PYC Therapeutics

6 November 2023

PYC AND FDA ALIGN ON CLINICAL TRIAL PATHWAY IN ADOA

- 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
- PYC's second drug candidate addresses the root cause of a blinding eye disease of childhood called Autosomal Dominant Optic Atrophy (ADOA)
- ADOA affects 1 in every 35,000 people and represents an estimated A$2 billion p.a.[1] market with no treatment options available for patients today
- PYC announces that it has aligned with the US Food and Drug Administration (FDA) on the proposed path through clinical trials for its investigational drug candidate for ADOA through a pre-Investigational New Drug (pre-IND) meeting
- The Company expects to progress this drug candidate to human trials in 1H 2024[2]

PERTH, Australia and SAN FRANCISCO, California – 6 November 2023

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. PYC's second drug program is an investigational drug candidate that addresses the root cause of a blinding eye disease of childhood called Autosomal Dominant Optic Atrophy (ADOA). PYC today announces that it has held a pre-IND meeting with the US FDA and agreed on a proposed pathway through clinical trials for this drug candidate.

PYC anticipates progression of this drug candidate to human trials in 1H 2024[3]. Successful progression of this drug candidate into human studies will mark the second first-in-class drug program that PYC has advanced into the clinic and the Company remains on track to realise its objective of progressing a third drug with disease-modifying potential into human studies before the end of 2024.

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] 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
[2] Subject to successful completion of Good Laboratory Toxicity studies and receiving regulatory endorsement
[3] Subject to successful completion of Good Laboratory Toxicity studies and receiving regulatory endorsement
[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 into human trials and is now progressing multiple 'fast-follower' programs into the clinic. 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

CONTACTS:

INVESTORS and MEDIA
info@pyctx.com