## Q1 Investor Update



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### Discussion topics - Q1 investor update



- An introduction to PYC
- An update on the global life sciences environment
- The RNA therapeutics backdrop within the industry
- Pipeline review
  - PYC's progress through Q4 2022 and YTD 2023
  - Forward view for PYC in 2023
- Q&A



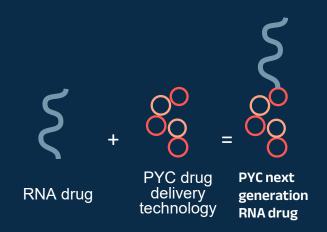
Introduction to PYC



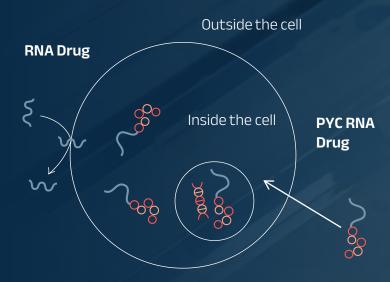
## PYC's RNA delivery platform overcomes the primary challenge for precision therapies – ensuring enough drug reaches its target



PYC combines existing RNA drug design technology with its proprietary drug delivery platform to create next generation RNA therapeutics



PYC's drug delivery platform is used to assist the RNA drug reach its target inside the cell



## PYC is creating therapies for patients through a strategy anchored on four critical features





## A HIGHER PROBABILITY OF SUCCESS

PYC focuses on monogenic indications. These have the highest likelihood of approval from the start of clinical trials to market of any indication\*1



#### A FASTER PATH TO MARKET

The potential for approval following two clinical trials (not three) due to the absence of existing treatment options for patients with the targeted indications



## LIKELY RAPID UPTAKE IN MARKET

First-in-class drugs in rare diseases achieve rapid market penetration with a very short lead time to peak sales



## ORPHAN DRUG PRICING

Median list price of ~US\$150,000² per patient per annum making for commercially attractive markets across the pipeline

<sup>\*</sup>Monogenic indications compared to polygenic indications

<sup>1.</sup> Advancing Human Genetics Research and Drug Discovery through Exome Sequencing of the UK Biobank. doi: https://doi.org/10.1101/2020.11.02.20222232

## The key to understanding PYC is the Company's focus on genetic validation of the targets it has selected





## A HIGHER PROBABILITY OF SUCCESS

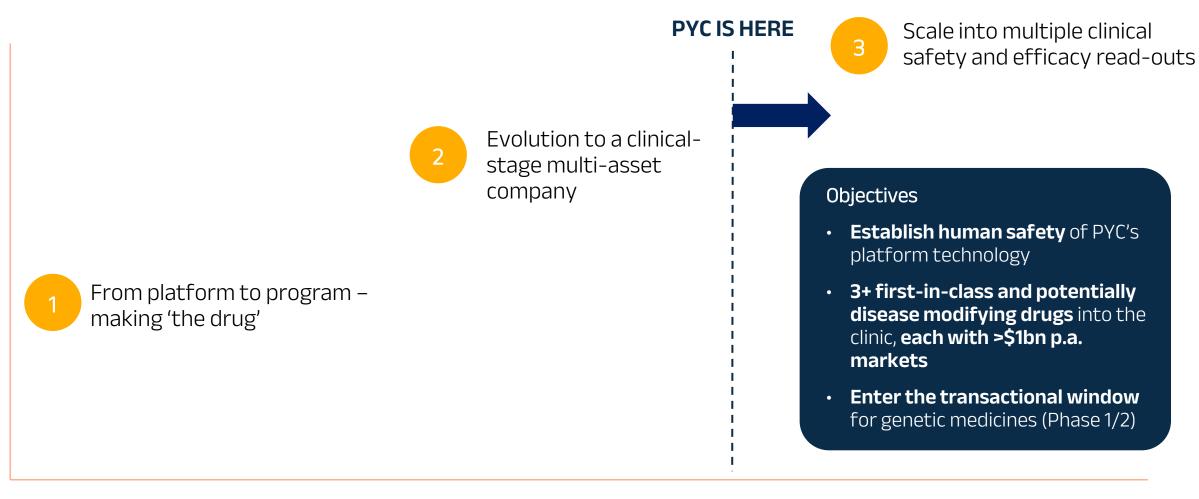
PYC focuses on monogenic indications. These have the highest likelihood of approval from the start of clinical trials to market of any indication\*1

"When you're following Nature's lamp posts... Much higher probability of success"

Atlas Ventures 2022 year in review

## PYC is looking to progress 3 first-in-class and disease-modifying drugs into the clinic within 24 months







Industry trends





## **Right Place**

"Many examples here from monogenic diseases, where we know this gene is the gene, and we know this is the problem... And now we know exactly what we can do. So that's a very different paradigm" <sup>1</sup>

## **Right Time**

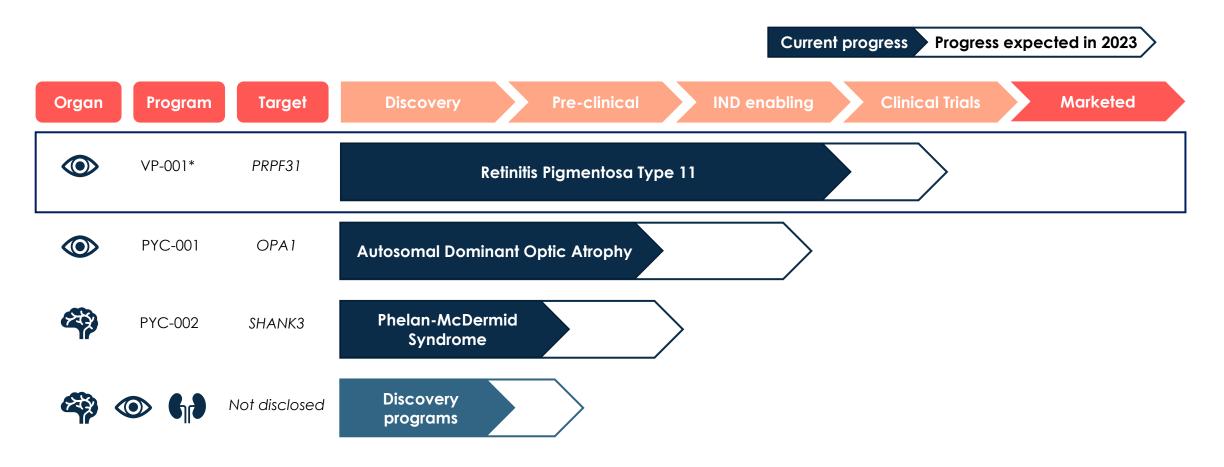
Novartis is focused on potential acquisitions that could fetch biotech companies valued at \$5 billion or lower with drugs in early- to mid-stage development, said CEO Vas Narasimhan. "If we can make acquisitions there, then we can participate in the upside" of the drugs, he said <sup>2</sup>



# Program deep-dives (progress and anticipated milestones)



### RP11 - progress and expected milestones



## VP-001 is set to become the first investigational drug with disease-modifying potential for patients with RP11



5,000 - 10,000 patients

Estimated addressable RP11 patient population in the western world<sup>1</sup>

USD \$150,000 p.a.

Median list price of orphan drugs (per patient<sup>2</sup>)

### **Patients are waiting**

RP11 patients on retinal disease registries are waiting for access to VP-001 – suggesting a rapid uptake in the event the drug is approved

#### **FDA** concessions

Potential to receive multiple FDA concessions:

- Orphan Drug Designation
- Rare Pediatric Disease Designation\*
- Accelerated approval<sup>3</sup>

<sup>1.</sup> Sullivan, L et al. Genomic rearrangements of the PRPF31 gene account for 3% of autosomal dominant retinitis pigmentosa. Invest Ophthalmol Vis Sci. 2006;47(10):4579-88

<sup>2.</sup> EvaluatePharma. Orphan Drug Report. 2019

<sup>3.</sup> FDA. Development and Approval Process | Drugs. 2022

### PYC is set to generate human safety and efficacy data for **VP-001**<sup>1</sup>



#### **Phase 1/2 Human Trial**

#### Part 1 Part 2

- Single Ascending Dose Study
- 3+3+3 dose escalation in RP11 patients
- Approx 1 year study

#### **Outcome measures**

Safety and **Tolerability** 

- Multiple dose expansion study
- 3 groups of 10 patients each
- Two active groups plus control group
- Approx 2 year study

#### **Outcome measures**

- Safety and **Tolerability**
- Efficacy readout

Trial design will be informed by:

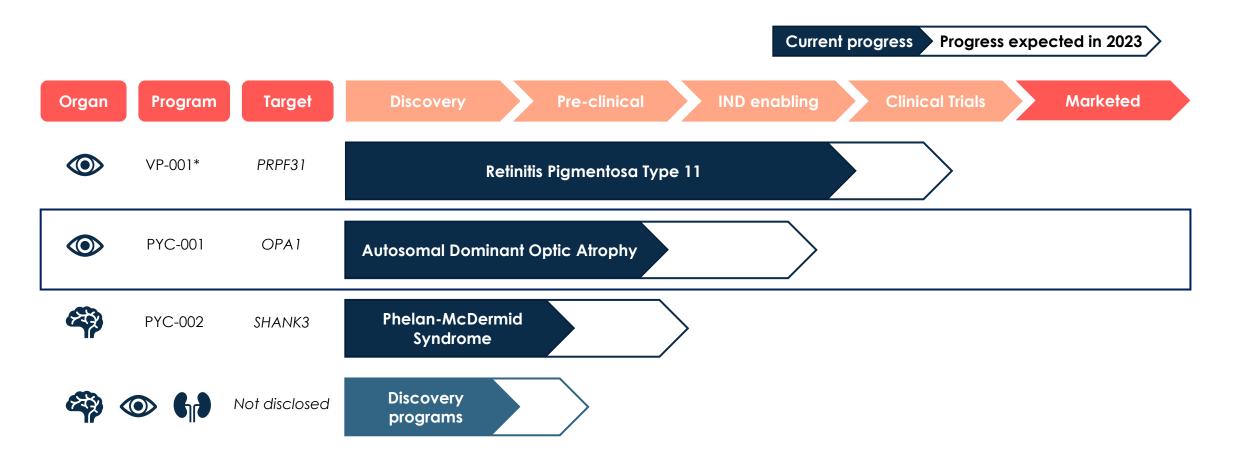
**Pivotal Trial** 

- Observations made in Phase 1/2 study; and
- Insights generated in the **Natural History Study**

Natural History Study running concurrently



### ADOA - progress and expected milestones



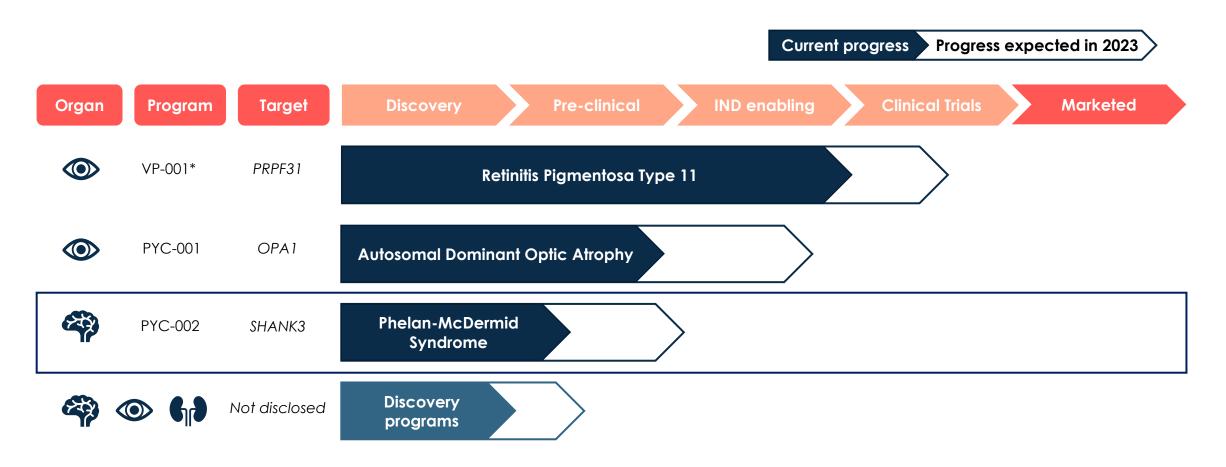
## Results from the testing of PYC-001 in 'mini-eyes' are anticipated in Q2



"The explosive developing field of induced pluripotent stem cells (iPSCs) reprogrammed from primary patient-derived cell cultures provides the remarkable and unique opportunity to obtain specialized terminally differentiated cells and organoids to study 'disease in a dish' models, including mini-eyes and mini-brains"

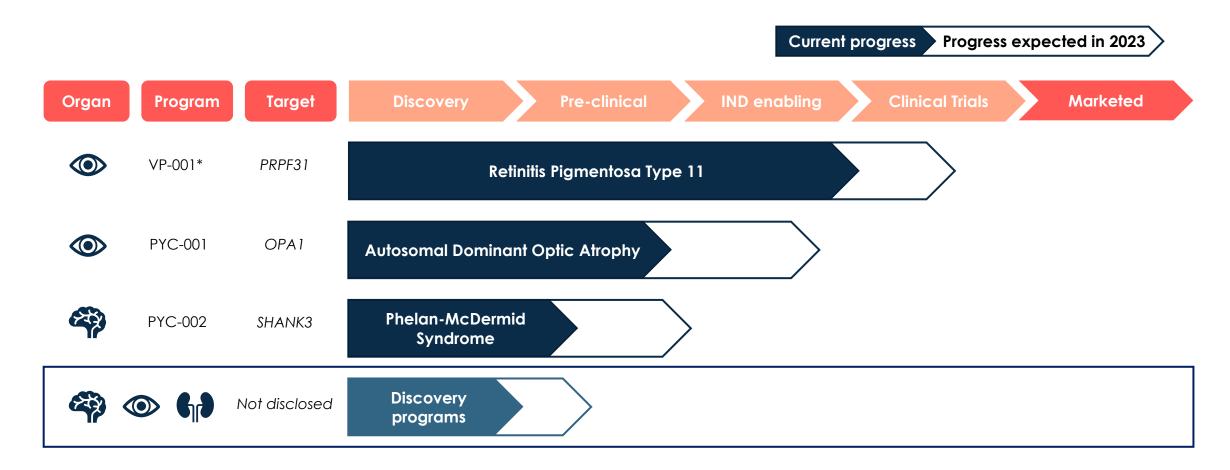


### PMS - progress and expected milestones





### Platform/discovery - progress and expected milestones



## PYC's technology is rapidly scalable – supporting the Company's ambition of progressing a new drug into the clinic every year



"Once the nucleic acid chemistry and the delivery method are established, the production of RNA-based drugs for a new target can be achieved in a relatively short period using these pre-established methodologies"



Q&A