PRESS RELEASE

ImCheck's ICT01 Receives FDA Orphan Drug Designation for Treatment of Acute Myeloid Leukemia

Clinical data showing unprecedented remission rates in newly diagnosed AML patients support advancing ICT01 into pivotal trials

Marseille, France, July 18, 2025, 11:00 am CET – ImCheck Therapeutics today announced that the U.S. Food and Drug Administration (FDA) 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). AML remains a significant clinical challenge, particularly for older or unfit patients who are not eligible for intensive chemotherapy.

"*Receiving FDA orphan drug designation for ICT01 is a significant recognition of ICT01's innovative therapeutic potential to meet the urgent unmet medical needs of AML patients,*" said **Stephan Braun, MD, PhD, Chief Medical Officer of ImCheck Therapeutics**. *"This important regulatory milestone reinforces our confidence that ICT01 will become the first immunotherapy for AML patients and supports our goal of rapidly advancing ICT01 into pivotal studies based on the unprecedented results observed in the clinic to date."*

In an oral presentation at the 2025 ASCO Annual Meeting, ImCheck reported results from the Phase I/II EVICTION study, evaluating ICT01 in combination with azacitidine and venetoclax (Aza-Ven) in newly diagnosed AML patients unfit for intensive chemotherapy. Remarkably high remission rates and a positive overall survival signal were observed across a broad range of molecular subtypes, in particular those that are typically less responsive to Aza-Ven. The combination demonstrated a clinically well-manageable safety profile, with Grade ≥3 adverse events consistent with the expected hematological toxicity of Aza-Ven and AML itself.

"Orphan drug designation is a catalyst," added **Pierre d'Epenoux, Chief Executive Officer of ImCheck Therapeutics.** *"It validates our regulatory strategy, de-risks and supports clinical development acceleration, and sends a strong signal about the unique potential of ICT01 to transform AML treatment as well as other solid tumor indications."*

The FDA's orphan drug designation is granted to drugs and biologics intended for the treatment, diagnosis, or prevention of rare diseases affecting fewer than 200,000 people in the United States. The designation is designed to encourage the development of therapies for underserved patient populations and offers benefits including tax credits for clinical trials, exemption from certain FDA fees, and up to seven years of marketing exclusivity upon approval. Additionally, the designation gives access to regulatory assistance for the drug development process.

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.

For further information: https://www.imcheckTherapeutics.com/

Press contacts:

US and EU
Trophic Communications
Gretchen Schweitzer
+49 (0) 172 861 8540
imcheck@trophic.eu

France
ATCG PARTNERS
Céline Voisin
+33 (0)6 62 12 53 39
imcheck@atcg-partners.com