PYC Therapeutics

30 August 2024

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 PYC-001 drug development program**
- **PYC is now eligible to receive a Priority Review Voucher (PRV) upon approval of PYC-001 for vision loss associated with mutations in the *OPA1* gene**

PERTH, Australia and SAN FRANCISCO, California – 30 August 2024

PYC Therapeutics (ASX:PYC) is a clinical-stage biotechnology company creating 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 for patients with a blinding eye disease called Autosomal Dominant Optic Atrophy (ADOA). This drug candidate is known as PYC-001.

The Company today announces that PYC-001 has been granted Rare Pediatric Disease (RPD) designation by the US Food and Drug Administration (FDA) for *OPA1*-associated vision loss. The Rare Pediatric Disease program aims to incentivise drug development for serious and rare diseases affecting children[1].

A sponsor who receives an approval for a drug with this designation may qualify for a Priority Review Voucher (PRV) that can be redeemed to receive priority review for a different product or sold to another sponsor.

PYC recently announced that it had received regulatory approval to commence human trials of PYC-001 in individuals with ADOA (See ASX announcement of 15 August 2024) and expects to commence dosing patients in a Phase 1 Single Ascending Dose (SAD) study imminently[2].

About PYC-001 – a first-in-class drug candidate with disease-modifying potential in ADOA

ADOA is a blinding eye disease that begins in childhood and ultimately leads to legal blindness in middle age in most patients. The disease affects ~1 in every 35,000 people and is caused by insufficient expression of the *OPA1* gene in the retina.

[1] US FDA. Rare Pediatric Disease Designation and Priority Review Voucher Programs – Information for product sponsors
[2] Subject to the risks and uncertainties set out in the Company's ASX disclosures of 14 March 2024

There are currently no treatment options available for patients with ADOA which represents an estimated >$2 billion p.a. addressable market[3].

PYC-001 is a precision therapy that aims to restore the expression of the *OPA1* gene back to levels required for the normal function of the retina. PYC-001 utilises PYC's proprietary drug delivery technology to overcome the major challenge for RNA drugs by ensuring that sufficient drug reaches its target inside the cells affected by ADOA. PYC-001 is effective at addressing the underlying cause of ADOA in both patient-derived 'retina in a dish' and Non-Human Primate models[4].

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**[5].

PYC's drug development programs

Retinitis Pigmentosa type 11

- A blinding eye disease of childhood affecting 1 in every 100,000 people[6]
- Currently progressing through clinical trials with human safety and efficacy read-outs anticipated in 2024[7]

Autosomal Dominant Optic Atrophy

- A blinding eye disease of childhood affecting 1 in every 35,000 people[8]
- Now entering clinical trials with human safety and efficacy read-outs anticipated in 2024 and 2025[9]

Autosomal Dominant Polycystic Kidney Disease

- A chronic kidney disease affecting 1 in every 1,000 people[10] 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[11]

Phelan McDermid Syndrome

[3] Estimated market in Australian dollars based on a target patient population of 7,500 in the Western World and median orphan drug pricing of US$150,000 per patient per annum
[4] Refer ASX announcement 4 October 2023
[5] Advancing Human Genetics Research and Drug Discovery through Exome Sequencing of the UK Biobank https://doi.org/10.1101/2020.11.02.20222232
[6] 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
[7] Subject to the risks outlined in the Company's ASX announcement of 14 March 2024
[8] 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
[9] Subject to the risks outlined in the Company's ASX announcement of 14 March 2024
[10] 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.
[11] Subject to the risks outlined in the Company's ASX announcement of 14 March 2024

- A severe neurodevelopmental disorder affecting 1 in every 10,000 people[12]
- 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.

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 CEO of PYC Therapeutics Limited

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