PYC Therapeutics

20 January 2025

FDA GRANTS RARE PEDIATRIC DISEASE DESIGNATION TO PYC DRUG DEVELOPMENT PROGRAM

- **The US Food and Drug Administration (FDA) has granted PYC Rare Pediatric Disease (RPD) designation for its VP-001 drug development program**

PERTH, Australia and SAN FRANCISCO, California – 20 January 2025

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[1] is an investigational drug candidate (known as VP-001) that addresses the underlying cause of a blinding eye disease of childhood known as Retinitis Pigmentosa type 11 (RP11). VP-001 is currently progressing through phase 1/2 studies and PYC is preparing to initiate a potentially registrational trial for VP-001 in 2025[2].

The Company today announces that VP-001 has been granted Rare Pediatric Disease (RPD) designation by the US Food and Drug Administration (FDA) for *PRPF31*-associated vision loss. The RPD program aims to incentivise drug development for serious and rare diseases affecting children[3].

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 and commercially proven RNA therapeutic class. PYC's drug development programs target monogenic diseases – **the indications with the highest likelihood of success in clinical development**[4].

[1] PYC owns 96% of VP-001 with the remaining 4% shareholding held by the Lions Eye Institute
[2] Subject to the risks and uncertainties set out in the Company's ASX filings of 14 March 2024
[3] US FDA. Rare Pediatric Disease Designation and Priority Review Voucher Programs – Information for product sponsors
[4] Advancing Human Genetics Research and Drug Discovery through Exome Sequencing of the UK Biobank https://doi.org/10.1101/2020.11.02.20222232

PYC's drug development programs

Retinitis Pigmentosa type 11

- A blinding eye disease of childhood affecting 1 in every 100,000 people[5]
- Currently progressing through phase 1/2 clinical trials with preparation under way for a potentially registrational trial to commence in 2025[6]

Autosomal Dominant Optic Atrophy

- A blinding eye disease of childhood affecting 1 in every 35,000 people[7]
- Currently progressing through clinical trials with human safety and efficacy read-outs anticipated in 2025[8]

Autosomal Dominant Polycystic Kidney Disease

- A chronic kidney disease affecting 1 in every 1,000 people[9] that leads to renal failure and the need for organ transplantation in the majority of patients
- Clinical trials are expected to commence in early 2025 with human safety and efficacy data anticipated in 2025 and 2026[10]

Phelan McDermid Syndrome

- A severe neurodevelopmental disorder affecting 1 in every 10,000 people[11]
- PYC will initiate Investigational New Drug (IND)-enabling studies in 2025 to facilitate progression into human trials

For more information, visit pyctx.com, or follow us on LinkedIn and Twitter.

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.

[5] 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

[6] Subject to the risks outlined in the Company's ASX announcement of 14 March 2024

[7] Yu-Wai-Man, P. et al. The Prevalence and Natural History of Dominant Optic Atrophy Due to OPA1 Mutations Ophthalmology. 2010;117(8):1538-46 doi: 10.1016/j.ophtha.2009.12.038

[8] Subject to the risks outlined in the Company's ASX announcement of 14 March 2024

[9] Harris PC, Torres VE. Polycystic Kidney Disease, Autosomal Dominant. 2002 Jan 10 [Updated 2022 Sep 29]. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. GeneReviews. Seattle (WA): University of Washington, Seattle; 1993-2023.

[10] Subject to the risks outlined in the Company's ASX announcement of 14 March 2024

[11] Phelan-McDermid Syndrome Foundation. https://pmsf.org/about-pms/

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.

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

CONTACTS:

INVESTORS and MEDIA
investor@pyctx.com