



**OTCQB: CYTR** 

# **Corporate Overview June 2019**

**Non-Confidential** 

## **CytRx Safe Harbor Statement**

THIS PRESENTATION CONTAINS FORWARD-LOOKING STATEMENTS THAT INVOLVE CERTAIN RISKS AND UNCERTAINTIES. ACTUAL RESULTS COULD DIFFER MATERIALLY FROM THOSE EXPRESSED OR IMPLIED IN THESE FORWARD-LOOKING STATEMENTS AS A RESULT OF VARIOUS RISKS AND UNCERTAINTIES, INCLUDING THOSE RISK FACTORS DISCUSSED IN THE ANNUAL AND QUARTERLY REPORTS THAT CYTRX FILES WITH THE U.S. SECURITIES AND EXCHANGE COMMISSION.



## **CytRx Highlights**

- CytRx's milestone and royalty agreement with Orphazyme for arimoclomol represents potentially significant near term value
- Orphazyme confirms its intention to file arimoclomol for European approval for Niemann-Pick disease Type C (NPC) in the first half of 2020 and Orphazyme will meet with the FDA in the Summer of 2019 on arimoclomol and provide an update once it receives written comments
- NantCell provided an update to its studies of aldoxorubicin in phase 1b/2 studies in combination with immunotherapy in pancreatic cancer, head and neck cancer, triple negative breast cancer and colorectal cancer at the Society for Immunotherapy of Cancer's (SITC) 33rd Annual Meeting
- Centurion BioPharma is a private subsidiary oncology company focused on oncology treatment and has completed the pre-clinical phase for its ultra high potency LADR™ drug candidates and accompanying companion diagnostic (ACDx)



# CytRx can build value with potential milestone/royalty payments along with our Centurion BioPharma pipeline

Orphazyme
Milestones
and Royalties

Orphazyme ("ORPHA.CO"): \$120M in potential milestones + royalties on Arimoclomol (currently undergoing clinical trials in 4 indications)

NantCell Milestones and Royalties

NantCell: \$343M in potential milestones + royalties on Aldoxorubicin

Centurion BioPharma Pipeline

Oncology personalized medicine: companion diagnostic + treatment

**Centurion BioPharma is a subsidiary of CytRx** 

# CytRx value – Orphazyme milestones and royalties

Orphazyme Milestones and Royalties

Orphazyme: up to \$120M in milestones + royalties on Arimoclomol

- After receiving constructive feedback from the European Medicines Agency (EMA)'s Scientific Advice Working Group, Orphazyme confirms its intention to file for European approval for Niemann-Pick disease Type C (NPC) in the first half of 2020
- Orphazyme plans to meet with the US Food and Drug Administration (FDA) to discuss the pathway forward for arimoclomol in NPC during the Summer and will provide an update after it has received written comments from the FDA
- Orphazyme has ongoing clinical trials in Amyotrophic Lateral Sclerosis (ALS),
   Sporadic Inclusion Body Myositis (sIBM) and Gaucher Disease



## **Orphazyme 2019 Objectives**

2019 Objectives | Orphazyme Annual Report 2018

# 2019 OBJECTIVES

Priority	Targeted milestone     Complete enrollment in H2		
ALS			
SIBM	Complete enrollment in H1		
NPC	Regulatory feedback mid-2019		
Gaucher disease	Phase II results in H2		
New molecular entitles (NME) program	Preclinical studies with NMEs in protein-misfolding diseases		

#### Update:

JUNE 7, 2019

ORPHAZYME TO PREPARE FOR FILING OF ARIMOCLOMOL IN EUROPE FOR NIEMANN-PICK DISEASE TYPE C (NPC)

· Expected submission of Marketing Authorization Application (MAA) in H1 2020





### WHAT IS NPC? Niemann-Pick Disease Type C

#### NPC IS A RARE, INHERITED, PROGRESSIVE, AND OFTEN FATAL NEURODEGENERATIVE DISEASE

NPC is a lysosomal storage disorder caused by genetic mutations that often lead to misfolded variants of NPC proteins. Misfolded NPC protein does not function properly and is subject to rapid degradation.



1-2000

people are diagnosed with NPC in the USA and EU





#### MANIFESTATIONS

The disease affects the brain, liver, succumb to the disease before reaching the end of their teens.

The disease is progressive and patients gradually loses:

Motor function and coordination

Speech

Cognition

Memory



95% have mutations in the NPC1 gene



is currently approved to treat NPC (Zavesca).



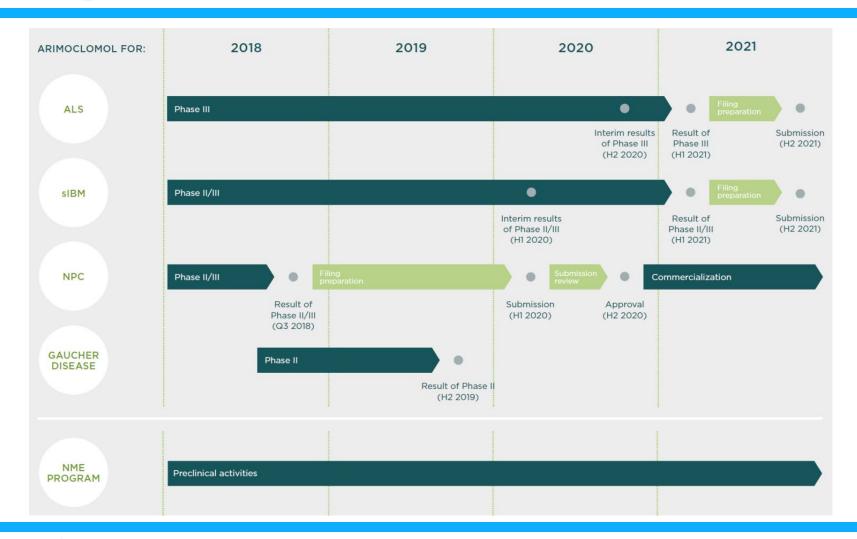
#### DIAGNOSIS

Difficult to diagnose, NPC is often diagnosed by ruling out other diseases, which may take years.

There is **NO CURE** for NPC



# Orphazyme development programs for arimoclomol





# CytRx value - NantCell milestones and royalties

NantCell Milestones and Royalties

NantCell: up to \$343M in milestones + royalties on Aldoxorubicin

- NantCell was founded in 2015 by Dr. Patrick Soon-Shiong and licensed aldoxorubicin from CytRx in July 2017
- Dr. Soon-Shiong is an oncology research pioneer with albumin based drugs and the inventor of a successful cancer drug called Abraxane
- NantCell is studying aldoxorubicin in phase 1b/2 studies in combination with immunotherapy in pancreatic cancer, head and neck cancer, triple negative breast cancer and colorectal cancer
- Early safety and efficacy data from a portion of the studies was presented at Society for Immunotherapy of Cancer's (SITC) 33rd Annual Meeting
- CytRx is entitled to increasing double-digit royalties on aldoxorubicin for soft tissue sarcomas and increasing single-digit royalties for all other indications



# CytRx partnered Pipeline with NantCell - Aldoxorubicin

Aldoxorubicin	Preclinical	Phase 1	Phase 2	Phase 3	
2 <sup>nd</sup> -Line Soft Tissue Sarcoma	Ph 3 – Completed; NantCell has IND				
2 <sup>nd</sup> -Line Small Cell Lung Cancer	Ph 2 – Fully enro	olled; NantCell has INE		,	
Combo with ifosfamide – STS	Ph 1b/2 – Nanto	Cell has IND	,		
Combination Trials with Immunotherapy					
Pancreatic Cancer	Ph 1b/2 – On-g	oing			
Squamous Cell Carcinoma	Ph 1b/2 – On-g	oing			
Triple-Negative Breast Cancer	Ph 1b/2 – On go	ping			
Colorectal Cancer	Ph 1b/2 – On go	ping			



### Update from NantCell at the Society for Immunotherapy of Cancer's (SITC) 33rd Annual Meeting

### <u>Preliminary Phase 1b Results in TNBC (triple negative breast cancer) and HNSCC (head and neck squamous cell carcinoma)</u>

- In this Phase 1b, single-arm, open-label trial, treatment was administered in 3-week cycles of low-dose chemotherapy (aldoxorubicin, cyclophosphamide, cisplatin, nab-paclitaxel, 5-FU/L), antiangiogenic therapy (bevacizumab), engineered allogeneic high affinity CD-16 NK-92 cells (haNK), IL-15RaFc (N803), adenoviral vector-based CEA, MUC1, Brachyury, HER2 vaccine, yeast vector-based RAS, Brachyury and CEA vaccine, and an IgG1 PD-L1 inhibitor, avelumab plus cetuximab. All patients in both trials received aldoxorubicin. The primary endpoint is incidence of treatment-related adverse events (AEs). Secondary endpoints include overall response rate (ORR), disease control rate (DCR), progression-free survival (PFS), and overall survival (OS).
- The abstract includes data from three patients with third-line or greater TNBC and two patients with fourth-line or greater HNSCC. All treatment was completed in the outpatient setting, with no immune-related AEs. Four hematologic dose limiting toxicities (DLTs) were observed and managed with a planned dose reduction of cisplatin. Of the three patients with TNBC, two (67%) experienced a partial response (PR). Of the two patients with HNSCC, both (100%) experienced objective tumor response (100% and 47% decrease, respectively). Overall, four out of the five TNBC and HNSCC patients (80%) had confirmed overall responses, including one patient (20%) with fifth-line metastatic disease who demonstrated a complete response (CR). All responding patients are still undergoing therapy. These preliminary data suggest that the NANT Cancer Vaccine (NCV), comprised of low-dose chemo-radiation combined with innate and adaptive immunotherapy, can be administered safely in an outpatient setting without any observed increase in immune-related AEs.



### Update from NantCell at the Society for Immunotherapy of Cancer's (SITC) 33rd Annual Meeting

#### **Preliminary Phase 1b Results in Metastatic Pancreatic Cancer**

- In this Phase 1b, single-arm, open-label trial, treatment was administered in 3-week cycles of low-dose chemotherapy (aldoxorubicin, cyclophosphamide, oxaliplatin, nab-paclitaxel, 5-FU/L), antiangiogenic therapy (bevacizumab), engineered allogeneic high affinity CD-16 NK-92 cells (haNK), IL-15RaFc (N-803), adenoviral vector-based CEA vaccine, yeast vector-based RAS vaccine, and an IgG1 PDL1 inhibitor, avelumab. All metastatic pancreatic cancer patients received aldoxorubicin. The primary endpoint is incidence of treatment-related AEs. Secondary endpoints include ORR, DCR, PFS, and OS.
- The abstract includes data from ten patients with third-line or greater metastatic pancreatic cancer. All treatment was safely administered in the outpatient setting. AEs were primarily hematologic which were managed by appropriate planned chemo dose reductions. No DLTs have occurred and no haNK-related AEs have occurred to date. Of the ten evaluable patients, nine have achieved stable disease (SD) for ≥ 8 weeks for a DCR of 90%. Median PFS was 5.8 months (95% confidence interval: 3.3 8.8) and OS was 9.5 months (95% CI: 5.0 upper limit not yet reached) with patients continuing treatment. One patient demonstrated resolution of a metastatic lung tumor within 8 weeks of initiating NCV therapy. These preliminary results suggest that the NCV treatment regimen was well tolerated and support the safety and tolerability of the regimen. These preliminary efficacy results are encouraging and the overall survival of 9.5 months currently exceeds all standards of care for patients at this advanced stage of disease.



# CytRx value – Centurion BioPharma pipeline

Centurion BioPharma Pipeline

Oncology personalized medicine: companion diagnostic + treatment

## LADR™ (linker activated drug release) <u>albumin</u> binding drug conjugates

LADR-7

LADR-8

LADR-9

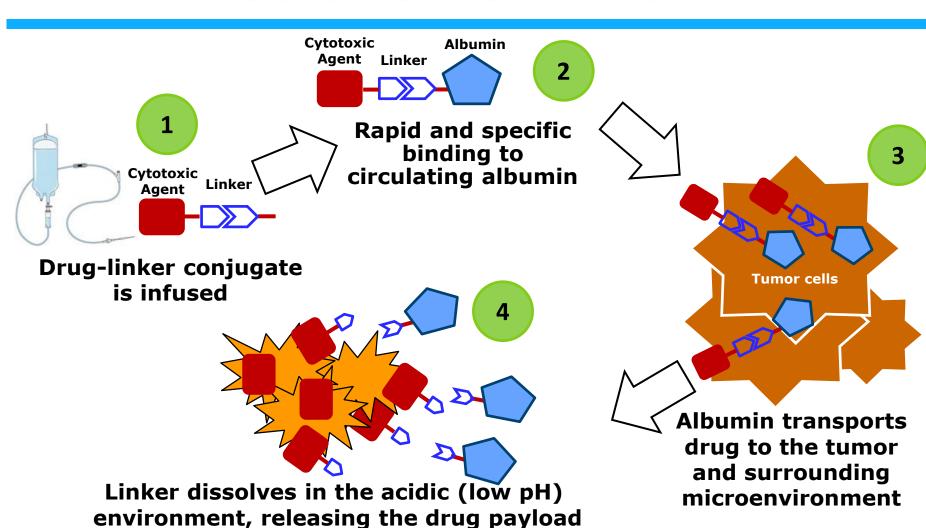
LADR-10

#### **Albumin** companion diagnostic (ACDx)

identifies tumors eligible for treatment with LADR™



### **LADR™** Mechanism of Action





# **Recent and Upcoming Catalysts**

#### 2019-2020

- √2019: Reduce cash burn rate to ~\$450,000 per month
- 2H 2020: Orphazyme gains EMEA (Europe) approval for arimoclomol in Niemann-Pick Type C disease
- 2H 2020: Orphazyme gains FDA (US) approval for arimoclomol in Niemann-Pick Type C disease
- 2H 2020: Orphazyme announces interim results of phase 3 clinical trial of arimoclomol in amyotrophic lateral sclerosis (ALS) and interim results of arimoclomol in phase 2/3 study of sporadic inclusion body myositis (sIBM)



## **Financial Summary**

Cash Position (3/31/2019)

\$20.1M

No Debt

Shares Outstanding

33.6M

• **Options** Weighted-average strike price: \$10.73

2.4M

Warrants

Weighted-average strike price: \$8.60

0.2M

Fully-Diluted Share Count (03/31/2019)

36.2M



## **Conclusion: CytRx Value**

- Orphazyme may deliver \$120M in milestones + royalties
- NantCell may deliver \$343M in milestones + royalties
- Centurion BioPharma is a private subsidiary of CytRx
- Potential to selectively leverage our cash reserve for opportunities
- Reduction in cash burn rate to ~\$450k per month