PRESS RELEASE

ImCheck's Announces EMA Orphan Drug Designation for ICT01 as Treatment for Acute Myeloid Leukemia

Following the FDA ODD, European designation underscores ICT01's potential as a novel immunotherapy approach in AML

Marseille, France, July 21, 2025 – ImCheck Therapeutics today announced that the European Medicines Agency (EMA) has granted Orphan Drug Designation (ODD) to its lead program, ICT01, a humanized anti-butyrophilin 3A (BTN3A) monoclonal antibody designed to selectively activate γ9δ2 T cells, for the treatment of acute myeloid leukemia (AML). The designation in the EU follows the recently granted U.S. FDA ODD and provides additional validation of the therapeutic potential of ICT01 in AML, a disease with high unmet medical need and limited treatment options for older or unfit patients who are not eligible for intensive chemotherapy.

"Securing orphan drug designation from both the EMA and the FDA in close succession is a major regulatory milestone for ImCheck," said Pierre d'Epenoux, Chief Executive Officer of ImCheck Therapeutics. *"It reflects growing international recognition of ICT01's potential and supports our objective to accelerate clinical development in both the U.S. and Europe. The EMA ODD's broad market exclusivity for ICT01 once approved provides additional value as we consider our development strategy."*

"The EMA's rapid decision reinforces the highly encouraging clinical data supporting ICT01 and its differentiated mechanism of action," added Stephan Braun, MD, PhD, Chief Medical Officer of ImCheck Therapeutics. *"By selectively activating γ9δ2 T cells, a powerful component of the immune system, ICT01 offers a novel therapeutic pathway in AML. This momentum brings us closer to our goal of delivering new therapeutic options to AML patients who currently have limited treatment options."*

The EMA's orphan drug designation is granted to medicines intended for the treatment of life-threatening or chronically debilitating rare conditions affecting fewer than 5 in 10,000 people in the EU. Benefits of the designation include protocol assistance, reduced regulatory fees, and ten years of market exclusivity in Europe following approval.

About the medical need in AML

Acute myeloid leukemia (AML) remains a significant clinical challenge, particularly for older or unfit patients who cannot tolerate intensive chemotherapy. While the combination of venetoclax and azacitidine has become the standard non-intensive regimen, it is not curative, and relapse rates remain high. Most patients are not eligible for stem cell transplantation, often due to age, comorbidities, or insufficient response, and face limited treatment options and poor overall survival. Despite AML's known sensitivity to immune-mediated control, current immunotherapies targeting PD-1, TIM-3, or CD47 have not delivered meaningful clinical benefit. This underscores the urgent need for novel immuno-oncology approaches. Recently, γ9δ2 T cells, with their cytotoxic activity and unique dual role in both innate and adaptive immunity, have emerged as promising immune modulators. Their association with reduced relapse and prolonged survival, particularly in the post-transplant setting, suggests that enhancing their anti-leukemic potential could offer a meaningful new treatment option for high-risk AML patients.

About ICT01

ICT01 is a humanized, anti-BTN3A (also known as CD277) monoclonal antibody that selectively activates γ9δ2 T cells, which are responsible for immunosurveillance of malignancy and infections. The three isoforms of BTN3A targeted by ICT01 are overexpressed on many solid tumors (e.g., melanoma, urothelial cell, colorectal, ovarian, pancreatic, and lung cancer) and hematologic malignancies (e.g., leukemia and lymphomas) and also expressed on the surface of innate (e.g., γδ T cells and NK cells) and adaptive immune cells (T cells and B cells). BTN3A is essential for the activation of the anti-tumor immune response of γ9δ2 T cells.

As demonstrated by data presented at past AACR, ASCO, ASH, ESMO and SITC conferences, ICT01 selectively activates circulating γ9δ2 T cells leading to migration of γ9δ2 T cells out of the circulation and into the tumor tissue and triggers a downstream immunological cascade through secretion of pro-inflammatory cytokines, including but not limited to IFNγ and TNFα, further augmenting the anti-tumor immune response. Anti-tumor activity and efficacy of ICT01 have been shown in patients across several cancer indications.

About IMCHECK THERAPEUTICS

ImCheck Therapeutics is developing a new generation of immunotherapeutic antibodies targeting butyrophilins, a novel superfamily of immunomodulators. By unlocking the power of γ9δ2 T cells, ImCheck's innovative approach has the potential to transform treatments across oncology, autoimmune, and infectious diseases.

The lead clinical-stage program, ICT01, has been advancing to late-stage trials, demonstrating a unique mechanism of action that modulates both innate and adaptive immunity. These "first-in-class" activating antibodies may deliver superior clinical outcomes compared to first-generation immunotherapy approaches, in particular in rationale combinations with immune checkpoint inhibitors and immunomodulatory anti-cancer drugs. Additionally, ImCheck's pipeline compounds are progressing toward clinical development for autoimmune and infectious diseases.

The company benefits from the pioneering research of Prof. Daniel Olive (INSERM, CNRS, Institut Paoli Calmettes, Aix-Marseille University), a global leader in γ9δ2 T cells and butyrophilins, as well as the expertise of a seasoned management team and the commitment of leading U.S. and European investors.

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