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## Engineered erythrocytes as an anti-tumor therapy through induction of apoptosis or immune-checkpoint inhibition

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Rubius Therapeutics has built a platform for producing allogeneic Red Cell Therapeutics (RCTs), genetically modified red blood cells expanded ex vivo. Using lentiviral gene delivery, RCTs are able to harbor active intracellular as well as extracellular proteins, ranging from enzymes and cell targeting moieties to agonists and antibodies. RCTs represent a potentially transformational oncology platform, enabling multiple distinct modalities including tumor starvation, enhanced apoptotic signaling and immune checkpoint inhibition, among others. Further studies are underway to evaluate the ability of these and other RCTs to access and kill tumor cells *in vitro* and *in vivo*. These data support the development of RCTs as a novel class of therapeutic, enabling multiple modalities and mechanisms applicable to oncology and other indications.

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## Stem cell therapy for the treatment of severe tissue damage after radiation exposure

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The late adverse effects of pelvic radiotherapy concerns 5 to 10% of them, which could be life threatening. However, a clear medical consensus concerning the clinical management of such healthy tissue sequelae does not exist. Our group has demonstrated in preclinical animal models, that systemic (Mesenchymal Stem Cells) MSC injection is a promising approach for the medical management of gastrointestinal disorder after irradiation. We have shown that MSC migrate to damaged tissues and restore gut functions after irradiation. The clinical status of four first patients suffering from severe pelvic side effects resulting from an over-dosage was improved following MSC injection in a compassionate situation. A quantity of 2x10<sup>6</sup> - 6x10<sup>6</sup> MSC/kg were infused intravenously to the patients. Pain, hemorrhage, frequency of diarrheas and fistulisation as well as the lymphocyte subsets in peripheral blood were evaluated before MSC therapy and during the follow-up. Two patients revealed a substantiated clinical response for pain and hemorrhage after MSC therapy. In one patient, pain reappeared after 6 months and again substantially responded on a second MSC infusion. A beginning fistulisation process could be stopped in one patient resulting in a stable remission for more than 3 years of follow-up. The frequency of painful diarrhea diminished from an average of 6/d to 3/d after the first and 2/d after the second MSC injection in one patient. In all patients, prostate cancer remained in stable complete remission. A modulation of the lymphocyte subsets towards a regulatory pattern and deminution of activated T cells accompanies clinical response in refractory irradiation-induced colitis. No toxicity occurred. MSC therapy was safe and effective, with no pain, diarrhea, haemorrhage, inflammation, fibrosis and limited fistulisation. For patients with refractory chronic inflammatory and fistulising bowel diseases, systemic MSC injections represent a safe option for salvage therapy. A clinical phase II trial will start in 2017.

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