

Poolbeg Pharma plc

Poolbeg granted FDA Orphan Drug Designation for POLB 001

27 May 2025 - [Poolbeg Pharma](#) (AIM: POLB, 'Poolbeg' or the 'Company'), a clinical-stage biopharmaceutical company focussed on the development of innovative medicines to address unmet medical needs, announces that the US Food and Drug Administration ("FDA") has granted Orphan Drug Designation ("ODD") to POLB 001 as an oral preventative therapy for T-cell engager bispecific antibody-induced Cytokine Release Syndrome ("CRS").

The FDA grants orphan status to support the development of medicines for rare disorders affecting less than 200,000 people in the US. ODD provides Poolbeg with clinical development and commercialisation benefits including the potential for a seven-year period of US market exclusivity following regulatory approval of POLB 001, potential waiver exemption of Prescription Drug User Fee Act application fees, and the potential for tax credits for qualifying clinical trials.

POLB 001 is a Phase 2 ready oral p38 MAP kinase inhibitor to prevent or treat cancer immunotherapy-induced CRS, a severe side-effect that occurs in more than 70%¹ of patients and which may lead to multi-organ failure and death. There are currently no approved preventative therapies for CRS. This ODD further validates POLB 001's scientific rationale and data which, if approved, has the potential to revolutionise cancer immunotherapy delivery by making it safer, enabling outpatient administration, reducing healthcare burden, and ultimately expanding patient access to these breakthrough immunotherapies.

Poolbeg expects the first patient to be dosed in its proposed Phase 2a in H2 2025, with interim analysis expected in H1 2026 and Phase 2a topline data in H2 2026. There are strong indications from Big Pharma to provide the necessary bispecific antibody, free of charge, to support the Phase 2a trial which represents a significant validation of the industry interest in POLB 001 and its potential. ODD strengthens POLB 001's commercial appeal for prospective partners and the Company sees potential for partnering on positive data from its Phase 2a trial.

Jeremy Skillington, PhD, Chief Executive Officer of Poolbeg Pharma, said: *"POLB 001 is potentially a breakthrough, orally delivered, preventative therapy for cancer immunotherapy-induced CRS which could significantly impact patients' lives. We were delighted to receive Orphan Drug Designation from the FDA, which is a significant development for Poolbeg and for POLB 001, one that we believe will enhance the commercial appeal for prospective partners and help bring POLB 001 to the market faster. If approved, we believe POLB 001 has the potential to improve quality of life for patients, reduce pressure on healthcare systems, and expand access to cancer immunotherapies."*

Professor Brendan Buckley, Non-Executive Director and Scientific Advisory Board Member added: *"Orphan Drug Designation from the FDA underscores the urgency and importance of developing innovative therapies for this critical unmet medical need. We look forward to progressing POLB 001 in our upcoming Phase 2a clinical trial and working closely with prospective partners and regulatory agencies to bring this potential therapy to patients as quickly as possible."*

Investor presentation

Poolbeg Pharma will provide a live presentation via the Investor Meet Company platform on Wednesday 28 May 2025 at 1:00pm BST.

The presentation is open to analysts and investors, those who already follow Poolbeg on the Investor Meet Company platform will automatically be invited. Investors can sign up to Investor Meet for free and add Poolbeg Pharma plc to their company dashboard [here](#).

References

1. Average rate from Summary of Product Characteristics (SmPCs) for Yescarta, Tecartus, Abecma, Kymriah, Carvykti, Breyanzi, Elrexfio, Columvi, Epkinly, Tecvayli and Talvey

This announcement was deemed by the company to contain inside information as stipulated under the Market Abuse Regulations (EU) no. 596/2014 as they form part of UK domestic law by virtue of the European Union (withdrawal) Act 2018. With the publication of this announcement, this information is now considered to be in the public domain.

Enquiries

Poolbeg Pharma Plc

Jeremy Skillington, CEO
Ian O'Connell, CFO

+44 (0) 207 183 1499

ir@poolbegpharma.com

Cavendish Capital Markets Ltd (NOMAD & Joint Broker)

Geoff Nash, Trisyia Jamaludin (Corporate Finance)
Nigel Birks (Life Science Specialist Sales)
Harriet Ward (ECM)

+44 (0) 207 220 0500

Shore Capital Stockbrokers Ltd (Joint Broker)

David Coaten, Harry Davies-Ball (Corporate Advisory)
Malachy McEntyre, Isobel Jones (Corporate Broking)

+44 (0) 207 408 4090

J&E Davy (Joint Broker)

Anthony Farrell, Niall Gilchrist

+353 (0) 1 679 6363

Optimum Strategic Communications

Nick Bastin, Vici Rabbetts, Elena Bates

+44 (0) 208 078 4357

poolbeg@optimumcomms.com

About Poolbeg Pharma plc

Poolbeg Pharma plc (AIM: POLB) is a clinical-stage biopharmaceutical company focussed on the development of innovative medicines to address unmet medical needs. The Company's clinical programmes target large addressable markets including, cancer immunotherapy-induced Cytokine Release Syndrome ("CRS") and metabolic conditions such as obesity with the development of an oral encapsulated glucagon-like peptide GLP-1R agonist.

For more information, please go to www.poolbegpharma.com or follow us on [X](#) and [LinkedIn](#) @PoolbegPharma.

Forward-Looking Statements

This announcement may contain forward-looking statements and the words "expect", "anticipate", "intends", "plan", "estimate", "aim", "forecast", "project" and similar expressions (or their negative) identify certain of these forward-looking statements. The forward-looking statements in this announcement are based on numerous assumptions and Poolbeg's present and future business strategies and the environment in which Poolbeg expects to operate in the future. Forward-looking statements involve inherent known and unknown risks, uncertainties and contingencies because they relate to events and depend on circumstances that may or may not occur in the future and may cause the actual results, performance or achievements to be materially different from those expressed or implied by such forward-looking statements. These statements are not guarantees of future performance or the ability to identify and consummate investments. Many of these risks and uncertainties relate to factors that are beyond Poolbeg's ability to control or estimate precisely, such as future market conditions, currency fluctuations, the behaviour of other market participants, the outcome of clinical trials, the actions of regulators and other factors such as Poolbeg's ability to obtain financing, changes in the political, social and regulatory framework in which Poolbeg operates or in economic, technological or consumer trends or conditions. Past performance should not be taken as an indication or guarantee of future results, and no representation or warranty, express or implied, is made regarding future performance. No person is under any obligation to update or keep current the information contained in this announcement or to provide the recipient of it with access to any additional relevant information.

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