PYC Therapeutics

24 May 2024

US FDA GRANTS ORPHAN DRUG DESIGNATION TO PYC DRUG CANDIDATE

- **PYC is a clinical-stage biotechnology company developing a pipeline of first-in-class precision medicines for patients who have genetic diseases and no treatment options available today**
- **The Company is set to commence clinical trials of an investigational drug candidate it has designed to address the root cause of a blinding eye disease called Autosomal Dominant Optic Atrophy (ADOA) in 3Q 2024**
- **PYC has today received Orphan Drug Designation (ODD) from the US Food and Drug Administration (FDA) providing several benefits for PYC's ADOA program as it progresses through clinical trials and regulatory approval[1]**

PERTH, Australia and SAN FRANCISCO, California – 24 May 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 set to commence a phase 1 clinical trial for patients with a blinding eye disease called Autosomal Dominant Optic Atrophy (ADOA) later this year[2].

PYC today announces the receipt of Orphan Drug Designation (ODD) from the US Food and Drug Administration (FDA) for this drug candidate (known as PYC-001) for the treatment of OPA1-associated vision loss. 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[3].

About ADOA

Autosomal Dominant Optic Atrophy (ADOA) is a progressive and irreversible blinding eye disease. ADOA affects approximately 1 in every 35,000 people representing a market size of ~$2 billion per annum[4].

[1] Subject to the risks set out in the Company's ASX filing of 14 March 2024
[2] Subject to successful regulatory engagement
[3] See FDA guidance entitled 'Medical products for rare diseases and conditions'
[4] Based on median price of orphan drugs of US$150,000 p.a. from EvaluatePharma. Orphan Drug Report. 2019

ADOA is caused by a mutation in one copy of the *OPA1* gene and in ~85% of patients, this mutation leads to insufficient levels of *OPA1* gene expression to support normal cellular function in the retinal ganglion cells of the eye. The abnormal function of the affected cells due to the *OPA1* deficiency causes cell stress and ultimately cell death. Loss of retinal ganglion cells due to cell death interrupts the normal processing of the visual signal from the retina to the brain leading to the loss of vision in ADOA patients.

PYC's investigational drug candidate for ADOA (known as 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.

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].

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 is progressing a second drug program targeting a blinding eye disease (Autosomal Dominant Optic Atrophy) and a third program targeting Polycystic Kidney Disease which are anticipated to commence human trials in mid-2024 and early 2025 respectively.

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

[5] Advancing Human Genetics Research and Drug Discovery through Exome Sequencing of the UK Biobank https://doi.org/10.1101/2020.11.02.20222232

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