About Rubius Therapeutics

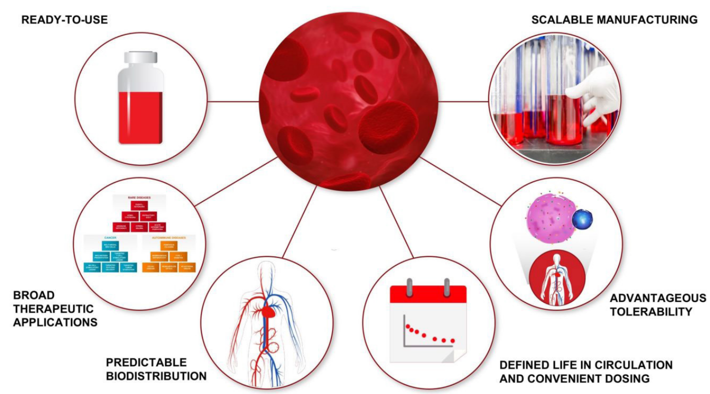
Rubius Therapeutics is a biopharmaceutical company pioneering a new era of cellular medicines. Our proprietary RED PLATFORM™ was designed to genetically engineer and culture Red Cell Therapeutics™ that are selective, potent and ready-to-use cellular therapies for the potential treatment of several diseases across multiple therapeutic areas.

Our initial focus is to advance RCT™ product candidates for the treatment of rare diseases, cancer and autoimmune diseases by leveraging three distinct therapeutic modalities — cellular shielding, potent cell-cell interaction and tolerance induction.

We plan to file our first Investigational New Drug application (IND) for RTX-134 in phenylketonuria during the first quarter of 2019. We are planning to file additional INDs in rare diseases, cancer and autoimmune diseases in 2019, 2020 and thereafter.

Versatile Platform Enabling Multiple Modalities

Our RED PLATFORM™ and RCT™ product candidates potentially represent a transformative step in the evolution of cellular therapies and are designed to provide the following attributes:



A Broad and Diverse Pipeline

Our first product candidates were selected based on:

- Potential to address unmet medical needs
- Feasibility as determined by our preclinical research and development efforts
- Potential to rapidly achieve proof-of-concept based on easy-to-measure validated regulatory endpoints
- Significant commercial potential

PRODUCT CATEGORY	MODALITY	CANDIDATE	DISCOVERY	LEAD OPTIMIZATION	IND ENABLING	PHASE 1	PHASE 2	PHASE 3
RARE DISEASES	Cellular Shielding	RTX-134	Phenylketonuria			Estimated IND Filing 1Q'19		
		RTX-Uricase/URAT1	Refractory Gout					
		RTX-CBS	Homocystinuria					
		RTX-OxOx	Hyperoxaluria					
		RTX-ALA-D	AIP					
CANCER	Potent Cell-Cell Interaction	RTX-212	R/R aPD1 Solid Tumor					
		RTX-212	R/R AML Post-HSCT					
		RTX-Targeted-4-1BBL	Tumor Targeted					
		RTX-4-1BBL	Solid Tumor					
		RTX-aAPC	Cancer					
AUTOIMMUNE DISEASES	Tolerance Induction	RTX-PV	Pemphigus Vulgaris					
		RTX-T1D	Type 1 Diabetes					

Lead Programs

Rare Diseases

RTX-134

We plan to file an IND application for RTX-134 during the first quarter of 2019 for the potential treatment of phenylketonuria (PKU), a rare inherited disease that causes build-up of the amino acid phenylalanine (Phe). Without the functional enzymes to process Phe, a buildup can occur, causing impaired cognitive function. RTX-134 is an RCT™ product candidate that expresses the enzyme phenylalanine ammonia lyase and coverts Phe to the metabolite transcinnamic acid, which is naturally cleared by the body.

RTX-Uricase/URAT1

Gout is a metabolic and inflammatory disease often affecting middle-aged to elderly men and postmenopausal women. After years of repetitive attacks, patients develop chronic refractory gout, which is characterized by the buildup of tophi, or deposits of uric acid crystals in the joints, kidney and heart. Tophi can lead to the development of chronic arthritis and an increased risk of developing kidney stones, chronic renal insufficiency and cardiovascular disease. Once patients reach this stage, they generally experience multiple attacks per year. RTX-Uricase/URAT1 is an RCT™ product candidate that has been genetically engineered to express the enzyme uricase along with URAT1, a uric acid transporter that ensures optimal uptake of uric acid into the cell. We expect RTX-Uricase/URAT1 to augment a patient’s ability to clear uric acid levels in the blood.

Cancer

RTX-212

We are developing a pipeline of RCT™ product candidates that target T cells, natural killer (NK) cells, dendritic cells and tumor cells. RTX-212 is an RCT™ product candidate that co-expresses 4-1BBL, a ligand that binds to and activates the co-stimulatory receptor 4-1BB, an important regulator of the immune system, and IL-15TP, a fusion of IL-15 and IL-15 receptor alpha, with the goal of improving anti-tumor activity and overcoming resistance to immunotherapy.

RTX-212 is our first potential treatment for patients whose disease has progressed on checkpoint inhibitor therapy across a range of solid tumor types and in patients with acute myeloid leukemia, or AML, following hematopoietic stem cell transplantation. AML is characterized by the rapid growth of abnormal cells that build up in the bone marrow and bone and interfere with normal blood cells, including platelets, red blood cells and neutrophils.

Autoimmune Diseases

We plan to select our first clinical candidate designed to induce tolerance in an autoimmune disease in 2019.

Ticker Symbol

NASDAQ: RUBY

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