



LLS TAP

THERAPY ACCELERATION PROGRAM

LORE GRUENBAUM, VP, TAP
JAVEED FROOZAN, VP, BD & SA

JULY 2020

BEATING CANCER IS IN OUR BLOOD.

LEUKEMIA &
LYMPHOMA
SOCIETY®

The mission of The Leukemia & Lymphoma Society (LLS) is to cure leukemia, lymphoma, Hodgkin's disease and myeloma, and improve the quality of life of patients and their families.

We fund **RESEARCH** to advance lifesaving treatments.

We provide patients, survivors, caregivers, families and healthcare professionals with hope, guidance, **EDUCATION** and **SUPPORT**.

We drive **ADVOCACY** for policies that protect patient access to lifesaving treatment.



Approximately every **3 minutes** someone in the U.S. is diagnosed with blood cancer



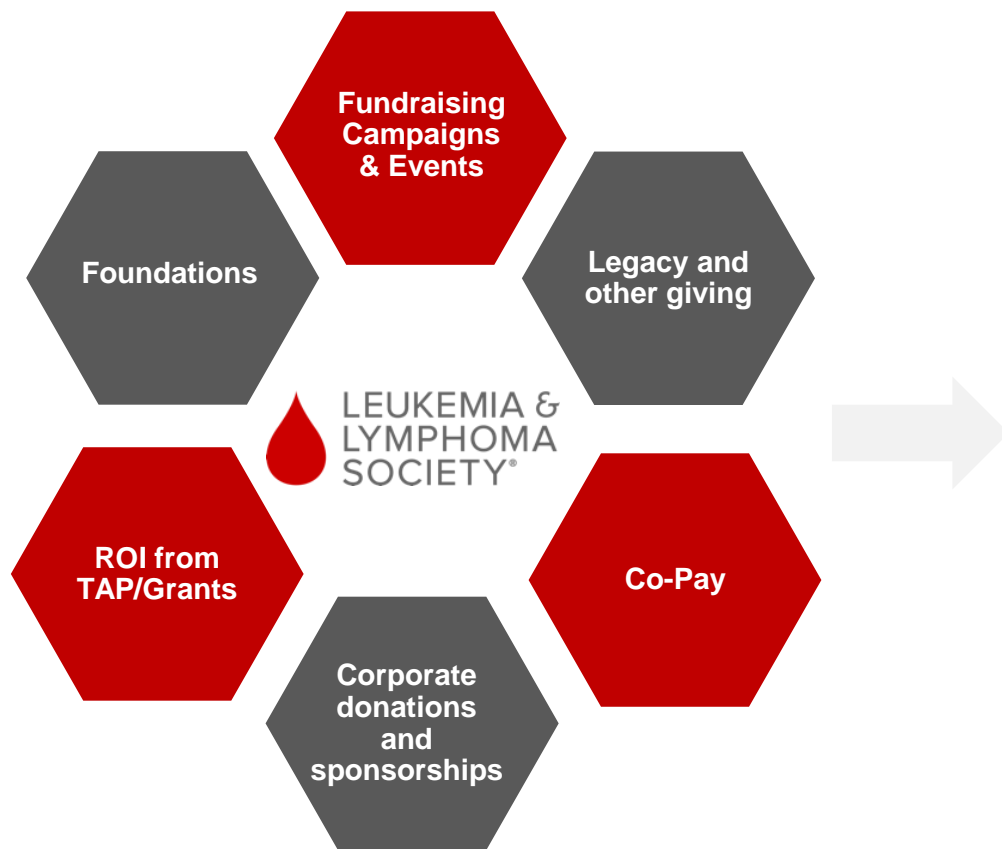
Nearly **1.4 million** people in the U.S. are living with or in remission from leukemia, lymphoma or myeloma



About **30 percent** of blood cancer patients still do not survive five years after diagnosis



About **40 percent** of all pediatric cancers are blood cancers



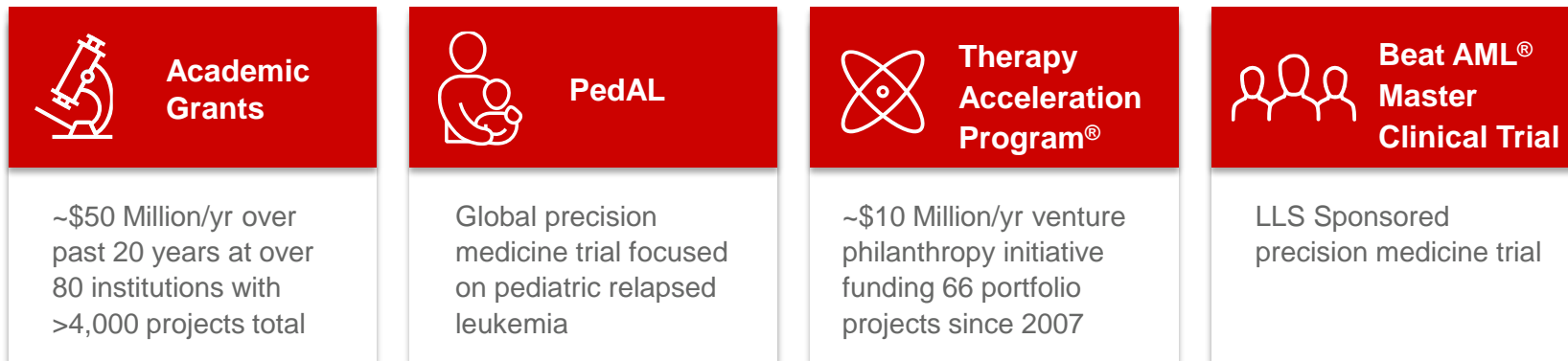
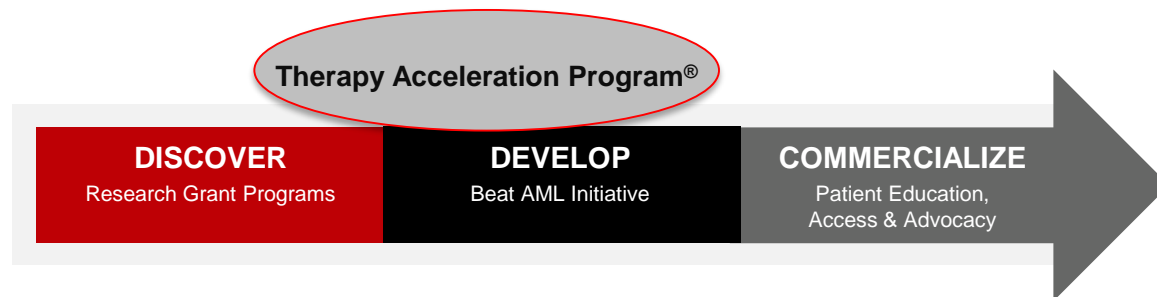
OUR IMPACT

- Invested nearly \$1.3 billion in research and development worldwide since founded in 1949
- Helped advance 52 of 60 FDA approved blood cancer drugs
- Supported >93,000 patients since inception
- Responded to 20,000 inquiries in 2019

LLS GLOBAL RESEARCH AND DEVELOPMENT FOCUS

PAGE 4

Research and development programs and clinical trials using LLS resources



LLS THERAPY ACCELERATION PROGRAM (TAP)

PAGE 5

Venture philanthropy funding to support novel therapies

Established in 2007

>\$120 Million invested to date

- Biotech: >\$80 Million
- Institutions: ~\$40 Million
- >60 financings of companies and assets
- >20 assets currently in clinical development

3 FDA-Approved Therapies

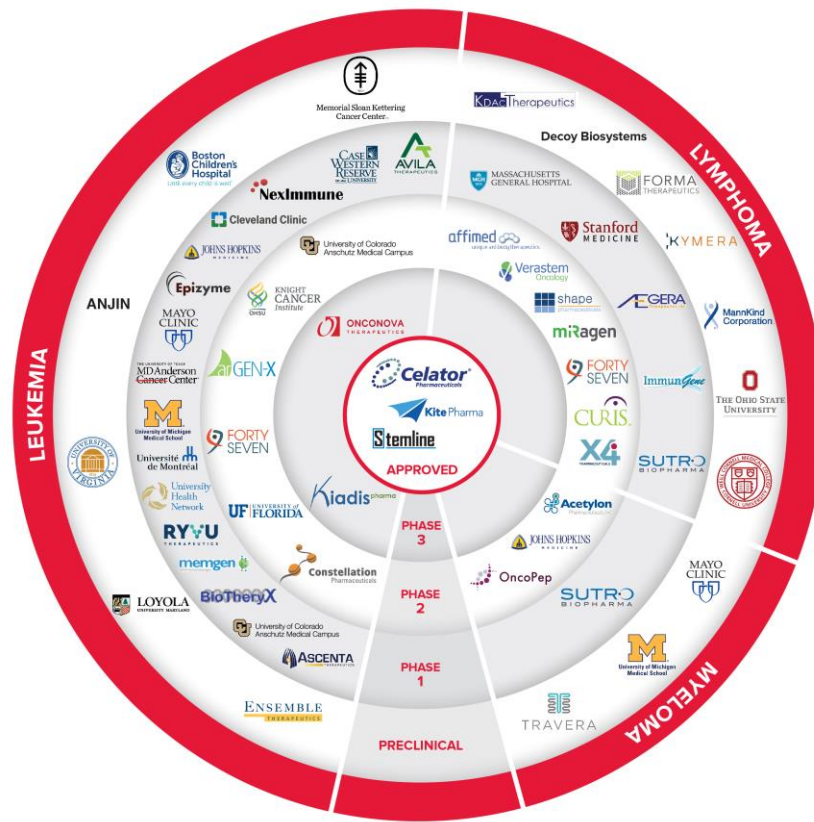
- Vyxeos (AML)
- Yescarta (DLBCL, tFL, PMBCL)
- Elzonris (BPDCN)

ROI Focus:

- FDA Approvals
- Assets in clinical development
- Strategic transactions & financing for portfolio companies
- Financial ROI to LLS

www.LLS.org/therapy-acceleration-program

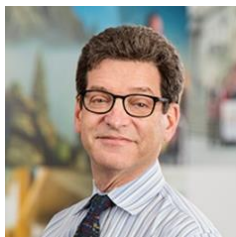
BEATING CANCER IS IN OUR BLOOD.





Lore Gruenbaum, PhD
VP, TAP

- 20 years drug discovery & clinical development
- VP, Gotham Therapeutics; Exec Dir, Applied Biomath
- Biomarker Head, Virology, Roche; Group Leader, BI
- Yale postdoctoral work, principal investigator and collaborator on several SBIR grants



Lee Greenberger, PhD
SVP, Chief Scientific Officer

- 20 years big pharma and biotech
- Oversight responsibility for >\$50 M annual research budget
- Advanced > 10 oncology therapeutics into the clinic
- Search & due diligence experience with big pharma



Javeed Froozan, MBA, BS
VP, Business Development

- 25 years biopharma and health technology value creation
- Sr. Dir, Emergent BioSolutions, Multiple start-ups/exits, 2 IPOs
- Business lead on EBS-Trubion M&A transaction. Alliance Manager for Pfizer relationship
- Strategic Investments, M&A, Business Development, Asset Management, and Economic Development



Blaine Robinson, PhD
Executive Director, TAP

- 15 years research & clinical development in blood cancer
- Search & Diligence on 100+ projects, managed over 20 TAP partnerships including Constellation, Epizyme, miRagen & most recently Kymera
- Pediatric leukemia researcher, Children's Hospital of Philadelphia



Jun Xu, PhD
Executive Director –
TAP Lead

- 20 years oncology/immunology drug discovery/development
- Search & Diligence on 100+ projects
- Scientific lead for over 20 TAP projects including multiple high impact ones, such as Stemline, Kite, argenX, and Forty Seven

Therapy Acceleration Program Committee: <https://www.lls.org/therapy-acceleration-program/oversight>

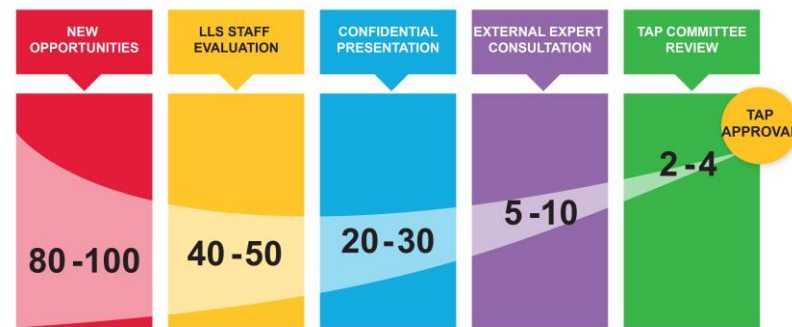
TAP GOALS & INVESTMENT STRATEGY

PAGE 7

Accelerate innovative blood cancer therapies and generate ROI for LLS mission

Focus on high-value assets:

- Existing and emerging populations with high unmet needs
- Gaps in current and emerging treatment landscape
- Innovative science, first-in-class assets
- First-in-heme/onc and registration trials
- Strong intellectual property, management, and finances



Average annual number

2 PATHS TO CO-INVEST WITH INVESTORS AND VENTURE PHILANTHROPIES



Strategic

- Range of Investment: \$2 Million to \$10 Million
- Presentation to TAP Committee
- Typically 3-6 month to reach TAP Committee



Opportunistic

- Target Investment: \$500,000
- LLS TAP team briefs TAP Committee Chair
- Transaction completion in 1-3 months

TAP VALUE ADD TO BIOTECH COMPANIES

TAP-funded companies benefit from LLS blood cancer insight

- Deep knowledge of indications and rapidly changing SoC
- Unique scientific, clinical, and drug development expertise
- Patient access services to enable understanding of patient needs
- Immediate access to extensive KOL network
- Pharmaceutical, biotech, and research institution partner connections
- Regulatory insight through LLS initiatives (Beat AML Master Clinical Trial®)

TAP record of success provides scientific & investment credibility, and visibility enabling companies to raise additional funds.

TAP ACADEMIC CLINICAL MODEL

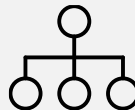
PAGE 10

Co-fund assets with institutions on clinical stage projects



Therapeutics Development

Collaborate with leading institutions on assets with potential to outlicense or spin out



Clinical Indication Expansion

Actively solicit and fund innovative ISTs to impact the current SoC



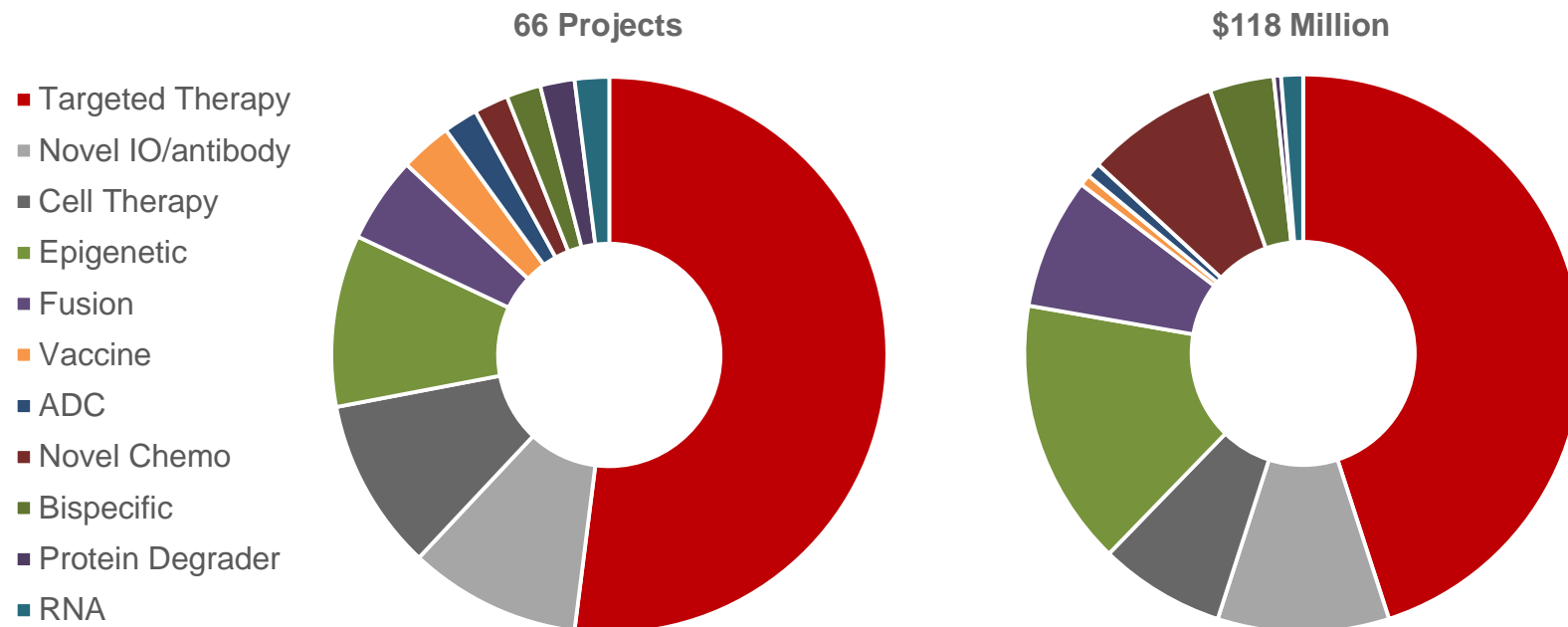
Special Projects

Build collaborations with non-profits and other cooperative groups

TAP PORTFOLIO THERAPEUTIC PLATFORMS FUNDED (2007-2020)

PAGE 11

Portfolio is aligned with strong industry focus on Targeted Therapy and reflects growing interest in Cell and IO Therapies in blood cancer



TAP PORTFOLIO INVESTMENTS IN ACUTE MYELOID LEUKEMIA (AML)

PAGE 12

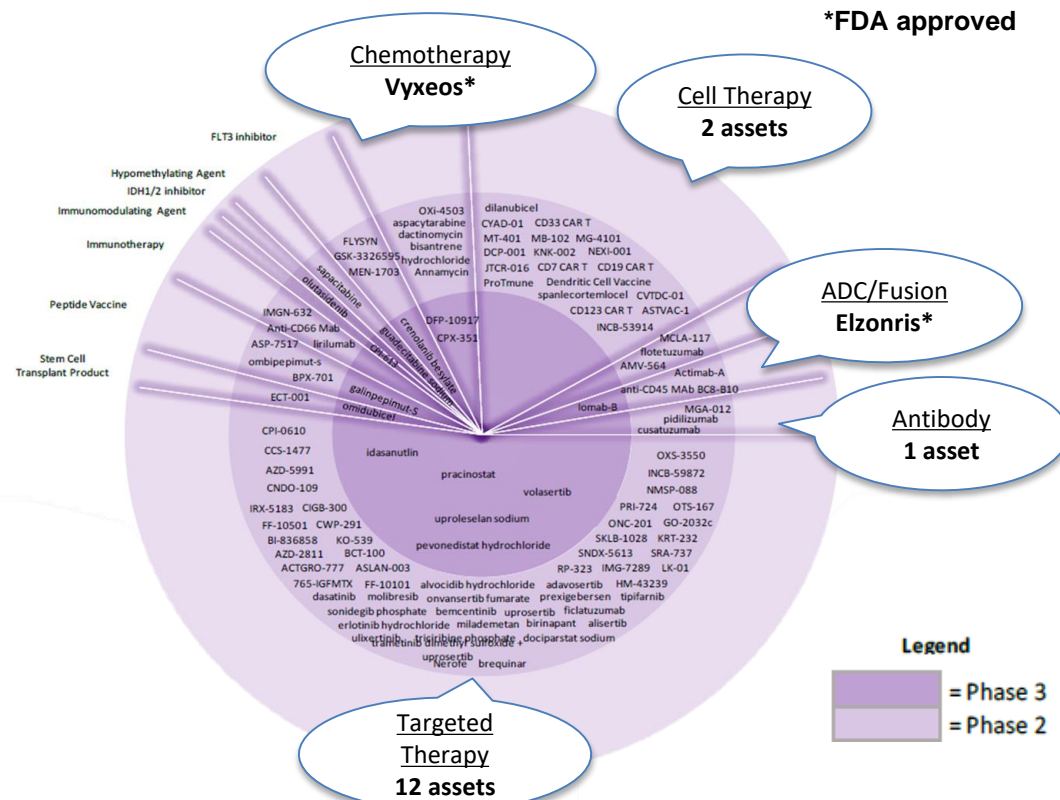
TAP team understands & successfully invests in complex therapeutic areas

High Unmet Medical Need

- 72,000 newly diagnosed in 8 major markets (2019)
- >10,000 deaths per year in US
- Complex, heterogeneous disease
- Ineffective long-term disease control with current therapies
- Elderly patients not fit for chemo
- Growing use of targeted therapies and combinations













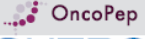

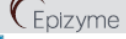






Significant Market Opportunity

- Global AML market \$1.4 Billion (2019)
- CAGR 13.6% (projected to 2029)



TAP PORTFOLIO ASSETS IN CLINICAL DEVELOPMENT

PAGE 13

| Therapy | Target/Modality | Indications | Preclinical | Phase 1 | Phase 2 | Phase 2 Reg / Phase 3 |
|------------------------------------|--|-------------------------------------|--|--|---|---|
| Rigosertib | PLK1/PI3K Small molecule | MDS | | | |  |
| Magrolimab + Azacitidine | Anti-CD47 Antibody | MDS | | | |  |
| Magrolimab + Rituximab | Anti-CD47 Antibody | DLBCL | | | |  |
| AFM13 | CD30/CD16A Bispecific Engager | PTCL | | | |  |
| CPI-0610 +/- Ruxolitinib | BET Small molecule | MPN | | |  | |
| ARGX-110 +/- Azacitidine or Ven | Anti-CD70 Antibody | AML | | |  | |
| Duvelisib + Romidepsin | PI3K δ / γ Small molecule | PTCL | | |  | |
| Cobomarsen | miR-155 Small molecule | CTCL | | |  | |
| Fimepinostat + Ven or Rux/Benda | HDAC/PI3K Small molecule | DLBCL | | |  | |
| X4P-001 + Ibrutinib | CXCR4 Small molecule | Waldenström's | |  | | |
| K-NK002 | NK Cell Therapy | AML/MDS | |  | | |
| SEL120 | CDK8/19 Small molecule | AML/MDS | |  | | |
| PCX-410 + ACY-241 +/- Len | Vaccine | Smoldering myeloma | |  | | |
| STRO-001 | Anti-CD74 Antibody | MM/DLBCL/MCL/FL | |  | | |
| Pinometostat + Aza or SOC | DOT1L Small molecule | MLL leukemia | |  | | |
| CAR-37 | T Cell Therapy | B-cell lymphoma/ T-cell lymphoma | |  | | |
| BTX-A51 | CK1- α /CDK7/CDK9 Small molecule | AML/MDS | |  | | |
| NK-cells + ALT-803 | NK Cell Therapy | Heme malignancies | |  | | |
| KO-539 | Menin Small molecule | MLL leukemia | |  | | |
| NEXI-001 | T Cell Therapy | AML | |  | | |
| PROTACs | IRAK4/STAT3 Small molecule | Lymphoma |  | | | |

Updated June 2020

TAP FUNDED ASSETS CREATE STRATEGIC VALUE

TAP portfolio partners have had successful M&A, collaboration and license transactions

LLS TAP
Funding

\$40 Million

| | → | |
|--|---|--|
|   | |  |
|  | |  |
|  | |  |
|  | |  <small>PHARMACEUTICAL COMPANIES OF Johnson & Johnson</small> |
|   | |  |
|  | | |
|  | |  |
|  | |  |

Strategic
Transactions

\$20 Billion

SIGNIFICANT EQUITY FINANCING RAISED SINCE LLS TAP FUNDING

| Equity since TAP Funding* | TAP Portfolio Company |
|---------------------------|---|
| >\$1 Billion | arGEN-X EpiZyme |
| >\$500 Million | Constellation Kura ² [Onconova] |
| \$250-\$500 Million | Forty Seven ¹ |
| \$100-\$250 Million | Affimed Sutro MiRagen ² X4 ² Curis Kymera ² |
| \$50-\$100 Million | BioTheryx ² Ryvu WindMIL ² |
| <\$50 Million | Neximmune ² OncoPep ² |

KEY POINTS

LLS TAP has established record of success

- Targeting unmet medical needs
- Leading to FDA approvals of life changing therapeutics
- Creating value for patients, companies and ROI for the LLS mission

LLS would like to expand the reach & impact of the TAP program

- Leverage its unique expertise in novel collaborations
- Attract more companies and investors to blood cancer indications
- Expand TAP capacity to support the most promising assets

For more information, contact:

Lore Gruenbaum, PhD 914.821.8361 | Lore.Gruenbaum@LLS.org

Javeed Froozan, MBA 914.821.8817 | Javeed.Froozan@LLS.org



TAP SUCCESS STORIES

BEATING CANCER IS IN OUR BLOOD.



TAP SUCCESS: NOVEL LIPOSOMAL CYTOTOXIC THERAPY

PAGE 18

Vyxeos™ is the first FDA-approved treatment for two types of poor-prognosis AML (2017)

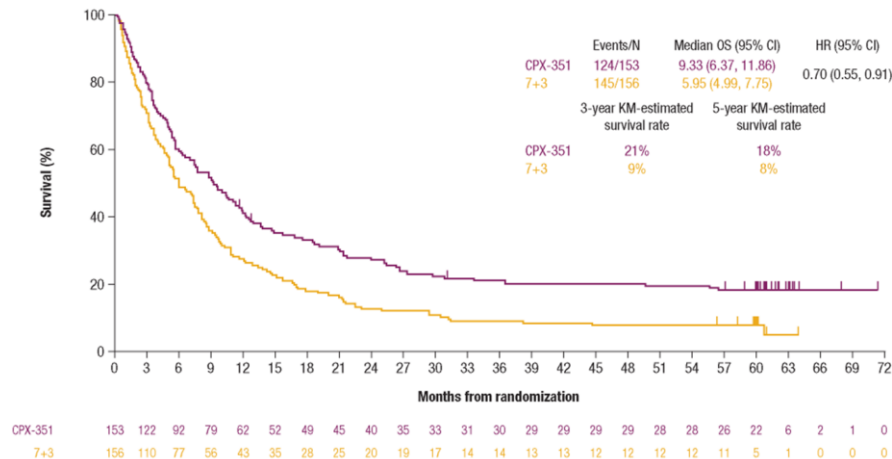


**ACQUIRED BY JAZZ PHARMA
FOR \$1.5 BILLION IN 2016**

LLS TAP PROVIDED:

\$9.15 MILLION ASSET FUNDING

ROI: \$25.3 MILLION



Five-year final results of a phase 3 study of CPX-351 versus 7+3 in older adults with newly diagnosed high-risk/secondary AML

J. Lancet et al., ASCO 2020

TAP SUCCESS: CD19 CHIMERIC ANTIGEN RECEPTOR (CAR) T-CELL THERAPY

PAGE 19

Yescarta™ is the first FDA-approved CAR-T Therapy in NHL (2017)

LLS has invested > \$80 M in Cellular Immunotherapy since 1998

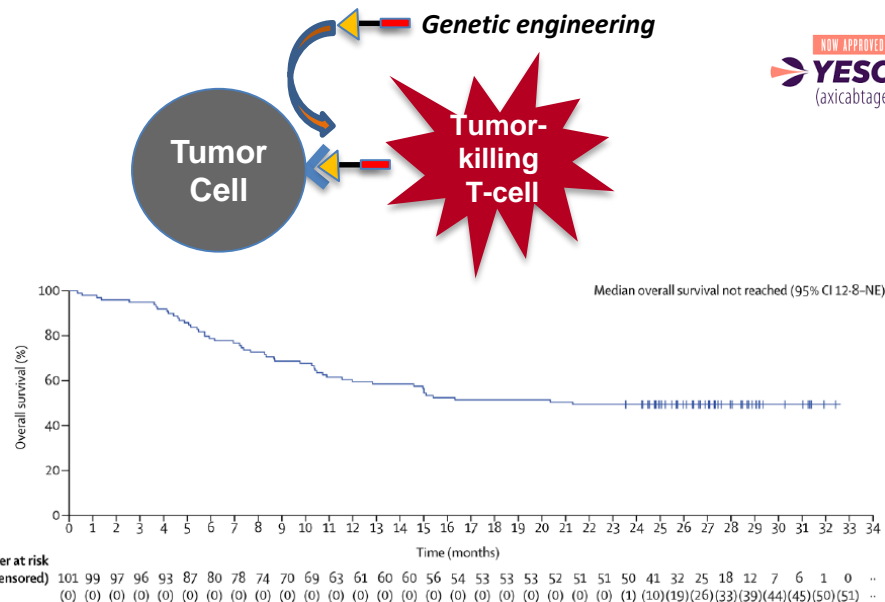


ACQUIRED BY GILEAD FOR \$11.9 BILLION IN 2017

LLS TAP PROVIDED:

\$2.5 MILLION ASSET FUNDING

ROI: \$6.25 MILLION



Long-term safety and activity of axicabtagene ciloleucel in refractory large B-cell lymphoma (ZUMA-1): a single-arm, multicenter, Ph 1-2 trial

Locke et al. 2019. Lancet Oncology

TAP SUCCESS: NOVEL TARGETED CD123 FUSION PROTEIN

PAGE 20

Elzonris is the first approved therapy for rare blood cancer indication BPDCN (2018)



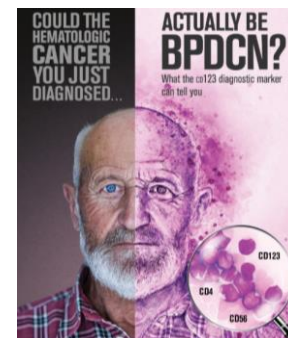
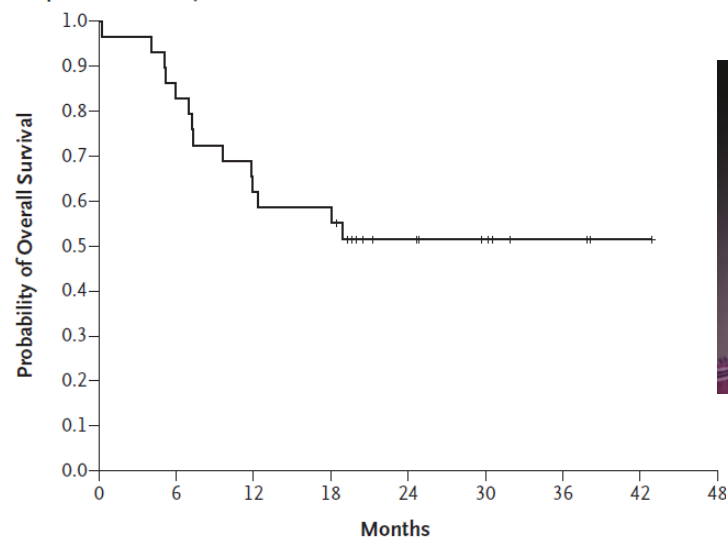
ACQUIRED BY MENARINI GROUP
FOR \$677 MILLION IN 2020

LLS TAP PROVIDED:

\$2.9 MILLION NET ASSET FUNDING

ROI: \$5.8 MILLION TO DATE

B Kaplan-Meier Analysis of Overall Survival



Treatment outcomes of 29 patients with blastic plasmacytoid dendritic cell neoplasm (BPDCN) who received first-line treatment with tagraxofusp: Probability of overall survival

Pemmaraju et al., 2019. NEM

TAP SUCCESS: MAGROLIMAB (ANTI-CD47 ANTIBODY)

PAGE 21

Magrolimab + Azacitidine induces high response rates in MDS and AML

Initiation of registration-enabling studies in 2020

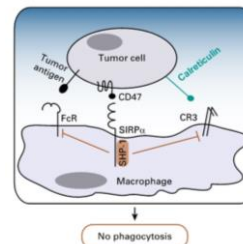


ACQUIRED BY GILEAD FOR \$4.9 BILLION IN 2020

LLS TAP PROVIDED:

\$4.175 MILLION ASSET FUNDING
\$3 MILLION EQUITY INVESTMENT

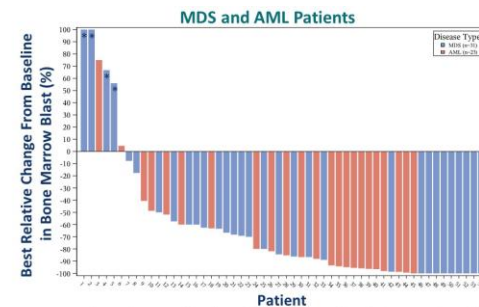
ROI: >\$40 MILLION



Magrolimab blocks the 'don't eat me' signal on tumor cells

| Best Overall Response | 1L MDS N=33 | 1L AML N=25 |
|------------------------------|----------------------------------|----------------|
| ORR | 30 (91%) | 16 (64%) |
| CR | 14 (42%) | 10 (40%) |
| CRi | NA | 4 (16%) |
| PR | 1 (3%) | 1 (4%) |
| MLFS/marrow CR | 8 (24%) 4 with marrow CR + HI | 1 (4%) |
| Hematologic improvement (HI) | 7 (21%) | NA |
| SD | 3 (9%) | 8 (32%) |
| PD | 0 | 1 (4%) |

Response assessments per 2008 IWG MDS criteria and 2017 AML ELN criteria. Patients with at least 1 post-treatment response assessment are shown; all other patients are on therapy and are too early for first response assessment, except for 2 MDS patients not evaluable (withdrawal of consent) and 3 AML patients (1 AE, 2 early withdrawal).



- Magrolimab + AZA induces a 91% ORR (42% CR) in MDS and 64% ORR (56% CR/CRi) in AML
- Responses deepened over time with a 56% 6-month CR rate in MDS patients (assessed in all patients 6 months after initial treatment)
- Median time to response is 1.9 months, more rapid than AZA alone
- Magrolimab + AZA efficacy compares favorably to AZA monotherapy (CR rate 6-17%^{1,2})

1. Azacitidine USPL 2. Fenaux P, et al. *Lancet Oncol*. 2009;10(3):223-232.

PRESENTED AT: 2020 ASCO ANNUAL MEETING

#ASCO20
ORAL AND POSTER PRESENTATIONS OF THE LATEST RESEARCH RESULTS IN CANCER

PRESENTED BY: DAVID A. SALLMAN, MD

11

TAP SUCCESS: KO-539 (MENIN INHIBITOR)

PAGE 22

First-in-class inhibitor of the menin-MLL interaction in Ph1 trial for patients with relapsed/refractory AML



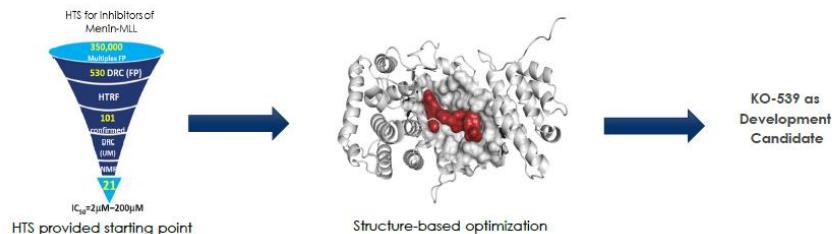
**PRECLINICAL COMPOUNDS
RELATED TO KO-539 LICENSED
TO KURA ONCOLOGY IN 2015**

LLS TAP PROVIDED:

**\$6.31 MILLION ASSET FUNDING
TO U MICHIGAN**

**ROI: EQUITY: 26,000+ SHARES
+ \$26,000+ CASH TO DATE**

**KO-539 is a potent & selective menin-MLL inhibitor
with robust activity in models of MLL-rearranged AML**



Burrows et al. 2017. AACR poster

- Grants initially and then TAP supported preclinical development (including chemistry) of menin-MLL interaction inhibitors by Jolanta Grembecka at University of Michigan and licensing of assets to Kura Oncology in Dec 2014
- KO-539 received orphan drug designation in July 2019; in ph1/2a trial for r/r AML with MLL fusions/NPM1 mutations (FPI in Sep 2019)

Casey Cunningham, MD (Chair) +
Santé Ventures

Giulio Draetta, MD, PhD
The University of Texas MD Anderson Cancer Center

Christopher Flowers, MD, MS, FASCO +
The University of Texas MD Anderson Cancer Center

Patrick Fortune, PhD, MBA
Partners Healthcare Systems

Laura Kaufman, PhD, DABT
Private Consultant

Ronald Levy, MD
Stanford University School of Medicine

Vern Norviel, Esq.
Wilson Sonsini Goodrich & Rosati

Robert Rosen, JD +
Grewhawke Capital Advisors

Steven Rosen, MD, FACP +
City of Hope

Robert Spiegel, MD, FACP
Spiegel Consulting LLC

David Weinstock, MD
Dana-Farber Cancer Institute

+ National Board Member

THANK YOU!

PAGE 24

LLS Research Grants and TAP



Lee Greenberger, PhD
CSO & SVP Research



Michael Yaffe, PhD
VP of Research



Erik Nelson, PhD
Exec. Dir. Research



James Kasper, MS
Sr. Dir. Research



Orsi Giricz, PhD
Dir. Research



Lore Gruenbaum, PhD
VP of TAP



Jun Xu, Ph.D.
Exec. Dir. TAP



Blaine Robinson, PhD
Exec. Dir. TAP



Javeed Froozan, MBA
VP of BD & Alliance