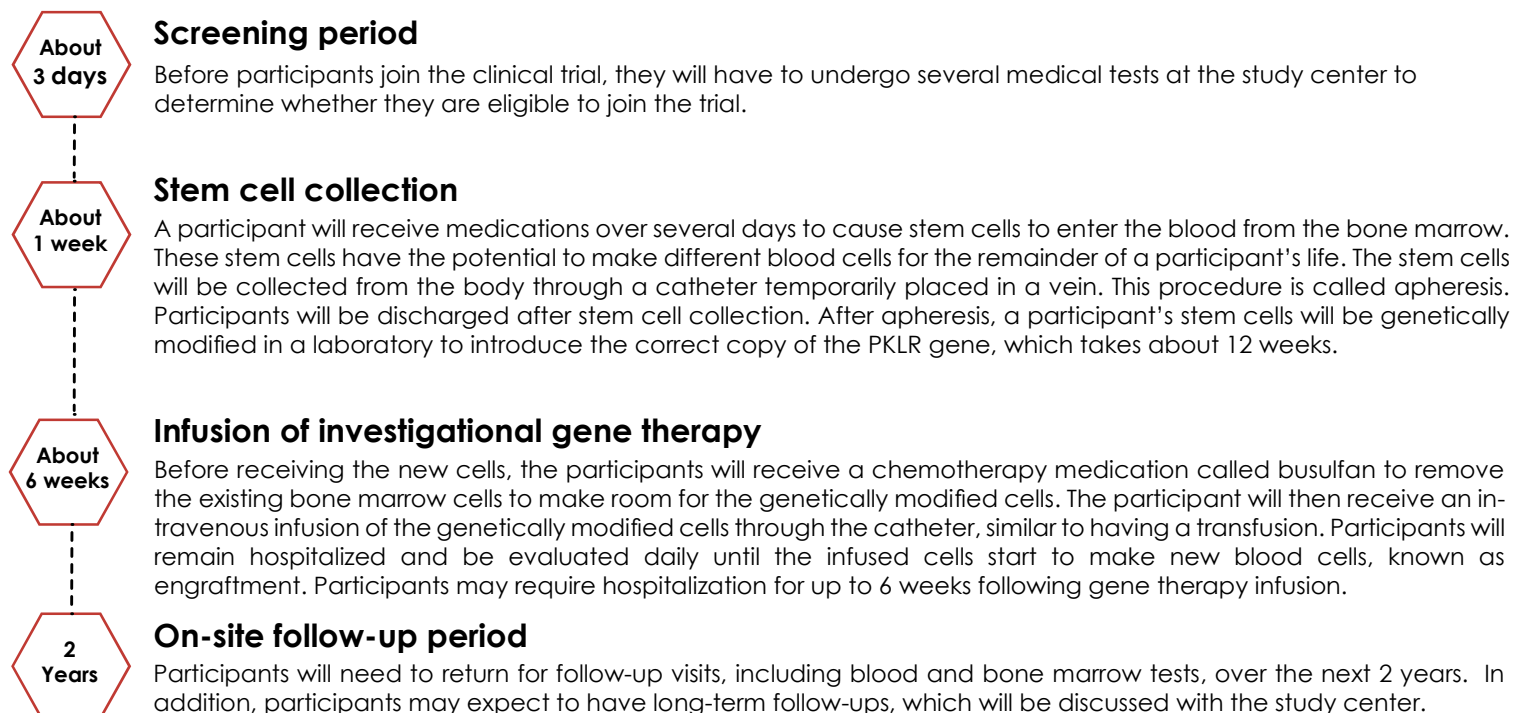
PKD is a rare, inherited blood disorder. PKD is caused by a defect in the *PKLR* gene, leading to red blood cell (RBC) break down, which causes anemia, jaundice, and chronic fatigue.

### Who is eligible to participate in the gene therapy clinical trial?

Individuals who:

- Have a PKD diagnosis with a confirmed *PKLR* mutation;
- Are 8 through 55 years of age;
- Have severe, transfusion-dependent anemia after removal of the spleen;
- Are not receiving other experimental therapies; and
- Have no evidence of severe organ damage

### What does participation in the PKD gene therapy clinical trial involve?



### What is the impact of the investigational gene therapy on future fertility?

Study participants, both adult and parent(s)/guardian(s) of pediatric participants, will be informed by the study doctor of the risks of infertility/impaired fertility during the consent process. Rocket Pharma covers fertility preservation procedures for study participants who are at risk of their fertility being impaired due to participation in the investigational gene therapy clinical trial.

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