



Roca Therapeutics secures first-ever EMA recognition of Radiation Maculopathy as distinct indication - and Orphan Drug Designation

Lead clinical candidate, RCT002, first-in-class eye-drop therapy designed to address both resistant neovascularization and inflammation in patients experiencing vision loss after radiotherapy for uveal melanoma, head and neck or brain cancers, on track to enter first-in-human clinical trials in 2026

Nice, France, August 26, 2025 – Roca Therapeutics (Roca), a pioneer in non-invasive, first-in-class therapies for severe ocular diseases, today announces the granting of Orphan Drug Designation (ODD) by the European Medicines Agency (EMA) for its lead clinical candidate, RCT002, in the treatment of Radiation Maculopathy. This regulatory milestone is the result of Roca's strategic and sustained efforts to elevate Radiation Maculopathy from being viewed merely as a secondary medical complication to being formally recognized by the EMA as a distinct and differentiated clinical indication.

Radiation Maculopathy is a vision-threatening retinal complication that arises from exposure of the macular region to ionizing radiation. It commonly affects patients who have undergone radiotherapy, not only for ocular or periocular tumors, but also for malignancies of the head and neck or brain, where incidental radiation to the eye may occur. For this indication, there is currently no approved standard of care and off-label treatment remains the prevailing clinical practice, albeit with poor results.

Developed within two years from its proprietary platform, RCT002 is a game-changing first-in-class small molecule eye-drop. RCT002 has been rationally designed to safely and concomitantly address resistant neovascularization, inflammation, fibrosis and oxidative stress.

"Recognition of Radiation Maculopathy as a distinct indication marks a critical step forward in patient care - enabling earlier diagnosis, targeted therapeutic development and expanded access to specialized treatments for patients experiencing vision loss after radiotherapy for eye, head and neck or brain cancers," said Zaki Sellam, executive chairman of Roca Therapeutics. "This orphan designation also opens the door to accelerated innovation and regulatory support, creating a clear pathway for investment in a high-need, under-addressed therapeutic area."

Roca is currently fundraising to support RCT002's first-in-human clinical trial, on track to commence in 2026.

About RCT002

RCT002 is a topically administered small-molecule drug candidate designed to target CXCR2 and DRAK1, two key drivers of treatment resistance in retinal diseases. By simultaneously addressing exacerbated angiogenesis, oxidative stress and chronic macrophage-driven inflammation, RCT002 offers a novel, multi-mechanistic approach distinct from existing anti-VEGF therapies. The development of RCT002 is supported by a solid translational evidence pharmacological data package. Its lead indication is Radiation Maculopathy (RM); secondary larger indications include Diabetic Macular Edema (DME) and other neovascular retinopathies.

About Roca Therapeutics

Co-founded from the ground up in 2021 by a team of academic innovators (Université Côte d'Azur/CNRS/Inserm/IRCAN/ICN) and Landmark BioVentures AG - who led the corporate and drug development strategy, Roca Therapeutics, a French biotechnology company, is pioneering non-invasive, first-in-class therapies for severe ocular diseases with high unmet medical need.

Roca Therapeutics' name was inspired by Monaco's 'La Roca' (The Rock), an iconic landmark crowned by the Prince's Palace. This was proposed by one of the co-founders, native to Monaco, to reflect both the company's origins and its ambitious mission to combat disease.

The company is developing topical small-molecule drugs based on a systems biology approach, simultaneously targeting key pathogenic mechanisms such as exacerbated angiogenesis, oxidative stress and chronic macrophage-driven inflammation - hallmarks of resistance to standard anti-VEGF therapies.

Roca has secured early-stage financial and strategic backing from the 3B Future Health Fund. The company is also supported by a broad coalition of institutional partners, including SATT Sud-Est, Bpifrance, Université Côte d'Azur, Eurobiomed, Incubateur PACA-Est, Métropole Nice Côte d'Azur, the Provence-Alpes-Côte d'Azur Regional Council and European Regional Development Fund (FEDER). These strategic partnerships and financial milestones reflect strong confidence in RCT002's potential to reshape the therapeutic landscape in retinal diseases and position Roca as a frontrunner in ophthalmic innovation.

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