RACE ONCOLOGY

ASX Announcement

US FDA Rare Paediatric Disease Designation Granted to RC220 Bisantrene for the Treatment of Paediatric AML

- US FDA Rare Paediatric Disease Designation (RPDD) has been extended to Race's novel RC220 bisantrene drug product
- RPDD provides eligibility for RC220 to receive a Priority Review Voucher (PRV) upon marketing approval that can be transferred/sold to other parties
- Past sales of PRVs have averaged more than US$100 million.

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18 June 2024 – Race Oncology Limited ('Race') is pleased to announce that the United States Food and Drug Administration (US FDA) has extended Rare Paediatric Disease Designation (RPDD) to RC220 bisantrene for the treatment of childhood (paediatric) subtypes of AML. RPDD was previously granted by the US FDA to RC110 bisantrene in 2018 (ASX announcement: 18 July 2018).

US FDA RPDD is granted for new treatments of serious or life-threatening diseases which affect fewer than 200,000 people in the US and which primarily affect individuals less than 18 years of age. Approximately 70% of rare diseases are exclusively paediatric in onset, with 95% of rare diseases having no approved treatments[1].

RPDD qualifies a sponsor eligible to receive a Priority Review Voucher (PRV) from the US FDA at the time of marketing approval or authorisation for drug in the paediatric rare disease area. The RPDD for paediatric AML may enable Race to be eligible to receive a PRV that can be redeemed for an accelerated 6-month review of RC220 bisantrene or any other new drug application submitted to the US FDA. Granted PRVs may also transferred or sold to other companies for use in the same manner on the secondary market.

The reported purchase prices of PRVs to third parties on the open market have averaged more than US$100 million (range US$67.5 million to $350 million)[2]. Two PRVs have been sold in recent times for US$110 million[3,4].

In support of Race's paediatric AML program, the company has been in discussion with Associate Professor Dr Himalee Sabnis, Department of Pediatrics, Emory University School of Medicine, and a large dedicated international paediatric oncology cooperative group to explore undertaking a sponsored or investigator-initiated trial of RC220 bisantrene as a salvage treatment for paediatric AML patients.

Dr. Sabnis is a world-leading, key opinion leader in the treatment of acute myeloid leukemia (AML) in paediatric patients. While no definitive agreement has yet been established with Dr Sabnis or the paediatric oncology cooperative group, the continued and ongoing interest of eminent oncology paediatricians in bisantrene as a potential paediatric AML treatment is highly encouraging.

Race Chief Medical Officer, Dr Michelle Rashford comments: *"There is a need for new medicines designed to treat these rare childhood cancers which can be devastating for families. The US government has created incentives like the Priority Review Voucher scheme to encourage companies to invest in research and clinical studies in paediatric cancers. To be able to contribute to better treating childhood cancers like paediatric AML by collaboratively working with a dedicated international paediatric cooperative group would be very rewarding."*

Race Chief Executive Officer, Dr Daniel Tillett comments: *"US FDA RPDD is incredibly valuable as not only does it offer eligibility for the award of a PRV, but the ability to work with passionate clinicians and regulators to bring help to children and adolescents facing an enormously challenging disease with few effective treatment options."*

Q&A

What would need to be clinically demonstrated for Race to be awarded a Priority Review Voucher by the US FDA?

Race would need to demonstrate that RC220 bisantrene is able to significantly improve specific paediatric AML clinical outcomes, by either increasing patient survival or reducing serious side-effects such as damage to the heart. The ultra-rare nature of paediatric AML (less than 7 cases per million children younger than 15 years[5]) would require that any trial be modest in size, likely under 30 patients, however both the size and trial design will be discussed with the US FDA during review.

Isn't the RPD program ending in 2026?

Yes, but the RPD program is expected by everyone (including the FDA) to be extended again by the US Congress. The RPDD costs the US taxpayers nothing, is supported by all political parties in the USA, and has been extended each time it has come up for renewal with unanimous congressional support.

How advanced are the talks with the international paediatric cooperative oncology group and Dr Sabnis?

Several proposals from the international paediatric cooperative oncology group and Dr Sabnis have been received by Race and possible options for conducting the trial have been discussed, including a fully Race sponsored trial, a hybrid approach where costs and data would be shared, or a fully investigator sponsored trial under the management of the paediatric cooperative oncology group where the data could later be made available to Race. No final agreement on the best approach has been reached, but Race is highly encouraged by the continued and ongoing interest from both the paediatric cooperative oncology group and Dr Sabnis and is in regular discussion with both.

What needs to happen before any paediatric AML trial could begin?

Prior to starting any trial in the USA and other countries, a US FDA IND for RC220 bisantrene must be in place and agreement reached with the FDA and the international paediatric collaborative oncology group on the trial design and funding model must be established. Race plans to submit a US FDA Investigational New Drug application for RC220 bisantrene in 2025.

How can I learn more?

Executive Director Dr Peter Smith explains more here:
https://announcements.raceoncology.com/link/weYLoy

References

1. Health, T. L. G. *The landscape for rare diseases in 2024.* (2024) Lancet Glob. Heal. 12, e341

2. Ridley D. *Priority Review Vouchers* (2021) https://sites.fuqua.duke.edu/prioritypeviewvoucher/value/

3. *Mirum Pharmaceuticals entered into a definitive agreement to sell its Rare Pediatric Disease Priority Review Voucher for $110 million to an undisclosed company as announced investor news release* (2021) https://ir.mirumpharma.com/news-events/News/default.aspx

4. *BioMarin Sells Priority Review Voucher for $110 Million* (2022) https://investors.biomarin.com/news/news-details/2022/BioMarin-Sells-Priority-Review-Voucher-for-110-Million-02-09-2022/default.aspx

5. Creutzig, U. et al. *Diagnosis and management of acute myeloid leukemia in children and adolescents: recommendations from an international expert panel.* (2012). Blood 120, 3187–3205

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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 anticancer 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