



CREATING TOMORROW, TODAY.

**OTCQB: CYTR**

# **Corporate Overview**

## **December 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 potential near term payments to CytRx
- Orphazyme has positive meetings with the FDA and EMEA authorities and plans to file arimoclomol for approval
- NantCell/ImmunityBio\* 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 oncology company focused on oncology treatment and has completed the pre-clinical phase for its ultra high potency **LADR™** drug candidates and accompanying albumin companion diagnostic (ACDx)

# CytRx has potential milestone/royalty payments and a subsidiary called Centurion BioPharma

## Orphazyme Milestones and Royalties

Orphazyme: \$120M in potential milestones + royalties on arimoclomol

## NantCell / ImmunityBio Milestones and Royalties

NantCell/ImmunityBio: \$343M in potential milestones + royalties on aldoxorubicin

## Centurion BioPharma Pipeline

Oncology personalized medicine: companion diagnostic + treatment

Centurion BioPharma is a subsidiary of CytRx

# CytRx may receive milestones and royalties from Orphazyme for arimoclomol

## Orphazyme Milestones and Royalties

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

- Orphazyme has had a positive meeting with the FDA and remains on track to submit an NDA for arimoclomol for Niemann-Pick disease Type C (NPC) in the first half of 2020
- Orphazyme plans to introduce an Early Access Program for NPC in the fall of 2019, to further accelerate access to treatment with arimoclomol for people living with NPC
- 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 NPC in the first half of 2020
- Orphazyme has ongoing clinical trials in Amyotrophic Lateral Sclerosis (ALS), Sporadic Inclusion Body Myositis (sIBM) and Gaucher Disease

# Orphazyme 2019 Objectives

## 2019 arimoclomol objectives

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

### Updates:

- Orphazyme has completed enrollment early in ALS phase III clinical trial; top line results expected in H1 2021.
- Phase II/III trial for the treatment of sporadic Inclusion Body Myositis (sIBM) is fully enrolled. Performance of interim analysis and study completion expected in H1 2020 and by end 2020, respectively. Results are expected in H1 2021.
- NPC updates as explained on previous slide.

## 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, spleen and lungs. Often patients 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



**20 years**  
is the average life expectancy

**95%** have mutations  
in the **NPC1** gene



**ONLY  
1 DRUG**

is currently approved  
to treat NPC  
(Zavesca).



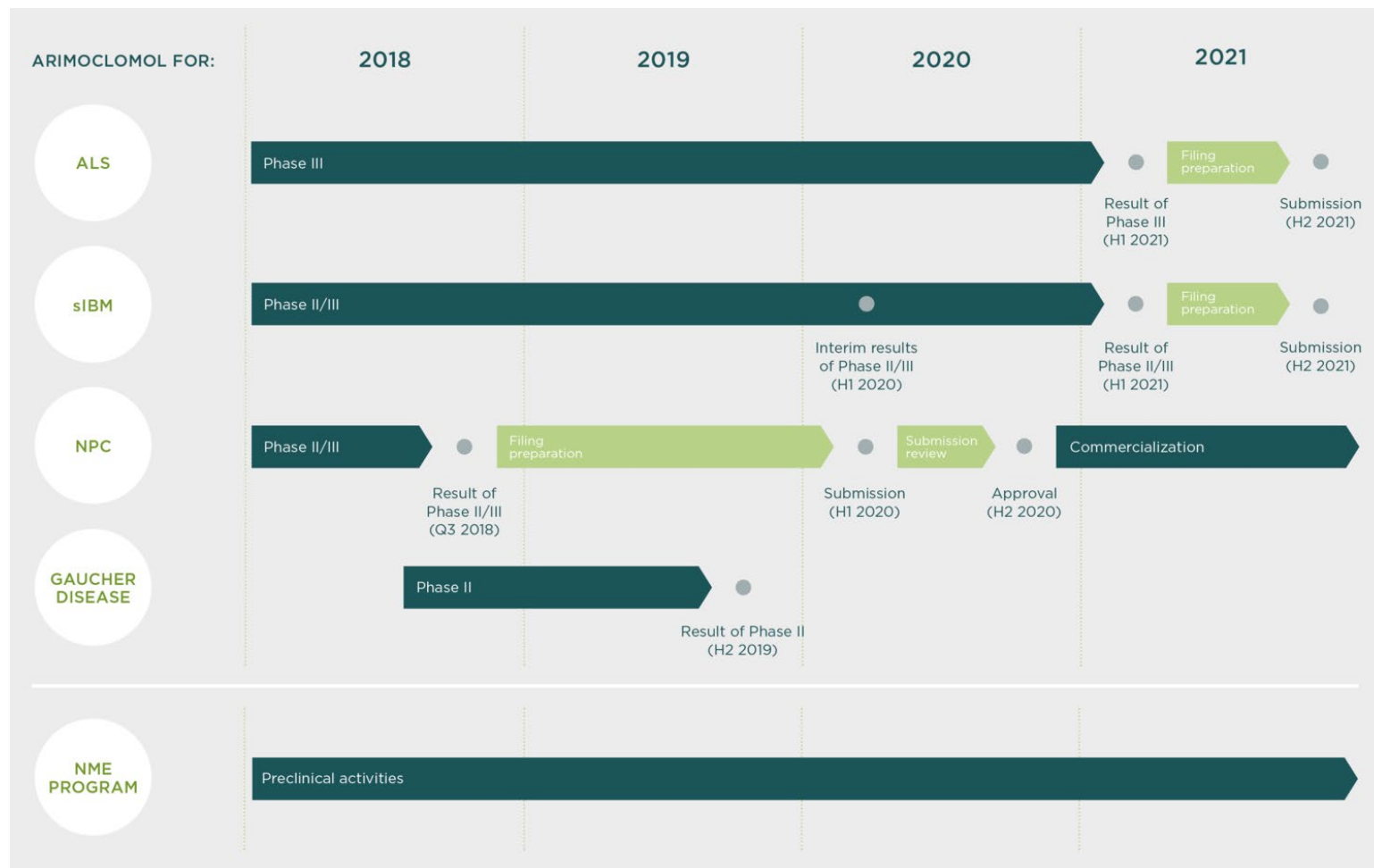
### DIAGNOSIS

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 may receive milestones and royalties from NantCell/ImmunityBio for aldoxorubicin

## NantCell / ImmunityBio Milestones and Royalties

NantCell/ImmunityBio: up to \$343M in milestones + royalties on aldoxorubicin

- NantCell is now called ImmunityBio and is a privately held company involved in late stage clinical development
- NantCell/ImmunityBio 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/ImmunityBio - 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 enrolled; NantCell has IND			
Combo with ifosfamide – STS	Ph 1b/2 – NantCell has IND			
Combination Trials with Immunotherapy				
Pancreatic Cancer	Ph 1b/2 – On-going			
Squamous Cell Carcinoma	Ph 1b/2 – On-going			
Triple-Negative Breast Cancer	Ph 1b/2 – On going			
Colorectal Cancer	Ph 1b/2 – On going			

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

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

- 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/ImmunityBio 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-15R $\alpha$ Fc (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  $\geq 8$  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 subsidiary Centurion BioPharma has an oncology preclinical pipeline

## Centurion BioPharma Pipeline

Oncology personalized medicine: companion  
diagnostic + treatment

**LADR™** (linker activated drug release) albumin binding drug  
conjugates

LADR-7

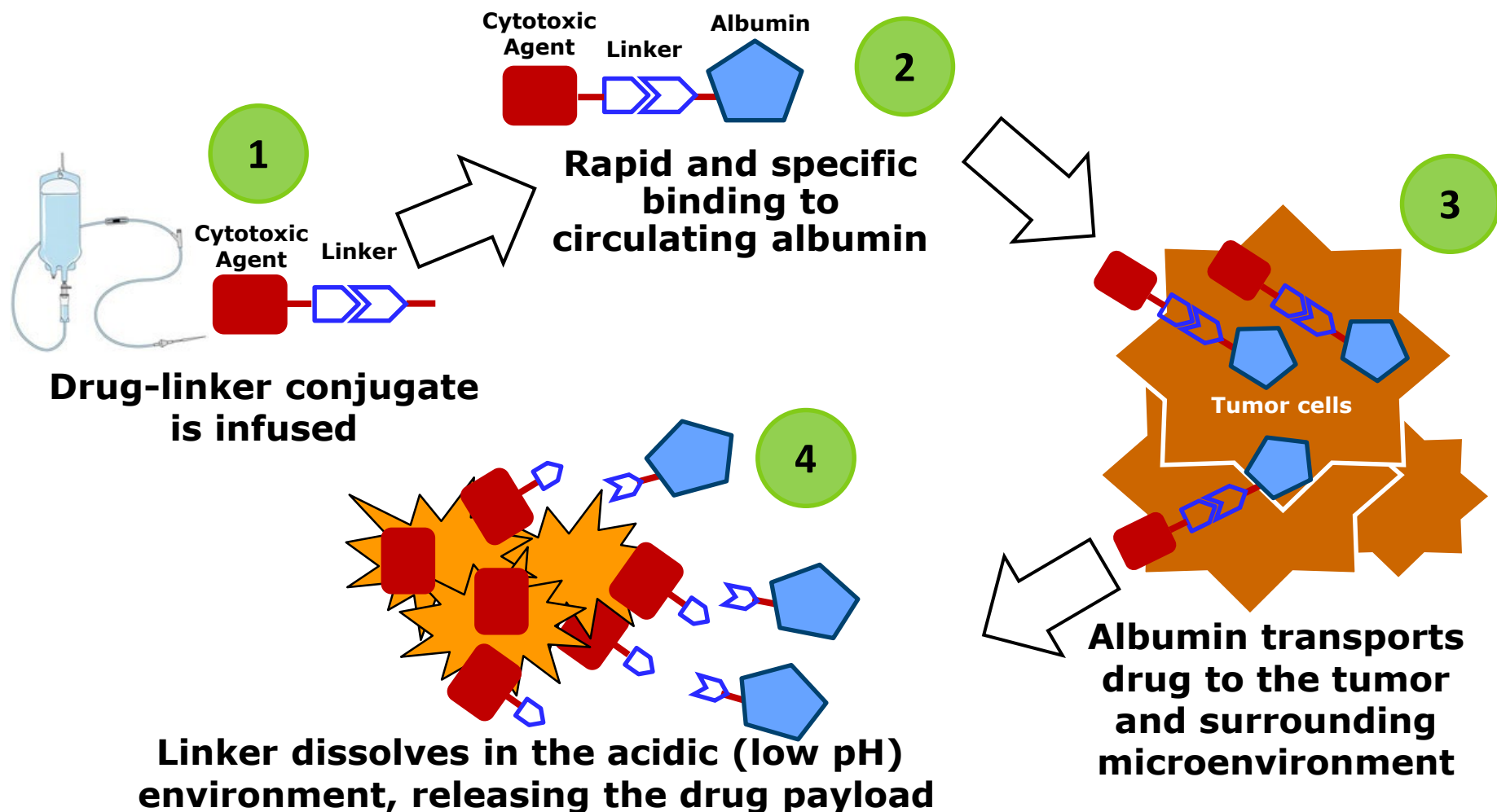
LADR-8

LADR-9

LADR-10

**Albumin companion diagnostic (ACDx)**  
identifies tumors eligible for treatment with **LADR™**

# LADR™ Mechanism of Action



# Recent and Upcoming Catalysts

## 2019-2021

- ✓ **2019:** Reduce cash burn rate to ~\$385,000 per month
- **1H 2020:** Orphazyme to announce phase 2/3 trial interim analysis performance in sporadic inclusion body myositis (sIBM)
- **2H 2020:** Orphazyme could gain FDA (US) and EMEA (Europe) approval for arimoclomol in Niemann-Pick Type C disease
- **2020-2021:** Upon approval, CytRx is to receive a \$10 million milestone payment if both the US and Europe are approved (\$6 million for US and \$4 million for Europe)
- **1H 2021:** Orphazyme to announce top line results from the full analysis of phase 3 clinical trial of arimoclomol in amyotrophic lateral sclerosis (ALS)
- **1H 2021:** Orphazyme to announce results of sIBM phase 2/3 clinical trial

# Financial Summary

---

▪ <b>Cash Position (9/30/2019)</b>	<b>\$18.5M</b>
▪ <b>No Debt</b>	
▪ <b>Shares Outstanding</b>	<b>33.6M</b>
▪ <b>Options</b> Weighted-average strike price: \$10.69	<b>2.4M</b>
▪ <b>Warrants</b>	
▪ Weighted-average strike price: \$8.60	<b>0.2M</b>
▪ <b>Fully-Diluted Share Count (09/30/2019)</b>	<b>36.2M</b>



# Summary

---

- Orphazyme could deliver milestones + royalties
- NantCell/ImmunityBio could deliver milestones + royalties
- Reduction in cash burn rate to ~\$385k per month
- Potential to selectively leverage our cash reserve for new business opportunities