



**OTCQB: CYTR** 

# Corporate Overview 1<sup>st</sup> Quarter 2020

**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.



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## **CytRx Highlights**

- CytRx's agreement with Orphazyme for arimoclomol represents potential near-term milestone and royalty payments to CytRx
- Orphazyme preparing to file NDA for arimoclomol in NPC with the FDA and EMEA authorities in 2020
- NantCell/ImmunityBio\* provided an update to its studies of aldoxorubicin in phase 1b/2 studies in combination with immunotherapy in multiple cancer indications, including a complete response in metastatic pancreatic cancer and two in TNBC, at J.P. Morgan Healthcare Conference

# 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

### Niemann-Pick disease ("NPC")

- Orphazyme plans a regulatory filing with the FDA in H1 2020 and a regulatory filing with the EMA in H2 2020, both for arimoclomol for Niemann-Pick disease Type C (NPC).
- Orphazyme launched an Early Access Program for NPC in January 2020 to further accelerate access to treatment with arimoclomol for people living with NPC.
- Expected price range is \$300,000 \$600,000.
- Total worldwide patients numbering approximately 3,000.
- Go to market in US H1 2021 and EU/RoW H2 2021.





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

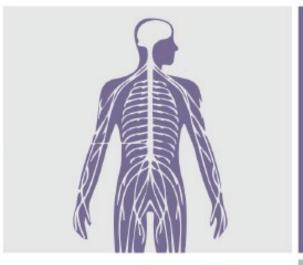
#### NPC IS A RARE, INHERITED, PROGRESSIVE, AND OFTEN FATAL NEURODEGENERATIVE DISEASE

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.





Source: www.orphazyme.com

### **Orphazyme – other indications**

### **Sporadic Inclusion Body Myositis (sIBM)**

- Phase I 24 patient pilot trial results where 83% of arimoclomol treated patients were stabilized versus 25% on placebo. 4 months of continuous treatment resulted in a 60% reduction in progression, and at 8 months, there was a 75% reduction in progression.
- Phase II/III trial is fully enrolled. Study completion expected by end of 2020 Results are expected in H1 2021 and regulatory submission in H2 2021.
- Estimated 40,000 patients in US/EU.



Source: www.orphazyme.com

### Orphazyme – other indications with Arimoclomol

### Amyotrophic Lateral Sclerosis (ALS)

- Enrollment completed in P3 trial last July 2019.
- Announcement of P3 results in ALS in H1 2021.
- Regulatory submission in H2 2021.

#### Gaucher Disease

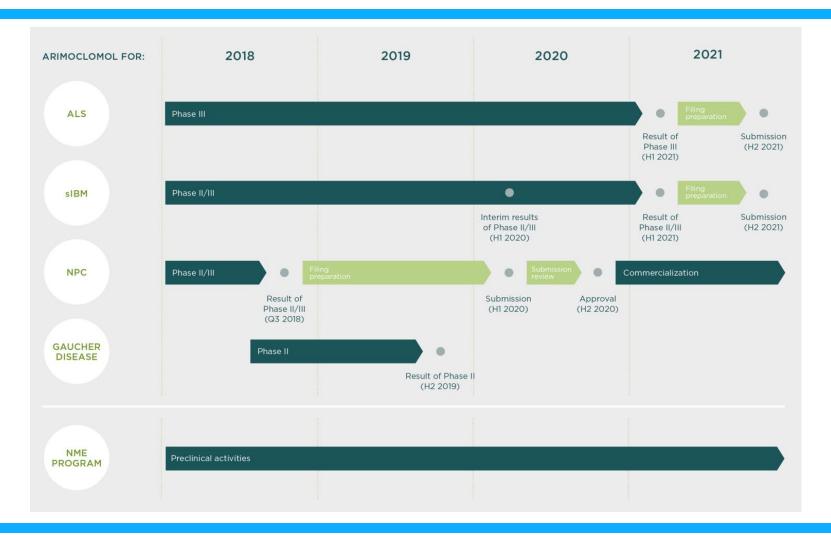
Announcement of results of P2 trial H1 2020

### Parkinson's Disease

Commenced pre-clinical work with arimoclomol



# Orphazyme development programs for arimoclomol





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Source: www.orphazyme.com

# CytRx may receive milestones and royalties from NantCell/ImmunityBio for aldoxorubicin

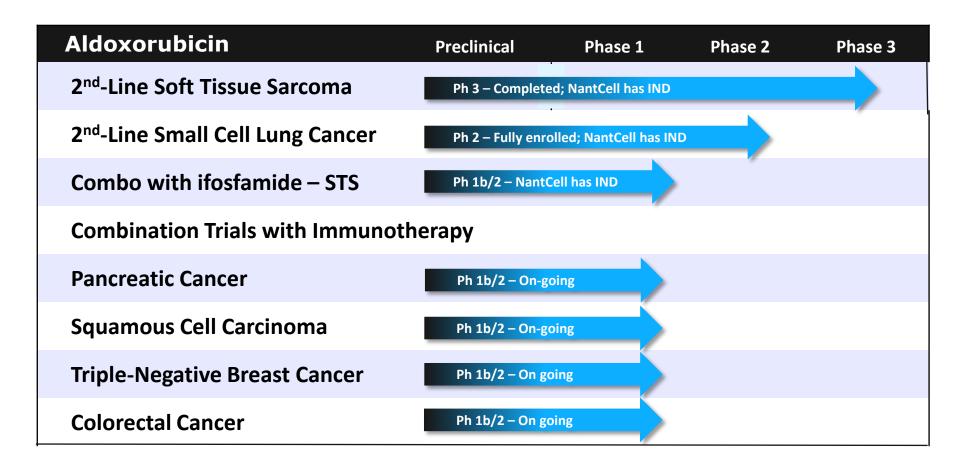
NantCell /
ImmunityBio
Milestones
and Royalties

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 (TNBC) and colorectal cancer
- Results from P1b study in TNBC presented at San Antonio Breast Cancer Symposium
- 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





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# Update from NantCell/ImmunityBio at the San Antonio Breast Cancer Symposium

### Phase 1b Results in TNBC (triple negative breast cancer)

- 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). This immunotherapy includes aldoxorubicin as part of its innovative chemoradiation therapy.
- The data highlights include of the nine patients treated efficacy results include a disease control rate of 78% (7/9 patients) and an overall response rate of 67% (6/9 patients). 2 out of 9 patients to date have ongoing complete responses with durations from 8 to 11 months, with a 3<sup>rd</sup> patient demonstrating a partial response (near complete response) in the target lesion after initiation of targeted and endocrine therapy off-study. To date, 7 patients are alive with durations of responses ranging from 2 to 12 months with 4 patients remaining on study. Median progression-free survival rate is 13.7 months. All patients were treated in an outpatient setting with treatment generally safe and well tolerated and no observed cytokine release syndrome.
- NantKwest indicated they plan to initiate a registration trial in TNBC.



# Update from NantCell/ImmunityBio at the J.P. Morgan Healthcare Conference in January 2020

### <u>Complete Response in one Patient in its Phase 1b Trial in Metastatic Pancreatic</u> Cancer

- Based on the safety and efficacy of this Phase 1b in 11 patients who had received 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, an expanded regime trial was authorized to study a patient with metastatic pancreatic cancer who had failed standard of care. After five infusions of this treatment, a complete response was confirmed. All metastatic pancreatic cancer patients received aldoxorubicin. The primary endpoint is incidence of treatment-related AEs. Secondary endpoints include ORR, DCR, PFS, and OS.
- It is expected that this patient's progress as well as report data from the full 11 metastatic pancreatic patients enrolled will be announced in 2020.
- NantKwest indicated they plan to initiate a registration trial in pancreatic cancer patients that failed standard of care.

# CytRx subsidiary Centurion BioPharma has an oncology preclinical 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™



## **Recent and Upcoming Catalysts**

### 2019-2021

- √2019: Reduce cash burn rate to ~\$385,000 per month
- 1H 2020: Orphazyme to file for FDA approval for arimoclomol in Niemann-Pick Type C disease
- 2H 2020: Orphazyme to file for 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**

Cash Position (12/31/2019)

\$16.1M

No Debt

Shares Outstanding

33.6M

• **Options** Weighted-average strike price: \$3.32

**7.7M** 

Warrants

Weighted-average strike price: \$8.60

0.2M

Fully-Diluted Share Count (12/31/2019)

41.5M



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### **Summary**

- Orphazyme could deliver milestones + royalties
- 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

