RubiusTherapeutics™

About Rubius Therapeutics

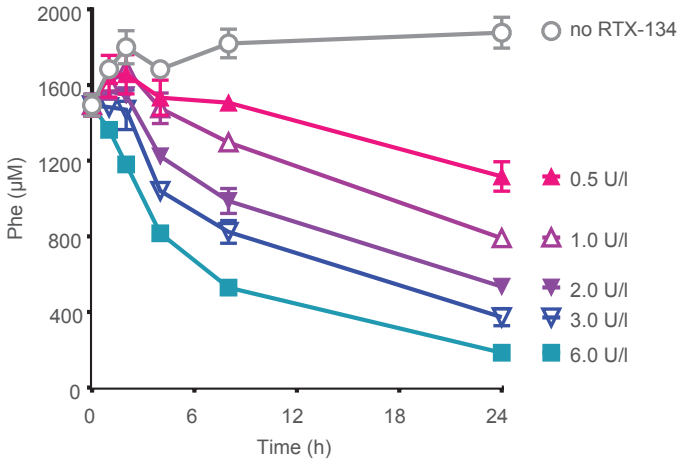
Rubius Therapeutics is developing an entirely new class of allogeneic cellular therapies. The Company has designed its proprietary RED PLATFORM® to genetically engineer and culture Red Cell Therapeutics™ (RCTs) to express biotherapeutic proteins within or on the surface of the cell for the potential treatment of rare diseases, cancer and autoimmune diseases.

About Phenylketonuria (PKU) and RTX-134

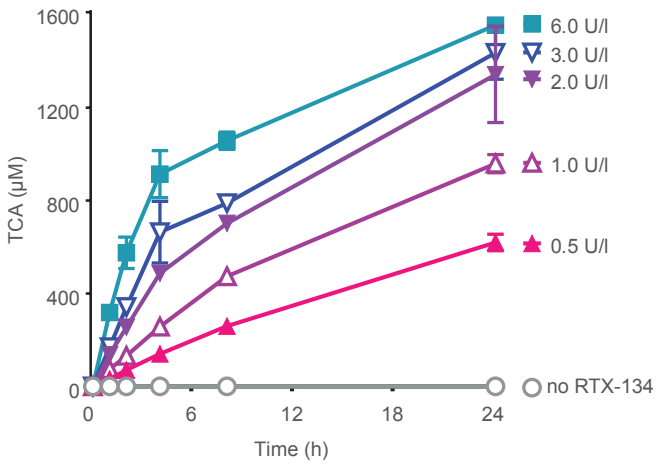
PKU is an inherited, rare disease in which the body is unable to effectively metabolize the amino acid phenylalanine. The accumulation of phenylalanine in the blood causes damage to the central nervous system and a range of symptoms, including intellectual disability, delayed development and impaired cognitive function.

RTX-134 is an allogeneic cellular therapy for the treatment of PKU in development at Rubius Therapeutics. RTX-134 expresses the enzyme phenylalanine ammonia lyase (PAL) inside the cell and is designed to shield PAL from being cleared by the immune system. In preclinical studies, phenylalanine was shown to diffuse into RTX-134, where PAL converted phenylalanine into ammonia and trans-cinnamic acid, metabolites that are cleared by the body. Compared to current therapeutics interventions, RTX-134 may have a longer and more sustained treatment duration given the 120-day half-life of red blood cells and may avoid immune-driven adverse events and reduction in efficacy, resulting from antibody formation.

IN VITRO PHENYLALANINE DEPLETION



IN VITRO TRANS-CINNAMIC ACID PRODUCTION



PAL was highly active when expressed inside the cell of RTX-134
PAL converted phenylalanine to trans-cinnamic acid

About RTX-134 Phase 1/2a Clinical Trial

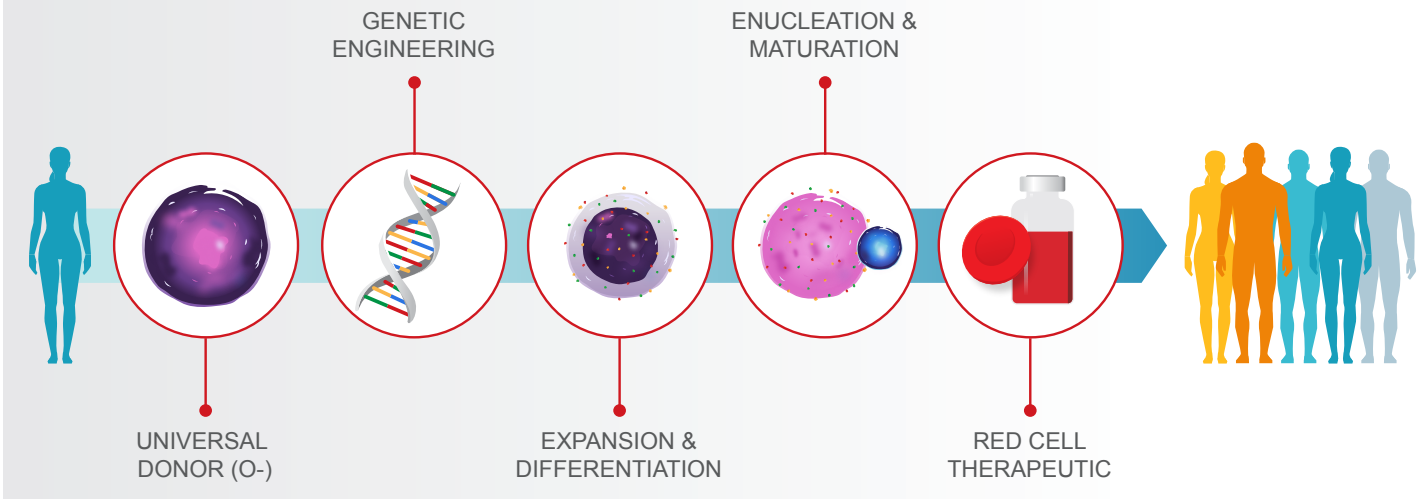
Rubius Therapeutics plans to submit an Investigational New Drug (IND) application to the FDA during the first quarter of 2019. This clinical study will evaluate 25-35 patients with classic and moderate PKU to determine preliminary safety, efficacy and dose schedule. The study is expected to take about 12 months to enroll.

Manufacturing RCT™ Products

With the RED PLATFORM®, Rubius Therapeutics can generate a wide variety of allogeneic, ready-to-use RCT product candidates with a universal and proprietary process:

- CD34+ hematopoietic precursor cells are collected by apheresis, purified and isolated from a healthy O negative donor
- These precursor cell populations are expanded and genetically engineered in a bioreactor to express one or more biotherapeutic proteins
- The cells are then exposed to a defined media formulation to promote further expansion and differentiation until they mature into enucleated reticulocytes
- The RCTs are purified to separate the mature RCTs from nucleated erythroid precursor cells, formulated and stored at 4°C
- The remaining cell looks and behaves like a normal red blood cell and now has the biotherapeutic protein(s) of interest inside or on the cell surface
- A donation from one O negative donor can result in hundreds to thousands of doses depending on the intended therapeutic application

RED PLATFORM™



Rubius Therapeutics has additional RCT product candidates in preclinical development for the potential treatment of chronic refractory gout, homocystinuria, hyperoxaluria and acute intermittent porphyria. For more information about Rubius Therapeutics and RTX-134, please visit www.rubiustx.com.

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