

## **US FDA GRANTS ORPHAN DRUG DESIGNATION TO PYC DRUG CANDIDATE**

- **The US Food and Drug Administration (FDA) has granted PYC Orphan Drug Designation for its drug candidate targeting Retinitis Pigmentosa type 11 (RP11) (known as VP-001)**
- **VP-001 is the first drug candidate for patients with this blinding disease of childhood to have progressed into human trials**
- **The encouraging efficacy data from PYC's single dose studies in patients with RP11 is set to be complemented by additional data from two concurrent multiple dose studies in Q4 2024**
- **The Orphan Drug Designation confers a range of benefits including tax credits and reduced regulatory fees on the VP-001 program as it progresses towards a New Drug Application<sup>1</sup>**

### **PERTH, Australia and SAN FRANCISCO, California – 21 October 2024**

PYC Therapeutics (ASX:PYC) is a clinical-stage biotechnology company creating first in class precision therapies for patients with genetic diseases and no treatment options available. One of the Company's assets is a first-in-class drug candidate currently progressing through multiple concurrent clinical trials in patients with a blinding eye disease called Retinitis Pigmentosa type 11 (RP11)<sup>2</sup>.

PYC today announces the receipt of Orphan Drug Designation (ODD) from the US Food and Drug Administration (FDA) for this drug candidate (known as VP-001). ODD is given to drug candidates designed to treat rare diseases. Benefits of an ODD include tax credits for qualified clinical trials, exemptions from some regulatory fees and the potential for 7 years of market exclusivity post approval<sup>3</sup>.

### **PYC's RP11 Program Overview**

- Retinitis Pigmentosa type 11 (RP11) is a blinding disease of childhood affecting 1 in every 100,000 people
- RP11 is caused by a mutation in 1 copy of the *PRPF31* gene leading to a protein insufficiency in photoreceptor and Retinal Pigment Epithelial (RPE) cells

<sup>1</sup> Subject to the risks set out in the Company's ASX filing of 14 March 2024

<sup>2</sup> See ASX announcement 12 August 2024

<sup>3</sup> See FDA guidance entitled 'Medical products for rare diseases and conditions'

- VP-001 increases expression of *PRPF31* back to wild-type ('unaffected') levels in RP11 patient-derived retinal organoids and iPSC-RPE<sup>4</sup> (RPE grown from patients after turning a skin sample from the patient into an induced Pluripotent Stem Cell (iPSC) and then into the specific cell type in the eye that is affected by the disease to provide a human model of the disease-affected eye outside of a human)
- VP-001 is the first drug candidate to have progressed into human trials for RP11 and has been granted fast track and orphan status by the FDA<sup>5</sup>
- RP11 represents an estimated >\$1 billion p.a. addressable market<sup>6</sup>

### About PYC Therapeutics

PYC Therapeutics (ASX: PYC) is a clinical-stage biotechnology company creating a new generation of RNA therapies to change the lives of patients with genetic diseases. The Company utilises its proprietary drug delivery platform to enhance the potency of precision medicines within the rapidly growing RNA therapeutic class. PYC's drug development programs target monogenic diseases – **the indications with the highest likelihood of success in clinical development**<sup>7</sup>.

The Company was the first to progress a drug candidate for a blinding eye disease of childhood (Retinitis Pigmentosa type 11) into human trials. The Company has also commenced human trials in a second drug program targeting another blinding eye disease (Autosomal Dominant Optic Atrophy) and has a third drug development program targeting Polycystic Kidney Disease which is anticipated to commence human trials in early 2025.

For more information, visit [pyctx.com](http://pyctx.com), or follow us on LinkedIn and X.

### Forward looking statements

*Any forward-looking statements in this ASX announcement have been prepared on the basis of a number of assumptions which may prove incorrect and the current intentions, plans, expectations, and beliefs about future events are subject to risks, uncertainties and other factors, many of which are outside the Company's control. Important factors that could cause actual results to differ materially from assumptions or expectations expressed or implied in this ASX announcement include known and unknown risks. Because actual results could differ materially to assumptions made and the Company's current intentions, plans, expectations, and beliefs about the future, you are urged to view all forward-looking statements contained in this ASX announcement with caution. The Company undertakes no obligation to publicly update any forward-looking statement whether as a result of new information, future events or otherwise.*

*This ASX announcement should not be relied on as a recommendation or forecast by the Company. Nothing in this ASX announcement should be construed as either an offer to sell or a solicitation of an offer to buy or sell shares in any jurisdiction.*

<sup>4</sup> See ASX Announcement of 7 October 2020

<sup>5</sup> FDA: US Food and Drug Administration. Refer to ASX announcements of 2 August 2023 and 21 October 2024

<sup>6</sup> Market valuation informed by patient prevalence (See: Sullivan L, et al. Genomic rearrangements of the *PRPF31* gene account for 2.5% of autosomal dominant retinitis pigmentosa. *Invest Ophthalmol Vis Sci.* 2006;47(10):4579-88) and median orphan drug pricing of \$150k p.a. (Evaluate Pharma. Orphan Drug Report. 2019)

<sup>7</sup> Advancing Human Genetics Research and Drug Discovery through Exome Sequencing of the UK Biobank <https://doi.org/10.1101/2020.11.02.2022232>

*This ASX announcement was approved and authorised for release by the CEO of PYC Therapeutics Limited*

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