



**OTCQB: CYTR** 

GCFF Healthcare Investment Conference / GCFF线上医疗保健投资会议

> June 10, 2021 / 2021年6月10日

### **CytRx Safe Harbor Statement**

### 安全港声明

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 亮点

- CytRx's milestone and royalty agreement with Orphazyme for arimoclomol represents
  potential near term payments to CytRx / CytRx与Orphazyme就arimoclomol达成的里程碑和权
  利金协议代表了CytRx近期可能获得付款。
- Orphazyme has filed a New Drug Application (NDA) with the FDA for arimoclomol for NPC, which is currently under Priority Review with a target action date of June 17, 2021 / Orphazyme已经向美国食品药品管理局提交了治疗NPC的arimoclomol的新药申请(NDA),该申请目前 正处于优先审查阶段,目标行动日期为2021年6月17日。
- It also submitted a Marketing Authorisation Application with EMEA authorities for arimoclomol for NPC 它还向欧洲药品评估局提交了arimoclomol用于治疗NPC的市场授权申请。
- ImmunityBio has initiated a Phase 2 registrational-intent study for first-line and second-line locally advanced or metastatic pancreatic cancer, which includes aldoxorubicin / ImmunityBio已经启动了一项针对一线和二线局部晚期或转移性胰腺癌的2期注册意向研究,其中包括 aldoxorubicin
- Centurion BioPharma is a private oncology drug development company focused on cancer and has completed extensive pre-clinical work for its ultra high potency LADR™ drug candidates and albumin companion diagnostic (ACDx) / Centurion BioPharma是一家专注于 癌症的私营肿瘤药物开发公司,已经为其超高效力的LADR™候选药物和白蛋白辅助诊断(ACDx)完成了大量临 床前工作。



## CytRx has potential milestone/royalty payments and a subsidiary called Centurion BioPharma / CytRx有望收到里程碑/权利金付款,还有一个名为Centurion BioPharma的子公司。

Orphazyme Milestones and Royalties / Orphazyme里程 碑付款和权利金

Orphazyme: \$100M in potential milestones; plus royalties on arimoclomol / 潜在的\$1亿里程碑付款,另加 arimoclomol的权利金

ImmunityBio Milestones and Royalties / ImmunityBio 程碑付款和权利金

ImmunityBio: \$343M in potential milestones; plus royalties on aldoxorubicin / 潜在的\$3.43亿里程碑付款,另加aldoxorubicin的权利金

Centurion BioPharma Pipeline / Centurion BioPharma管线

Oncology drug development with a companion diagnostic 肿瘤药物开发和伴随诊断

Centurion BioPharma is a subsidiary of CytRx / Centurion BioPharma是CytRx的子公司



# CytRx milestones and royalties from Orphazyme for Arimoclomol / CytRx从Orphazyme就 Arimoclomol获得里程碑付款和权利金

Orphazyme Milestones and Royalties / Orphazyme里程 碑付款和权利金

Orphazyme: up to \$100M in milestones in addition to royalties on arimoclomol / 最高\$1亿里程碑付款,另加arimoclomol的权利金

### Niemann-Pick disease 尼曼匹克症("NPC")

- Orphazyme filed an NDA with the FDA with Priority Review, and a target action date of June 17, 2021; also submitted an MAA with the EMA, both for arimoclomol for Niemann-Pick disease Type C (NPC). / Orphazyme向美国食品药品管理局提交了一份优先审查的新药申请,目标行动日期为2021年6月17日;还向欧洲药品管理局提交了一份上市许可申请,都是关于arimoclomol治疗C型尼曼匹克病。
- Orphazyme has also received Breakthrough Therapy Designation for NPC. /Orphazyme还获得了NPC 的突破性疗法资格。
- Orphazyme launched an Early Access Program for NPC in January 2020 to further accelerate access to treatment with arimoclomol for people living with NPC. / Orphazyme于2020年1月启动了一项针对 NPC的早期治疗计划,以进一步加快NPC患者获得arimoclomol 的治疗。
- Total worldwide patients approximately 3,000. 全世界患者总数约为3000人。
- Expected price range is \$300,000 \$600,000; market potential \$500 Million. 预计价格范围为\$30万-\$60万: 市场潜力为\$5亿。
- Go to market in US Q3 2021 and EU/RoW H2 2021. / 2021年第三季度在美国上市,2021年下半年在欧盟 /世界其他地区上市。





### 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





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:

Cognition



CREATING TOMORROW, TODAY.

have mutations 95% nave 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 preparing for commercialization in 2021 for arimoclomol / Orphazyme准备在2021年对 arimoclomol进行商业化

Orphazyme: Preparing for commercialization in 2021 PDUFA date June 2021 for NPC



Building a highly specialized commercial footprint in US and EU



Source 来源: www.orphazyme.com

# CytRx potential milestones and royalties from ImmunityBio for aldoxorubicin / CytRx有望从 ImmunityBio就aldoxorubicin获得里程碑付款和权利金

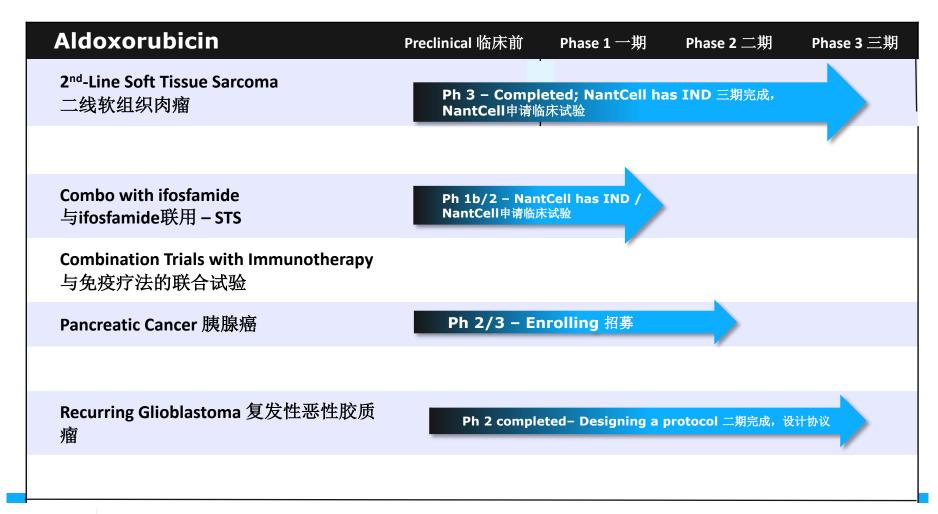
ImmunityBio Milestones and Royalties / ImmunityBio支付 里程碑付款和权利金

ImmunityBio: up to \$343M in milestones In addition to royalties on aldoxorubicin 最高\$3.43亿里程碑付款,另加aldoxorubicin的权利金

- ImmunityBio recently announced it was merging with NantKwest (NK) / ImmunityBio 最近宣布正在与NantKwest (NK) 合并。
- ImmunityBio has highlighted aldoxorubicin as one of three separate modalities of its platform / ImmunityBio强调aldoxorubicin是其平台的三个独立模式之一。
- ImmunityBio announced initiation of a phase 2 registrational-intent study using aldoxorubicin in combination with immunotherapy in metastatic pancreatic cancer / ImmunityBio宣布启动一项使用 aldoxorubicin联合免疫疗法治疗转移性胰腺癌的2期注册意向性研究。
- ImmunityBio, to date, plans to use aldoxorubicin in studies in glioblastoma, in addition to metastatic pancreatic cancer.迄今为止,ImmunityBio计划在胶质母细胞瘤的研究中使用aldoxorubicin,此外还有转移性胰腺癌。
- CytRx is entitled to increasing double-digit royalties on aldoxorubicin for soft tissue sarcomas and increasing single-digit royalties for all other indications / CytRx有权获得用于软组织肉瘤的 aldoxorubicin的两位数权利金,以及所有其他适应症的一位数权利金。
- ImmunityBio is reviewing options in Soft Tissue Sarcoma / ImmunityBio正在审查软组织肉瘤的备选方案



# CytRx partnered Pipeline with ImmunityBio / CytRx与ImmunityBio合作的管线 - aldoxorubicin





# Update from NantKwest/ImmunityBio at JP Morgan Conference in January 2021 / 2021年1月摩根医疗大会上NantKwest/ImmunityBio发布的更新信息

<u>Metastatic Pancreatic Cancer QUILT-88: early indications of increased survival rate with no other approved treatment options 转移性胰腺癌QUILT-88: 早期迹象表明 存活率提高,没有其他获批的治疗方案</u>

- In initial QUILT trials, median overall survival rate more than doubled compared to historical controls在最初的QUILT试验中,与历史对照组相比,中位总生存率提高了一倍多
- A single-arm Phase 2 trial was initiated in October 2020, for which the primary endpoint is overall survival and 83% of patients enrolled with second-line or greater pancreatic cancer remain alive to date / 2020年10月启动了一项单臂2期试验,其主要终点是总生存期,83%的二线或以上胰腺癌入组患者至今仍然存活。
- Former Senate Majority Leader Harry Reid's stage IV pancreatic cancer is now in "complete remission" after receiving this experimental combination immunotherapy that included aldoxorubicin 前参议院多数党领袖Harry Reid的第四期胰腺癌在接受了这种包括aldoxorubicin在内的实验性联合免疫疗法后,现在已经 "完全缓解"。
- Initiation of a <u>Registrational-Intent</u> Phase 2 randomized, three-cohort, open-label study for first and second-line treatment of locally advanced or metastatic pancreatic cancer 启动一项注册-意向性2期随机、三组、开放标签研究,用于局部晚期或转移性胰腺癌的一线和二线治疗
- Randomized trials in first and second-line pancreatic cancer are actively recruiting at three sites with more than 50 patients enrolled or being evaluated in QUILT-88 to date 一线和二线胰腺癌的随机试验正在三个地点积极招募,迄今已有50多名患者加入或正在接受QUILT-88的评估。



## Centurion BioPharma Highlights / Centurion BioPharma亮点



Centurion is a private, preclinical-stage oncology-focused biotechnology company pioneering the development of ultra-high potency cytotoxins with a diagnostic for patients with advanced solid malignancies / Centurion是一家私营的、处于临床前阶段的、以 肿瘤学为重点的生物技术公司,致力于开发超高效力的细胞毒素,为晚期实体恶性肿瘤患者提供诊断。



Centurion's LADR<sup>TM</sup> technology was developed by our Freiburg, Germany laboratory personnel who were early innovators in developing acid sensitive linkers attached to cytotoxins / Centurion的 LADR<sup>TM</sup>技术是由我们在德国弗莱堡的实验室人员开发的,他们是开发连接到细胞毒素的酸敏连接物的早期创新者。



Our **4 preclinical product** candidates **LADR-7**, **LADR-8**, **LADR-9**, and **LADR-10** were **developed by us exclusively**, as well as **our diagnostic ACDx (Albumin Companion Diagnostic)**我们的4个临床前候选产品LADR-7、LADR-8、LADR-9和LADR-10是由我们独家开发的,还有我们的诊断性ACDx(白蛋白辅助诊断)。



Centurion retains worldwide development and commercialization rights to all of its product candidates / Centurion保留了其所有 候选产品的全球开发和商业化权利。



Total capital investment to date in the LADR program and the diagnostic ACDx is over \$20 million / 迄今为止,LADR项目和诊断性 ACDx的资本投资总额超过\$2000万



Our plans are to initiate IND enabling studies and the clinical Phase 1-2 trial(s) with our diagnostic ACDx / 我们的计划是用我们的 诊断性ACDx 启动IND 启用研究和临床1-2期试验。



## CytRx subsidiary Centurion BioPharma has an oncology preclinical pipeline and diagnostic / CytRx的子公司 Centurion BioPharma拥有一个肿瘤学临床前管线和诊断系统

Centurion BioPharma Pipeline / Centurion BioPharma管线

Oncology drug development with a companion diagnostic 采用辅助诊断的肿瘤药物开发

LADR™ (linker activated drug release) <u>albumin</u> binding drug conjugates / LADR™ (连接剂激活药物释放) 白蛋白结合药物偶联物

LADR-7 (auristatin)

LADR-8 (auristatin)

LADR-9 (maytansinoid)

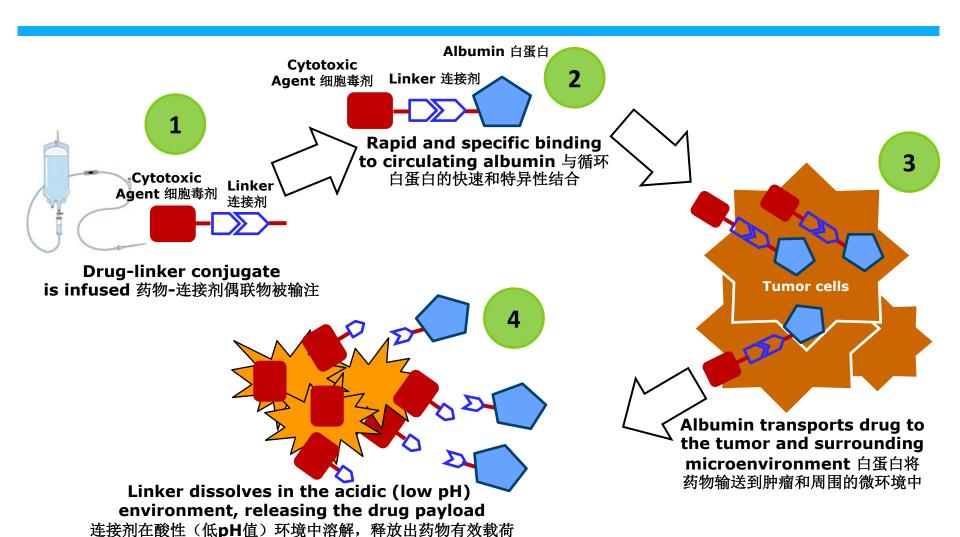
LADR-10 (maytansinoid)

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

identifies tumors eligible for treatment with LADR™ 白蛋白辅助诊断(ACDx)识别符合LADR™治疗的肿瘤



### **LADR™** Mechanism of Action 作用机理





## Centurion BioPharma Pipeline / Centurion BioPharma管线

- ACDx and four ultra high potency LADR™ drugs were selected for development / ACDx和四种超高效力的LADR™药物 被选中用于开发
- Non-GMP batches made and next step is technology transfer to make GMP material 制作了非GMP批次,下一步是技术转让,制作GMP材料。
- IND enabling studies can be initiated for 4 lead candidates. An IND submission is targeted for 2022 and starting of our Phase 1-2 clinical trial in the latter half of 2022. 可以为4个领先的候选药物启动IND授权研究。我们的目标是在2022年提交IND申请,并在2022年下半年开始进行1-2期临床试验。
- Long term patent protection (2035-2038) for **LADR™** technology, drug candidates, and diagnostic / **LADR™**技术、 候选药物和诊断的长期专利保护(2035-2038)。

LADR™ Albumin Binding Drug
Conjugates / LADR™白蛋白结合药物偶联物

Auristatin Program
LADR-7
LADR-8

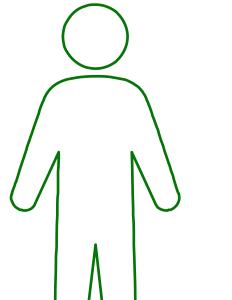
Maytansinoid Program
LADR-9
LADR-10

Companion Diagnostic 伴随诊断 – ACDx identifies patients across solid tumors which have the potential to respond / ACDx可识别有可能产生反应的各种实体瘤的患者

# Auristatin and Maytansinoid LADR™s Are Efficacious in Different Xenograft Tumor Models / Auristatin 和 Maytansinoid 的LADR™对不同的异种移植肿瘤模型具有疗效



Breast 乳房







Head and Neck 头颈



Ovarian 卵巢



Melanoma 黑素瘤



Pes et al., Journal of Controlled Release (2019) 296:81 and Supplemental Material; Poster LADR 9 and 10



### **Recent and Upcoming Catalysts**

### 最近和未来催化剂

### 2020-2021

- ✓ 1H 2020: Orphazyme filed for FDA approval for arimoclomol in Niemann-Pick Type C disease with a target action date of 06/17/21 2020年上半年: Orphazyme申请FDA批准arimoclomol用于尼曼匹克C型疾病,目标行动日期为2021年6月17日。
- ✓ 2H 2020: Orphazyme has submitted for EMEA (Europe) approval for arimoclomol in Niemann-Pick Type C disease 2020年下半年: Orphazyme公司已经提请EMEA(欧洲)批准关于arimoclomol治疗尼曼匹克C型疾病。
- 2020-2021: Upon approval, CytRx is to receive a \$12 million milestone payment if the US, Europe and Japan are approved (\$6 million for US, \$4 million for Europe and \$2 million for Japan)
   2020-2021: 一旦获得批准,如果美国、欧洲和日本获得批准,CytRx将获得\$1200万的
  - **2020-2021:** 一旦获得批准,如果美国、欧洲和日本获得批准,CytRx将获得\$1200万的里程碑付款(美国为\$600万,欧洲为\$400万,日本为\$200万)。



## Financial Summary 财务数据

• Cash Position 现金 ( 03/31/21)

\$9.3M / \$930万

No Debt 无债务

■ Shares Outstanding 已发行股票

36.5M /3650万

■ **Options** Weighted-average strike price 期权,加权平均价格: \$7.43

3.2M/320万

 Fully-Diluted Share Count 完全摊薄后股数(3/31/2021) 39.7M /3970万



## Summary 总结

- Orphazyme could deliver milestones and royalties / Orphazyme可能支付里程碑付款和权利金
- ImmunityBio could deliver milestones and royalties / ImmunityBio可能支付里程碑付款和权利金
- Cash burn rate is ~\$430k per month 资金消耗率约为每月\$43万
- Potential to shelter future income with non-restrictive netoperating carry-forward losses ("NOL's") of approximately \$250 million 有可能通过非限制性净运营结转损失("NOL")来保护未来的收入,大约有\$2.5亿。

