RACE ONCOLOGY

ASX Announcement

US FDA Orphan Drug Designation Awarded to RC220 Bisantrene for Acute Myeloid Leukemia

- **The US FDA has extended Orphan Drug Designation (ODD) to Race's re-formulated RC220 bisantrene drug product for the treatment of Acute Myeloid Leukemia**
- **ODD confers a range of commercial advantages including US tax credits, fee waivers, grant eligibility, FDA regulatory assistance and 7-years market exclusivity in the US.**

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11 June 2024 – Race Oncology Limited ('Race') is pleased to announce that the United States Food and Drug Administration (FDA) has extended Orphan Drug Designation (ODD) to Race's proprietary formulation of bisantrene, RC220. US FDA ODD was first granted to Update Pharma Inc. in 2014, covering the RC110 formulation of bisantrene for the treatment of acute myeloid leukemia (AML). The ODD was transferred to Race in 2017 and this designation has been maintained by annual reporting of clinical and non-clinical activities involving bisantrene to the FDA.

The ODD program was established by the US FDA under *The Orphan Drug Act of 1983* to promote the development of drugs, biologics, devices or medical foods that aid in the diagnosis and/or treatment of rare diseases or conditions, defined as those affecting fewer than 200,000 people within the USA. The Orphan Drug Act has led to the development and approval of 1240 new treatments and drugs[1].

ODD provides a wide range of benefits to sponsors of new treatments for orphan diseases including:

- 7-year US marketing exclusivity for approved orphan products.
- 25% federal tax credit for expenses incurred from clinical research conducted within the United States. The tax credits may be applied towards up to 20 years of future taxes.
- Waiver of fees under the *Prescription Drug User Fee Act* (PDUFA) with a value of US$4.0m as of 2024[2].
- Qualification to apply for research grants from the Office of Orphan Products Development (OOPD) to support clinical studies of orphan drugs.
- Eligibility to receive additional regulatory assistance and guidance from the FDA in the design of an overall drug development plan.

Race Chief Executive Officer, Dr Daniel Tillett commented: *"I wish to thank the clinical team at Race for their foresight in seeking to extend FDA Orphan Drug Designation to our new RC220 bisantrene formulation. Race continues to advance bisantrene as a novel treatment for AML, however being able to leverage the additional regulatory and guidance support from the FDA that ODD provides is very welcome."*

Race Chief Medical Officer, Dr Michelle Rashford comments: *"Orphan Drug Designation is a major asset beyond AML as it enables Race to work closely and constructively with the FDA on all of our RC220 bisantrene clinical programs as we progress towards opening an FDA IND in 2025."*

Q&A

How does US FDA ODD of RC220 bisantrene directly benefit Race Oncology?

ODD qualifies Race for incentives, including tax credits for clinical trials, exemption from user fees (valued at more than US$4 million in FY2024), potentially seven years of market exclusivity post-approval, and regulatory guidance and assistance from the FDA throughout the drug development process. The additional support and guidance the FDA provides for orphan drugs is particularly valuable as we advance RC220 bisantrene as a cardioprotective anticancer agent outside of AML.

FDA ODD is in addition to IP protection for the RC220 bisantrene formulation obtainable via the patent processes which is expected to extent to 2044.

How do sponsors obtain US FDA ODD?

Companies seeking Orphan Drug Designation must submit a request to the US FDA that includes: (1) a description of the rare disease or condition of interest, along with reasons why the therapy is needed, (2) the scientific and clinical rationale for use of the drug in the rare disease, (3) a summary of the regulatory and marketing history of the drug, and (4) a justification that the disease or condition qualifies as a rare disease (i.e. affects less than 200,000 people per year in the USA).

Can Race also obtain EMA ODD for RC220 bisantrene?

The European Medicines Agency (EMA) has a similar ODD scheme to the FDA to help aid the development of new drugs and treatments for rare diseases. The major difference between the EMA and US FDA ODD programs is that the EMA ODD provides for up to 10-years post-approval marketing exclusivity. The EMA ODD requires a separate application process, but this is broadly similar to the US FDA ODD application process. Race has begun the EMA ODD process for bisantrene and will update investors on progress at the appropriate time.

References

1. *Orphan Drug Designations and Approvals Database* as of June 11 2024. www.accessdata.fda.gov/scripts/opdlisting/oopd/listResult.cfm

2. *Prescription Drug User Fee Amendments.* www.fda.gov/industry/fda-user-fee-programs/prescription-drug-user-fee-amendments

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About Race Oncology (ASX: RAC)

Race Oncology (ASX: RAC) is an ASX-listed clinical stage biopharmaceutical company with a dedicated mission to be at the heart of cancer care.

Race's lead asset, bisantrene, is a small molecule chemotherapeutic. Bisantrene has a rich and unique clinical history with demonstrated therapeutic benefits in both adult and paediatric patients, a well characterised safety profile, and compelling clinical data demonstrating an anticancer effect and less cardiotoxicity over certain anthracyclines, such as doxorubicin.

Race is advancing a reformulated bisantrene (RC220) to address the high unmet needs of patients across multiple oncology indications, with a clinical focus on anthracycline combinations, where we hope to deliver cardioprotection and enhanced anti-cancer activity in solid tumours. Race is also exploring RC220 as a low intensity treatment for acute myeloid leukaemia.

Race is investigating the effect of bisantrene on the m^6A RNA pathway, following independent research published by the City of Hope identifying bisantrene as a potent inhibitor of FTO (Fat mass and obesity-associated protein). Dysregulation of the m^6A RNA pathway has been described in numerous peer reviewed studies as a driver of a diverse range of cancers.

Race Oncology has collaborated with Astex, City of Hope, MD Anderson, Sheba City of Health, UNC School of Medicine, University of Wollongong and University of Newcastle, and is actively exploring partnerships, licence agreements or a commercial merger and acquisition to accelerate access to bisantrene for patients with cancer across the world.

Learn more at www.raceoncology.com.

If you have any questions on this announcement or any past Race Oncology announcements, please go to the Interactive Announcements page in our Investor Hub https://announcements.raceoncology.com

Race encourages all investors to go paperless by registering their details with the Company's share registry, Automic Registry Services, at www.automicgroup.com.au.

Release authorised by:

Daniel Tillett, CEO
info@raceoncology.com

Media contact:

Jane Lowe +61 411 117 774
jane.lowe@irdepartment.com.au